

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

**FORM S-1  
REGISTRATION STATEMENT  
UNDER  
THE SECURITIES ACT OF 1933**

**GENERATION BIO CO.**

(Exact name of registrant as specified in its charter)

Delaware  
(State or other jurisdiction of  
incorporation or organization)

2834  
(Primary Standard Industrial  
Classification Code Number)

81-4301284  
(I.R.S. Employer  
Identification No.)

301 Binney Street  
Cambridge, MA 02142  
(617) 655-7500  
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Geoff McDonough, M.D.  
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**Approximate date of commencement of proposed sale to the public:** As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box.

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.  \_\_\_\_\_

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.  \_\_\_\_\_

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering.  \_\_\_\_\_

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer  Accelerated filer   
Non-accelerated filer  Smaller reporting company   
Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 7(a)(2)(B) of the Securities Act.

**CALCULATION OF REGISTRATION FEE**

| Title of Each Class<br>of Securities to Be Registered  | Proposed<br>Maximum<br>Aggregate Offering Price(1) | Amount of Registration<br>Fee(2) |
|--|--|----------------------------------|
| Common stock, \$0.0001 par value per share   | \$   | \$                               |
| (1) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended. Includes the aggregate offering price of additional shares that the underwriters have the option to purchase. See "Underwriting." |  |                                  |
| (2) Calculated pursuant to Rule 457(o) under the Securities Act of 1933, as amended, based on an estimate of the proposed maximum aggregate offering price.  |  |                                  |

The registrant hereby amends this registration statement on such date or dates as may be necessary to delay its effective date until the registrant shall file a further amendment which specifically states that this registration statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the registration statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

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The information in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any state where the offer or sale is not permitted.

Subject to completion dated \_\_\_\_\_, 2020

Preliminary prospectus

shares



## Common stock

This is Generation Bio Co.'s initial public offering. We are selling \_\_\_\_\_ shares of our common stock.

We expect the public offering price to be between \$ \_\_\_\_\_ and \$ \_\_\_\_\_ per share. Currently, no public market exists for the shares. After the pricing of the offering, we expect that the shares will trade on the Nasdaq Global Market under the symbol "GBIO."

Investing in the common stock involves risks that are described in the "[Risk factors](#)" section beginning on page 12 of this prospectus.

We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012 and, as such, have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings. See "Prospectus summary—Implications of being an emerging growth company."

|                                  | Per Share | Total |
|----------------------------------|-----------|-------|
| Public offering price            | \$        | \$    |
| Underwriting discount(1)         | \$        | \$    |
| Proceeds, before expenses, to us | \$        | \$    |

(1) See "Underwriting" for a description of all compensation payable to the underwriters.

The underwriters may also exercise their option to purchase up to an additional \_\_\_\_\_ shares from us, at the public offering price, less the underwriting discount, for 30 days after the date of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The shares will be ready for delivery on or about \_\_\_\_\_, 2020.

*Joint bookrunning managers*

**J.P. Morgan**

**Jefferies**

**Cowen**

*Lead manager*

**Wedbush PacGrow**

\_\_\_\_\_, 2020

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Neither we nor the underwriters have authorized anyone to provide you with any information other than that contained in this prospectus, any amendment or supplement to this prospectus or in any free writing prospectus we may authorize to be delivered or made available to you. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

For investors outside the United States: we have not, and the underwriters have not, done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. Persons outside the United States who come into possession of this prospectus must inform themselves about, and observe any restrictions relating to, the offering of the shares of common stock and the distribution of this prospectus outside the United States.

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Through and including \_\_\_\_\_, 2020, (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

We own or have rights to trademarks, service marks and trade names that we use in connection with the operation of our business, including our corporate name, logos and website names. The service marks and trademarks that we own include the mark Generation Bio™ and the Generation Bio logo. Other trademarks, service marks and trade names appearing in this prospectus are the property of their respective owners. Solely for convenience, some of the trademarks, service marks and trade names referred to in this prospectus are listed without the ® and ™ symbols, but we will assert, to the fullest extent under applicable law, our rights to our trademarks, service marks and trade names.

## Prospectus summary

*This summary highlights information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, including our consolidated financial statements and the related notes appearing elsewhere in this prospectus and the information set forth in the sections titled “Risk factors” and “Management’s discussion and analysis of financial condition and results of operations.” As used in this prospectus, unless the context otherwise requires, references to “we,” “us,” “our” and “Generation Bio Co.” refer to the consolidated operations of Generation Bio Co. and its wholly-owned subsidiary.*

### Overview

We are an innovative genetic medicines company creating a new class of gene therapy utilizing our proprietary non-viral gene therapy platform to provide durable, redosable treatments for millions of patients living with rare and prevalent diseases. Our non-viral gene therapy platform incorporates our high-capacity DNA construct called closed-ended DNA, or ceDNA; our cell-targeted lipid nanoparticle delivery system, or ctLNP; and our established, scalable capsid-free manufacturing process. Using our approach, we are developing novel gene therapies to provide targeted delivery of genetic payloads that include large and multiple genes to a range of tissues across a broad array of diseases. We are also engineering our gene therapies to be redosable, which may enable individualized patient titration to reach the desired level of therapeutic expression and to maintain efficacy throughout a patient’s life.

We are focused on diseases with significant unmet need for which our non-viral gene therapy platform may substantially improve clinical efficacy relative to current gene therapy approaches. We are prioritizing programs for rare monogenic diseases of the liver and retina, which result from mutations in a single gene, that have well-established biomarkers and clear clinical and regulatory pathways. We plan to expand our portfolio by pursuing additional programs in rare and prevalent diseases of the liver and retina, as well as in skeletal muscle, the central nervous system, or CNS, and oncology by developing discrete ctLNPs, each engineered to reach a different tissue.

By creating this new class of gene therapy, we believe we can reach previously untreatable or under-treated patients and address new indications, including those with large patient populations, thereby unlocking the full potential of genetic medicine.

### ***Our non-viral gene therapy platform***

Our non-viral gene therapy platform is comprised of three essential components: our high-capacity ceDNA construct, which can accommodate large or multiple genes as well as native regulatory elements; our ctLNP delivery system, which enables highly specific delivery of ceDNA to a range of tissues; and our established, scalable capsid-free manufacturing process, that uses a cost-effective biologics infrastructure with the potential to reach patients with rare diseases and to expand access to patients with prevalent diseases, requiring millions of doses on a sustainable basis.

**ceDNA**—Our high-capacity ceDNA is an engineered, double-stranded, linear, covalently closed-ended DNA construct that includes the gene of interest and associated regulatory sequences. We have produced ceDNA constructs of 12 kilobases, or kb, which have almost three times the capacity of adeno-associated virus, or AAV, gene therapy approaches. We believe ceDNA can deliver a significant majority of the human coding sequences

known to be relevant for the treatment of diseases that result from mutations in a single gene, in multiple genes, or in those requiring more than one type of genetic correction.

*ctLNP*—Our ctLNP delivery system builds upon clinically validated lipid nanoparticles, or LNPs, and is designed to allow for repeat dosing of a genetic payload without stimulating an immune response, such as antibody production. We have taken a significant step beyond current LNP technologies by adding a biological targeting molecule, called a ligand, on the surface of our LNPs to direct their biodistribution to specific tissues. Different targeting ligands may enable our ctLNPs to actively target specific cell receptors in the liver, retina, skeletal muscle, CNS and tumors. In addition, our ctLNP delivery system may confer the advantages of predictable behavior across species, minimal off-target effects and a foundational platform approach that unlocks the ability to target the widest possible spectrum of diseases with our therapies.

*Manufacturing process*—Our established, scalable manufacturing employs a proprietary capsid-free process and utilizes standard biologics infrastructure, unlike traditional viral gene therapy manufacturing. This highly efficient and reproducible process includes rigorous industrial-scale purification that consistently yields greater than 99% pure ceDNA. We believe that our ability to conduct our manufacturing process at the 200-liter scale with high product quality suggests that further scaling to thousands of liters per batch is feasible using standard biological production equipment and engineering methods. We believe the combination of the expected multi-year durability of a single dose of ceDNA, tissue-specific delivery and manufacturing capacity may provide dosing for millions of patients living with prevalent diseases.

***Advantages of our non-viral gene therapy platform***

Our non-viral gene therapy platform is designed to overcome the limitations of current gene therapy approaches and disrupt the field of genetic medicine. Specifically, our platform may provide the following significant advantages for patients, physicians and payers:

- *Durable expression.* Our ceDNA is highly stable and potentially enables years-long expression for patients with each dose, minimizing treatment burden.
- *Redosable administration.* Our ctLNP delivery system has been designed to avoid stimulating an antibody response in patients, thereby enabling redosing, which may allow for individualized patient titration to reach desired expression levels as well as extended therapeutic expression and the ability to treat pediatric patients.
- *Greater opportunity to demonstrate efficacy in first-in-human trials.* Because we may be able to redose, we believe that a greater proportion of patients participating in our early clinical trials may achieve the desired level of gene expression.
- *Addressing untreated or under-treated patients.* Our therapy may be used to treat patients with pre-existing immunity to AAV viral capsids or whose efficacy outcomes on current gene therapies were insufficient in level of duration or expression.
- *Delivery of large genetic payloads.* The payload capacity of ceDNA enables our constructs to carry larger genes and/or multiple genes, or to incorporate native regulatory elements.
- *Targeted, multi-tissue delivery.* Our ctLNP delivery system has been engineered to use biological ligands to reach receptors in a specific tissue.

- *Scale to reach millions of patients.* We believe the combination of the expected multi-year durability of a single dose of ceDNA, tissue-specific delivery and manufacturing capacity may provide dosing for millions of patients living with prevalent diseases.
- *A sustainable payer model.* Our cost-effective manufacturing process, combined with the potential to redose patients as needed to extend expression, may allow payers to better predict clinical outcomes and, as a result, to cover our therapies within the current reimbursement paradigm.

**Our portfolio**

We are advancing a broad and expansive portfolio, including eight programs for rare and prevalent diseases of the liver and retina. We are focused on diseases with significant unmet need for which our non-viral gene therapy platform may substantially improve clinical efficacy relative to current gene therapy approaches. We are prioritizing rare monogenic diseases of the liver and retina that have well-established biomarkers and clear clinical and regulatory pathways. We plan to expand our portfolio by pursuing additional programs in rare and prevalent diseases of the liver and retina, as well as in skeletal muscle, the CNS and oncology by developing discrete ctLNPs, each engineered to reach a different tissue.

We believe our non-viral gene therapy platform may allow patients to produce antibody therapies from their own cells for years at a time from a single dose, and plan to advance antibody gene therapy programs across multiple therapeutic areas.



\* PREVALENCE DATA ARE APPROXIMATE

Over the course of [redacted], we expect to obtain additional preclinical *in vivo* data and to identify development candidates for phenylketonuria, or PKU, and hemophilia A, positioning us to undertake studies enabling investigational new drug, or IND, applications for these programs in [redacted] and to submit IND applications for these programs in [redacted]. We anticipate submitting IND applications for additional programs in [redacted] and beyond.

**Liver diseases**

For the majority of our liver programs, we have designed and manufactured disease-modifying ceDNAs that have shown expression *in vitro* and/or disease correction *in vivo*. We have employed GalNAc, a ligand that binds

to the asialoglycoprotein receptor on liver cells called hepatocytes, because the biology of this selective ligand-receptor pair for delivery to hepatocytes has been well-validated in human clinical trials. GalNAc targeting has been demonstrated to efficiently deliver nucleic acid payloads to up to 100% of hepatocytes, and we believe that broad biodistribution of ctLNP-GalNAc to hepatocytes will be a key strength of our pipeline programs since current gene therapy approaches deliver payload to 30% or fewer hepatocytes with a single dose. Additionally, our potential to redose patients until they are in the therapeutic range of expression may enable early and robust human proof of concept in early clinical trials and is a key differentiator from current gene therapy approaches.

Our most advanced liver disease programs include:

- *Phenylketonuria*, or PKU, is a rare autosomal recessive genetic disorder caused by deficiency of the hepatic enzyme phenylalanine hydroxylase, which results in metabolic abnormalities and neurocognitive deficits. PKU affects approximately 15,000 individuals in the United States and 41,000 individuals in the European Union. Our approach seeks to achieve sustained metabolic correction for patients of all ages upon initial dose or through individualized patient dose titration, normalizing their diet, eliminating the burden of ongoing treatment and stabilizing and/or preserving neurocognitive function.
- *Hemophilia A* is a rare X-linked hereditary bleeding disorder characterized by impaired blood coagulation as a result of deficiency in the production or function of coagulation Factor VIII that affects approximately 16,000 individuals in the United States and 320,000 individuals worldwide. Our approach aims to achieve therapeutic Factor VIII levels in patients of all ages resulting in normalization of bleeding risk to prevent irreversible tissue and organ dysfunction.

#### *Retinal diseases*

Approximately 200 million individuals suffer from inherited retinal diseases, in which a gene mutation leads to degeneration of the retina. Many of these diseases are caused by genes too large to be enclosed within AAV, including several types of Leber's Congenital Amaurosis, or LCA10, and Stargardt disease. We believe using ceDNA to deliver large gene payload efficiently and specifically to relevant cell types in the retina by minimally invasive routes represents an important therapeutic approach.

Our most advanced retinal disease programs include:

- *LCA10* is the most common genetic cause of childhood vision loss and affects approximately 2,200 individuals in the United States and 3,400 individuals in the European Union. Our approach may deliver the full gene to photoreceptors to correct the full spectrum of mutations with a single ceDNA construct in order to halt visual decline and restore vision.
- *Stargardt disease* is the most common inherited macular dystrophy and affects approximately 37,000 individuals in the United States and 66,000 individuals in the European Union. Our approach aims to deliver the full gene to photoreceptors and retinal pigment epithelial cells in order to halt disease progression and preserve vision.

#### **Our strategy**

Our goal is to become an industry leader in the field of genetic medicine, advancing our non-viral gene therapy platform to discover, develop, manufacture and globally commercialize a new class of gene therapy that is



durable, redosable and specifically deliverable to a range of tissues for the treatment of diseases caused by single, large or multiple gene defects. Key components of our strategy are to:

- Establish ceDNA as a new class of gene therapy, demonstrating its potential across rare monogenic diseases of the liver and retina;
- Leverage our non-viral gene therapy platform to advance additional product candidates for diseases of the liver and retina and to expand quickly into additional tissues;
- Expand manufacturing scale to access previously unattainable markets for gene therapy;
- Leverage our eight-week research cycle to rapidly design, produce and screen ceDNA constructs in order to evaluate disease correction;
- Expand patient access to our non-viral gene therapy through a high-value network of alliances and collaborations; and
- Build a sustainable leadership position in non-viral gene therapy as a fully integrated innovative biotechnology company.

Our organization is composed of more than 85 talented individuals with significant experience across discovery, preclinical research, manufacturing and clinical development. Our research and development efforts have resulted in numerous innovations and breakthroughs across every aspect of our platform. We have filed patent applications and taken other steps to protect our proprietary position with respect to these innovations and breakthroughs. Our wholly owned intellectual property, combined with licenses to background technology from our co-founder's prior work at the National Institutes of Health and the University of Massachusetts Medical School, supports the leading position of our platform and provides a strong foundation for its continued advancement.

To fund our operations, we have raised approximately \$227 million from investors, premier venture capitalists and institutional investors, including Atlas Venture, Fidelity, T. Rowe Price, Invus, Farallon, Wellington, Deerfield, Casdin Capital, Foresight Capital and Leerink Partners.

#### ***Risks associated with our business***

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk factors" section of this prospectus. These risks include, but are not limited to, the following:

- We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts;
- Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability;
- We are very early in our development efforts. We have not identified any product candidates for IND-enabling studies or clinical development. As a result it will be many years before we commercialize a product candidate, if ever. If we are unable to identify and advance product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them, or experience significant delays in doing so, our business will be materially harmed;

- We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, any of which could prevent us from commercializing any product candidates we may develop on a timely basis, if at all;
- Our non-viral gene therapy platform is based on novel technologies that are unproven, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval, if at all;
- If any product candidates we may develop cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval;
- The outcome of preclinical studies may not be predictive of later preclinical studies or clinical trials;
- Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate we may develop, or the approval may be for a more narrow indication than we expect;
- The manufacture of genetic medicine products is complex and difficult and is subject to a number of scientific and technical risks, some of which are common to the manufacture of drugs and biologics and others of which are unique to the manufacture of gene therapies. We could experience manufacturing problems that result in delays in our development or commercialization programs;
- We rely, and expect to continue to rely, on third parties to conduct some or all aspects of our product manufacturing, research, preclinical and clinical testing, and these third parties may not perform satisfactorily;
- If we fail to comply with our obligations under our existing license agreements, or under any future intellectual property licenses, or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose intellectual property rights that are important to our business;
- If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize any product candidates we may develop and technology may be adversely affected; and
- The ongoing COVID-19 pandemic and its effects on our business and operations are uncertain. We and our contract development and manufacturing organizations and contract research organizations have experienced a reduction in the capacity to undertake research scale production and to execute some preclinical studies, and we may face disruptions that affect our ability to initiate and complete preclinical studies and to procure items that are essential for our research and development activities.

### **Our corporate information**

We were incorporated under the laws of the state of Delaware on October 21, 2016 under the name Torus Therapeutics, Inc. On November 17, 2017 we changed our name to Generation Bio Co. Our principal executive offices are located at 301 Binney Street, Cambridge, MA 02142 and our telephone number is (617) 655-7500. Our website address is <http://www.generationbio.com>. The information contained on, or accessible through, our website does not constitute part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

### **Implications of being an emerging growth company**

We are an “emerging growth company” as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. As a result, we may take advantage of reduced reporting requirements that are otherwise applicable generally to public companies, including delaying auditor attestation of internal control over financial reporting, exemption from the requirements to hold non-binding advisory votes on executive compensation and golden parachute payments, providing only two years of audited financial statements and related Management’s discussion and analysis of financial condition and results of operations in this prospectus and reducing executive compensation disclosures.

We may remain an emerging growth company until the end of 2025. We will cease to be an emerging growth company prior to the end of 2025 if we become a “large accelerated filer,” our annual gross revenue exceeds \$1.07 billion, or we issue more than \$1.0 billion of non-convertible debt in any three-year period.

We have elected to take advantage of certain of the reduced disclosure obligations in the registration statement of which this prospectus is a part. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an emerging growth company. In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. We have elected not to “opt out” of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we can adopt the new or revised standard at the time private companies adopt the new or revised standard and may do so until such time that we either (1) irrevocably elect to “opt out” of such extended transition period or (2) no longer qualify as an emerging growth company.

## The offering

|  |  |
|--|--|
| Common stock offered by us                         | shares   |
| Common stock to be outstanding after this offering | shares   |
| Option to purchase additional shares               | We have granted the underwriters an option for a period of 30 days from the date of this prospectus to purchase up to additional shares of our common stock.   |
| Use of proceeds                                    | <p>We estimate that the net proceeds from this offering will be approximately \$ million (or approximately \$ million if the underwriters exercise their option to purchase additional shares in full), based on an assumed initial public offering price of \$ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.</p> <p>We intend to use the net proceeds to us from this offering, together with our existing cash and cash equivalents, for continued research and development of our programs, including preclinical and IND-enabling studies; continued development and enhancement of our platform technologies; and for working capital and other general corporate purposes. See "Use of proceeds" for more information.</p> |
| Risk factors                                       | You should read the "Risk factors" section of this prospectus beginning on page 12 for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.  |
| Proposed Nasdaq Global Market symbol               | "GBIO"   |

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The number of shares of our common stock to be outstanding after this offering is based on 12,383,404 shares of our common stock outstanding as of March 31, 2020, which includes 2,543,922 shares of unvested restricted stock subject to repurchase by us, and after giving effect to the conversion of all outstanding shares of our preferred stock into an aggregate of 47,856,346 shares of common stock upon the closing of this offering, and excludes:

- 8,894,975 shares of common stock issuable upon exercise of stock options outstanding as of March 31, 2020, under our 2017 Stock Incentive Plan, as amended, or the 2017 Plan, at a weighted-average exercise price of \$2.77 per share;

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- 1,669,606 shares of common stock available for future issuance as of March 31, 2020 under our 2017 Plan, which shares will become available for issuance under our 2020 Stock Incentive Plan, or the 2020 Plan, at the time our 2020 Plan becomes effective; and
- and additional shares of common stock that will become available for issuance under our 2020 Plan and our 2020 Employee Stock Purchase Plan, respectively, each of which will become effective immediately prior to the effectiveness of the registration statement of which this prospectus is a part, as well as any automatic increases in the number of shares of common stock reserved for future issuance under these plans.

Unless otherwise indicated, all information in this prospectus assumes:

- the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 47,856,346 shares of our common stock upon the closing of the offering;
- no exercise of the outstanding options described above;
- no exercise by the underwriters of their option to purchase additional shares of our common stock; and
- the filing and effectiveness of our restated certificate of incorporation and the adoption of our amended and restated bylaws upon the closing of this offering.

## Summary consolidated financial data

You should read the following summary consolidated financial data, together with our consolidated financial statements and the related notes appearing elsewhere in this prospectus and the “Selected consolidated financial data” and “Management’s discussion and analysis of financial condition and results of operations” sections of this prospectus. We have derived the following consolidated statement of operations data for the years ended December 31, 2018 and 2019 and the consolidated balance sheet data as of December 31, 2019 from our audited consolidated financial statements appearing elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that may be expected in the future.

|  | Year ended December 31, |             |
|--|-------------------------|-------------|
|  | 2018                    | 2019        |
| <b>(in thousands, except per share data)</b>   |                         |             |
| <b>Consolidated statement of operations data:</b>                                      |                         |             |
| Revenue  | \$ 36                   | \$ —        |
| Operating expenses:  |                         |             |
| Research and development   | 28,152                  | 50,134      |
| General and administrative   | 9,178                   | 12,168      |
| Total operating expenses   | 37,330                  | 62,302      |
| Loss from operations   | (37,294)                | (62,302)    |
| Other income (expense):  |                         |             |
| Interest income and other income (expense), net  | 1,491                   | 985         |
| Net loss and net loss attributable to common stockholders                              | \$ (35,803)             | \$ (61,317) |
| Net loss per share attributable to common stockholders, basic and diluted              | \$ (6.05)               | \$ (7.34)   |
| Weighted average common shares outstanding, basic and diluted                          | 5,918                   | 8,357       |
| Pro forma net loss per share attributable to common stockholders, basic and diluted(1) |                         | \$ (1.76)   |
| Pro forma weighted average common shares outstanding, basic and diluted(1)             |                         | 34,783      |

(1) See Notes 2 and 12 to our consolidated financial statements appearing elsewhere in this prospectus for details on the calculation of unaudited pro forma net loss per share attributable to common stockholders.

|   | As of December 31, 2019 |              |                          |
|---|-------------------------|--------------|--------------------------|
|   | Actual                  | Pro forma(2) | Pro forma as adjusted(3) |
| <b>(in thousands)</b>                   |                         |              |                          |
| <b>Consolidated balance sheet data:</b> |                         |              |                          |
| Cash and cash equivalents               | \$ 15,076               | \$           | \$                       |
| Working capital(1)                      | 8,998                   |              |                          |
| Total assets                            | 42,140                  |              |                          |
| Convertible preferred stock             | 115,593                 |              |                          |
| Total stockholders’ equity (deficit)    | (98,592)                |              |                          |

(1) We define working capital as current assets less current liabilities.

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- (2) The pro forma consolidated balance sheet data give effect to the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 47,856,346 shares of common stock upon the closing of this offering.
- (3) The pro forma as adjusted balance sheet data give further effect to our issuance and sale of \_\_\_\_\_ shares of our common stock in this offering at an assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The pro forma as adjusted information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. A \$1.00 increase (decrease) in the assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by \$ \_\_\_\_\_ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, working capital, total assets and total stockholders' equity by \$ \_\_\_\_\_ million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

## Risk factors

*Investing in our common stock involves a high degree of risk. You should carefully consider the risks and uncertainties described below together with all of the other information contained in this prospectus, including our consolidated financial statements and the related notes appearing elsewhere in prospectus, before deciding to invest in our common stock. The risks described below are not the only risks facing our company. The occurrence of any of the following risks, or of additional risks and uncertainties not presently known to us or that we currently believe to be immaterial, could cause our business, prospects, operating results and financial condition to suffer materially. In such event, the trading price of our common stock could decline, and you might lose all or part of your investment.*

### **Risks related to our financial position and need for additional capital**

***We have incurred significant losses since our inception, have no products approved for sale and we expect to incur losses over the next several years.***

Since inception, we have incurred significant operating losses. Our net losses were \$35.8 million for the year ended December 31, 2018 and \$61.3 million for the year ended December 31, 2019. As of December 31, 2019, we had an accumulated deficit of \$108.5 million. To date, we have financed our operations with the proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017) and the sale of convertible preferred stock. We have devoted substantially all of our financial resources and efforts to research and development. We are still in the early stages of development of our product candidates, and we have not commenced or completed clinical development. We expect to continue to incur significant expenses and operating losses over the next several years. Our operating expenses and net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase substantially as we:

- continue our current research programs and conduct additional research programs;
- advance any product candidates we identify through our research programs into preclinical and clinical development;
- expand the capabilities of our proprietary non-viral gene therapy platform;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- hire additional clinical, regulatory and scientific personnel;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates we may develop for which we may obtain regulatory approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development, future commercialization efforts and operations as a public company.

Even if we obtain regulatory approval of and are successful in commercializing one or more of any product candidates we may develop, we will continue to incur substantial research and development and other



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expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue.

***We have never generated revenue from product sales and may never achieve or maintain profitability.***

We have not initiated clinical development of any product candidate and expect that it will be many years, if ever, before we have a product candidate ready for commercialization. To become and remain profitable, we must succeed in developing, obtaining the necessary regulatory approvals for and eventually commercializing a product or products that generate significant revenue. The ability to achieve this success will require us to be effective in a range of challenging activities, including:

- identifying product candidates and completing preclinical and clinical development of any product candidates we may identify;
- obtaining regulatory approval for any product candidates we may develop;
- manufacturing, marketing and selling any products for which we may obtain regulatory approval;
- achieving market acceptance of any product candidates we may develop for which we obtain regulatory approval as a viable treatment option; and
- satisfying any post-marketing requirements.

We may never succeed in these activities and, even if we do, may never generate revenues that are significant enough to achieve profitability. We are currently only in the preclinical stage of our research programs. Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve profitability.

Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

***We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.***

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we identify, continue the research and development of, initiate preclinical testing and clinical trials of and potentially seek marketing approval for any product candidates we may develop. In addition, if we obtain marketing approval for any product candidates we may develop, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed, on attractive terms or at all, we may be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

As of December 31, 2019, we had cash and cash equivalents of \$15.1 million. We believe that the net proceeds from this offering, together with our existing cash and cash equivalents, including \$111.5 million of gross

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proceeds we raised in our series C preferred stock financing in January 2020, will enable us to fund our operating expenses and capital expenditure requirements through . However, we have based this estimate on assumptions that may prove to be wrong, and our operating plan may change as a result of many factors currently unknown to us. As a result, we could deplete our capital resources sooner than we currently expect and could be forced to seek additional funding sooner than planned.

Our future capital requirements will depend on many factors, including:

- the identification of additional research programs and additional product candidates;
- the scope, progress, costs and results of preclinical and clinical development for any product candidates we may develop;
- the costs, timing and outcome of regulatory review of any product candidates we may develop;
- the cost and timing of completion of commercial-scale manufacturing activities;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidates we may develop for which we receive marketing approval;
- the costs and scope of the continued development of our non-viral gene therapy platform;
- the costs of satisfying any post-marketing requirements;
- the revenue, if any, received from commercial sales of product candidates we may develop for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting applications for patents, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims, including claims of infringement, misappropriation or other violations of third-party intellectual property;
- the costs of operational, financial and management information systems and associated personnel;
- the associated costs in connection with any acquisition of in-licensed products, intellectual property and technologies; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, even if we successfully identify and develop product candidates and those are approved, we may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if at all, and such revenues may not be sufficient to sustain our operations. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize any product candidates. We cannot be certain that additional funding will be available on acceptable terms, or at all. We have no committed source of additional capital and, if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or other research and development initiatives. We could be required to seek collaborators for product candidates we may develop at an earlier stage than otherwise would be desirable or on terms that are

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less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to product candidates we may develop in markets where we otherwise would seek to pursue development or commercialization ourselves.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate we may develop, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations. Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

***Raising additional capital may cause dilution to our stockholders, including purchasers of our common stock in this offering, restrict our operations or require us to relinquish rights to our technologies or product candidates.***

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, selling or licensing our assets, making capital expenditures, declaring dividends or encumbering our assets to secure future indebtedness.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or other arrangements when needed or on terms acceptable to us, we would be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

***Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.***

We commenced operations in 2016, and our operations to date have been limited to organizing and staffing our company, business planning, raising capital, conducting research activities and filing and prosecuting patent applications. All of our research programs are still in the research or preclinical stage of development, and their risk of failure is high. We have not yet demonstrated our ability to initiate or complete any clinical trials, obtain marketing approvals, manufacture a commercial scale product or arrange for a third party to do so on our behalf, or conduct sales, marketing and distribution activities necessary for successful product commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing genetic medicine products.

Our limited operating history, particularly in light of the rapidly evolving genetic medicine field, may make it difficult to evaluate our technology and industry and predict our future performance. Our limited history as an operating company makes any assessment of our future success or viability subject to significant uncertainty. We will encounter risks and difficulties frequently experienced by early stage companies in rapidly evolving fields. If we do not address these risks successfully, our business will suffer.

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In addition, as our business grows, we may encounter unforeseen expenses, restrictions, difficulties, complications, delays and other known and unknown factors. We will need to transition at some point from a company with a research focus to a company capable of conducting development activities and then to a company supporting commercial activities. We may not be successful in such transitions.

### ***Our ability to utilize our net operating loss carryforwards may be subject to limitations.***

We have a history of cumulative losses and anticipate that we will continue to incur significant losses in the foreseeable future; thus, we do not know whether or when we will generate taxable income necessary to utilize our net operating losses, or NOLs, or research and development tax credit carryforwards. As of December 31, 2019, we had federal NOLs of \$91.9 million and state NOLs of \$90.6 million.

In general, under Sections 382 and 383 of the U.S. Internal Revenue Code of 1986, as amended, or the Code, and corresponding provisions of state law, a corporation that undergoes an “ownership change,” generally defined as a greater than 50 percentage point change (by value) in its equity ownership by certain stockholders over a three-year period, is subject to limitations on its ability to utilize its pre-change NOLs and research and development tax credit carryforwards to offset future taxable income. We have not conducted a study to assess whether any such ownership changes have occurred. We may have experienced such ownership changes in the past and may experience such ownership changes in the future as a result of this offering or of subsequent changes in our stock ownership (which may be outside our control). As a result, if, and to the extent that, we earn net taxable income, our ability to use our pre-change NOLs and research and development tax credit carryforwards to offset such taxable income may be subject to limitations.

There is also a risk that due to regulatory changes, such as suspensions on the use of NOLs, or other unforeseen reasons, our existing NOLs could expire or otherwise become unavailable to offset future income tax liabilities. As described below in “Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition,” the Tax Cuts and Jobs Act, or the Tax Act, as amended by the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, includes changes to U.S. federal tax rates and the rules governing NOL carryforwards that may significantly impact our ability to utilize our NOLs to offset taxable income in the future. In addition, state NOLs generated in one state cannot be used to offset income generated in another state. For these reasons, even if we attain profitability, we may be unable to use a material portion of our NOLs and other tax attributes.

## **Risks related to discovery and development**

***We are very early in our development efforts. We have not identified any product candidates for IND-enabling studies or clinical development and as a result it will be years before we commercialize a product candidate, if ever. If we are unable to identify and advance product candidates through preclinical studies and clinical trials, obtain marketing approval and ultimately commercialize them, or experience significant delays in doing so, our business will be materially harmed.***

We are very early in our development efforts and have invested our research efforts to date in developing our platform. We have a portfolio of programs, including those listed in the “Business—Our pipeline” section of this prospectus, that are in early stages of preclinical development and have not identified any product candidates for IND-enabling studies or clinical development. We may never identify any product candidates or advance to clinical-stage development. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of our product candidates, which may never occur. We currently generate no revenue from sales of any product, and we may never be able to develop or commercialize a marketable product.

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Commencing clinical trials in the United States is subject to acceptance by the U.S. Food and Drug Administration, or FDA, of an investigational new drug application, or IND, and finalizing the trial design based on discussions with the FDA and other regulatory authorities. In the event that the FDA requires us to complete additional preclinical studies or we are required to satisfy other FDA requests prior to commencing clinical trials, the start of our first clinical trials may be delayed. Even after we receive and incorporate guidance from these regulatory authorities, the FDA or other regulatory authorities could disagree that we have satisfied their requirements to commence any clinical trial or change their position on the acceptability of our trial design or the clinical endpoints selected, which may require us to complete additional preclinical studies or clinical trials or impose stricter approval conditions than we currently expect. There are equivalent processes and risks applicable to clinical trial applications in other countries, including countries in the European Union.

Commercialization of any product candidates we may develop will require preclinical and clinical development; regulatory and marketing approval in multiple jurisdictions, including by the FDA and the European Medicines Agency, or EMA; obtaining manufacturing supply, capacity and expertise; building of a commercial organization; and significant marketing efforts. The success of product candidates we may identify and develop will depend on many factors, including the following:

- timely and successful completion of preclinical studies, including toxicology studies, biodistribution studies and minimally efficacious dose studies in animals, where applicable;
- effective INDs or comparable foreign applications that allow commencement of our planned clinical trials or future clinical trials for any product candidates we may develop;
- successful enrollment and completion of clinical trials, including under the FDA's current Good Clinical Practices, or GCPs, current Good Laboratory Practices, or cGLPs, and any additional regulatory requirements from foreign regulatory authorities;
- positive results from our future clinical programs that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended populations of any product candidates we may develop;
- receipt of marketing approvals from applicable regulatory authorities;
- establishment of arrangements through our own facilities or with third-party manufacturers for clinical supply and, where applicable, commercial manufacturing capabilities;
- establishment, maintenance, defense and enforcement of patent, trademark, trade secret and other intellectual property protection or regulatory exclusivity for any product candidates we may develop;
- commercial launch of any product candidates we may develop, if approved, whether alone or in collaboration with others;
- acceptance of the benefits and use of our product candidates we may develop, including method of administration, if and when approved, by patients, the medical community and third-party payers;
- effective competition with other therapies;
- maintenance of a continued acceptable safety, tolerability and efficacy profile of any product candidates we may develop following approval; and
- establishment and maintenance of healthcare coverage and adequate reimbursement by payers.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize any product candidates we may develop, which would

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materially harm our business. If we are unable to advance our product candidates to clinical development, obtain regulatory approval and ultimately commercialize our product candidates, or experience significant delays in doing so, our business will be materially harmed.

***We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing any product candidates we determine to develop on a timely basis, if at all.***

The risk of failure for any product candidates we determine to develop is high. It is impossible to predict when or if any product candidate would prove effective or safe in humans or will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of product candidates in humans. We have not yet begun or completed a clinical trial of any product candidate. Clinical trials may fail to demonstrate that our product candidates are safe for humans and effective for indicated uses. Even if the clinical trials are successful, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application may cause delays in the approval or rejection of an application.

Before we can commence clinical trials for a product candidate, we must complete extensive preclinical testing and studies that support our INDs and other regulatory filings. We cannot be certain of the timely completion or outcome of our preclinical testing and studies and cannot predict if the FDA will accept our proposed clinical programs or if the outcome of our preclinical testing and studies will ultimately support the further development of any product candidates. As a result, we cannot be sure that we will be able to submit INDs for our preclinical programs on the timelines we expect, if at all, and we cannot be sure that submission of INDs will result in the FDA allowing clinical trials to begin. Furthermore, product candidates are subject to continued preclinical safety studies, which may be conducted concurrently with our clinical testing. The outcomes of these safety studies may delay the launch of or enrollment in future clinical trials and could impact our ability to continue to conduct our clinical trials.

Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. A failure of one or more clinical trials can occur at any stage of testing, which may result from a multitude of factors, including, but not limited to, flaws in trial design, dose selection issues, patient enrollment criteria and failure to demonstrate favorable safety or efficacy traits.

Identifying and qualifying patients to participate in clinical trials of any product candidates we may develop is critical to our success. We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our clinical trials in a timely manner. Patient enrollment and trial completion is affected by factors including:

- perceived risks and benefits of novel genetic medicine-based approaches;
- size of the patient population, in particular for rare diseases, and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease or disorder under investigation;
- proximity and availability of clinical trial sites for prospective patients;
- ability to obtain and maintain patient consent;

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- risk that enrolled patients will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would harm our business, financial condition, results of operations and prospects.

Other events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective clinical research organizations, or CLROs, and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board, or IRB, or independent ethics committee approval, or the equivalent review groups for sites outside the United States, at each clinical trial site;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;
- failure by us, any CLROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's GCPs;
- failure by physicians to adhere to delivery protocols leading to variable results;
- delays in the testing, validation, manufacturing and delivery of any product candidates we may develop to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- delays in having patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events associated with a product candidate in development by another company, which are viewed to outweigh its potential benefits, and which may negatively impact the perception of our product due to a similarity in technology or approach;
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols;
- changes in the legal or regulatory regimes domestically or internationally related to patient rights and privacy; or
- lack of adequate funding to continue the clinical trial.

Any inability to successfully complete preclinical studies and clinical trials could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and

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royalties. In addition, if we make manufacturing or formulation changes to any product candidates we may develop, we may need to conduct additional studies or trials to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize any product candidates we may develop or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize any product candidates we may develop and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of future clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with any product candidates we may develop, we may:

- be delayed in obtaining marketing approval for product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

***Genetic medicine is an emerging area of drug development that poses many scientific and other risks. We have only limited prior experience in genetic medicine research and manufacturing and no prior experience in genetic medicine clinical development. Our lack of experience for our genetic medicine programs may limit our ability to be successful or may delay our development efforts.***

Genetic medicine is an emerging field of drug development with only a small number of genetic medicines having received FDA or EMA approval to date. Our genetic medicine research programs are still at an early stage, and there remain several areas of drug development risk, which pose particular uncertainty for our programs given the relatively limited development history of, and our limited prior experience with, genetic medicines. Translational science, manufacturing materials and processes, safety concerns, regulatory pathway and clinical trial design and execution all pose particular risk to our drug development activities. Furthermore, the medical community's understanding of the genetic causes of many diseases continues to evolve and further research may change the medical community's views on what therapies and approaches are most effective for addressing certain diseases.

As an organization, we have not previously conducted any IND-enabling studies or clinical trials, including any later stage or pivotal clinical trials. In pursuing our new technologies, we have begun to establish our own genetic medicine technical capabilities, but we will need to continue to expand those capabilities by either hiring internally or seeking assistance from outside service providers. Genetic medicine is an area of significant investment by biotechnology and pharmaceutical companies and there may be a scarcity of talent available to us in these areas. If we are not able to expand our genetic medicine capabilities, we may not be able to develop in the way we intend or desire any promising product candidates that emerge from our program or our other collaborative genetic medicine sponsored research programs, which would limit our prospects for future



growth. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we may develop. Failure to commence or complete, or delays in, our clinical trials, could prevent us from or delay us in commercializing our product candidates.

We will need to build our internal and external capabilities in designing and executing a genetic medicine clinical trial. There are many known and unknown risks involved in translating preclinical development of gene therapies to clinical development, including selecting appropriate endpoints and dosage levels for dosing humans based on preclinical data. Furthermore, our genetic medicine programs are initially targeting rare diseases with relatively small populations, which limits the pool of potential subjects for our genetic medicine clinical trials. If we are unable to initiate and conduct our genetic medicine clinical trials in a manner that satisfies our expectations or regulatory requirements, the value of our genetic medicine programs may be diminished.

***Our non-viral gene therapy platform is based on novel technologies that are unproven, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval, if at all.***

We have concentrated our research and development efforts on our non-viral gene therapy platform, and our future success depends on the successful development of our platform.

However, the technologies that comprise our platform are new and largely unproven. These technologies have not been clinically tested and the scientific evidence to support the feasibility of developing product candidates based on those technologies is both preliminary and limited. Successful development of product candidates by us will require solving a number of issues, including the expansion of our cell-targeted lipid nanoparticle delivery system, or ctLNP, delivery system to tissues beyond the liver and retina and obtaining expression levels sufficient to address or ameliorate each target disease or indication. There can be no assurance we will be successful in solving any or all of these issues. We have concentrated our research efforts to date on developing the components of our platform, and our future success is highly dependent on the successful development of our ceDNA constructs, our ctLNP delivery system and therapeutic applications of these technologies. We may decide to alter or abandon our initial programs as new data become available and we gain experience in developing our therapeutics. We cannot be sure that our technologies will yield satisfactory products that are safe and effective, scalable or profitable in any indication we pursue.

There can be no assurance that any development problems we experience in the future related to our non-viral gene therapy platform will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience delays in developing a sustainable, reproducible and scalable manufacturing process or transferring that process to commercial partners, which may prevent us from initiating or conducting clinical trials or commercializing our products on a timely or profitable basis, if at all. In addition, the clinical trial requirements of the FDA, the EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. Only a small number of non-viral gene therapies have successfully reached the clinical trial phase of development, limiting insight into the regulatory review process for this field of genetic medicine. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals in either the United States or the European Union for any product candidates we may develop or how long it will take to commercialize any product candidate that receives marketing approval.

***If any product candidates we may develop cause undesirable side effects or have other unexpected adverse properties, such side effects or properties could delay or prevent regulatory approval, limit the commercial potential or result in significant negative consequences following any potential marketing approval.***

We have not evaluated any product candidates in human clinical trials. Moreover, there have been only a limited number of clinical trials involving the use of non-viral gene therapies and none involving ceDNA constructs or other technology similar to our technology. It is impossible to predict when or if any product candidates we may develop will prove safe in humans. In the genetic medicine field, there have been several significant adverse events from gene therapy treatments in the past, including reported cases of leukemia and death. There can be no assurance that our technologies will not cause undesirable side effects.

We use a ctLNP delivery system to deliver our ceDNA constructs. Lipid nanoparticles have been shown to induce necrosis in the liver at certain doses and induce infusion related reactions, as well as to initiate systemic inflammatory responses. While our ctLNPs are a new generation of LNP, there can be no assurance that our ctLNPs will not have undesired effects. Our ctLNPs could contribute, in whole or in part, to immune reactions, infusion reactions, complement reactions or antibody reactions. In addition, certain aspects of our non-viral gene therapies may induce immune reactions from the lipid as well as adverse reactions within liver pathways or degradation of the LNP into its component molecules or metabolites, any of which could lead to significant adverse events in one or more of our future clinical trials. Many of these types of side effects have been seen for LNPs. Once delivered to target cells, DNA-based payloads, such as those carried by our ceDNA constructs, may interact with host proteins or chromosomal DNA in the cell endosome, cytosol or nucleus.

Adeno-associated virus, or AAV, genomes have been shown in some cases to initiate intracellular immune activation, which can lead to transcriptional changes, and local tissue interferon responses, which may lead to immune infiltrates and tissue damage. AAV genetic material may also integrate into the host chromosome, which could contribute to modified cell function transformation. There may be uncertainty as to the underlying cause of any such adverse event, which would make it difficult to accurately predict side effects in future clinical trials and would result in significant delays in our programs.

If any product candidates we develop are associated with serious adverse events, undesirable side effects or unexpected characteristics, we may need to abandon their development or limit development to certain uses or subpopulations in which the serious adverse events, undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective, any of which would have a material adverse effect on our business, financial condition, results of operations and prospects. Many product candidates that initially showed promise in early stage testing for treating cancer or other diseases have later been found to cause side effects that prevented further clinical development of the product candidates.

If in the future we are unable to demonstrate that such side effects were caused by factors others than our product candidates, the FDA, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, any product candidates for any or all targeted indications. Even if we are able to demonstrate that any future serious adverse events are not product-related, and regulatory authorities do not order us to cease further development of our product candidates, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any product candidate, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

Regulatory approval of and/or demand for our potential products will depend in part on public acceptance of the use of genetic medicine for the prevention or treatment of human diseases. Safety issues that might arise in

trials for gene therapies other than our own could adversely impact public attitudes towards our platform and product candidates notwithstanding that the gene therapies we are developing are non-viral.

There are a number of clinical trials of gene therapies ongoing. There is a potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry genetic material. Possible adverse side effects that may occur with treatment with gene therapy products include an immunologic reaction early after administration that could substantially limit the effectiveness of the treatment or represent safety risks for patients.

Any of these events could prevent us from achieving or maintaining market acceptance of any product candidates we may develop and could significantly harm our business, prospects, financial condition and results of operations.

***The outcome of preclinical studies and earlier-stage clinical trials may not be predictive of future results or the success of later preclinical studies and clinical trials.***

We are in the early stage of research in the development of our platform and have not identified any product candidates or conducted any IND-enabling studies or any clinical trials. As a result, our belief in the capabilities of our platform is based on early research and preclinical studies. However, the results of preclinical studies may not be predictive of the results of preclinical studies or clinical trials, and the results of any early-stage clinical trials may not be predictive of the results of later clinical trials. In addition, initial success in clinical trials may not be indicative of results obtained when such trials are completed. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products. Our future clinical trials may not ultimately be successful or support further clinical development of any product candidates we may develop. There is a high failure rate for product candidates proceeding through clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in clinical development even after achieving encouraging results in earlier studies. Any such setbacks in our clinical development could materially harm our business and results of operations.

***We may not be successful in our efforts to identify, discover or develop potential product candidates.***

The success of our business depends primarily upon our ability to identify, develop and commercialize product candidates based on our non-viral gene therapy platform. All of our product development programs are still in the research or preclinical stage of development. Our research programs may fail to identify potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates, our potential product candidates may be shown to have harmful side effects in preclinical *in vitro* experiments or animal model studies, they may not show promising signals of therapeutic effect in such experiments or studies or they may have other characteristics that may make the product candidates impractical to manufacture, unmarketable or unlikely to receive marketing approval.

In addition, although we believe our platform will position us to rapidly expand our portfolio of programs beyond our current programs, we have not yet successfully developed any product candidate and our ability to expand our portfolio may never materialize. The process by which we identify and disclose product candidates may fail to yield product candidates for clinical development for a number of reasons, including those discussed in these risk factors and also:

- we may not be able to assemble sufficient resources to acquire or discover product candidates;
- competitors may develop alternatives that render our potential product candidates obsolete or less attractive;

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- potential product candidates we develop may nevertheless be covered by third parties' patents or other intellectual property rights;
- potential product candidates may, on further study, be shown to have harmful side effects, toxicities or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance;
- potential product candidates may not be effective in treating their targeted diseases or disorders;
- the market for a potential product candidate may change so that the continued development of that product candidate is no longer reasonable;
- a potential product candidate may not be capable of being produced in commercial quantities at an acceptable cost, or at all; or
- the regulatory pathway for a potential product candidate is too complex and difficult to navigate successfully or economically.

If we are unable to identify and discover suitable product candidates for clinical development, this would adversely impact our business strategy and our financial position and share price and could potentially cause us to cease operations.

***The genetic medicine field is relatively new and evolving rapidly. We are focusing our research and development efforts on our non-viral gene therapy platform, but other gene therapy technologies may be discovered that provide significant advantages over our platform, which could materially harm our business.***

To date, we have focused our efforts on the advancement of our non-viral gene therapy platform, which is designed to overcome the limitations of current viral gene therapy approaches. However, while these modalities have demonstrated their limitations, there are many companies that are developing new genetic medicines, including viral gene therapies, gene editing and messenger RNA, or mRNA. There can be no certainty that these companies will not develop genetic medicines that address some of these limitations and will be considered to have advantages over our non-viral gene therapy platform. For example, in December 2019, Dyno Therapeutics announced a new technique for AAV delivery, labeled BRAVE, that allows the researchers to engineer the virus shell to deliver the gene package to the exact cell type in the body they intend to treat. This new method may reduce concerns about off-target AAV delivery and make it a more attractive delivery system.

***We may expend our limited resources to pursue a particular program, product candidate or indication and fail to capitalize on programs, product candidates or indications that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited financial and managerial resources, we focus on research programs and expect to focus on product candidates that we identify for specific indications among many potential options. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential, or we may choose to focus our efforts and resources on a potential product candidate that ultimately proves to be unsuccessful. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable medicines. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Any such event could have a material adverse effect on our business, financial condition, results of operations and prospects.

***Clinical trial and product liability lawsuits against us could divert our resources, could cause us to incur substantial liabilities and could limit commercialization of any product candidates we may develop.***

We will face an inherent risk of clinical trial and product liability exposure related to the testing of any product candidates we may develop in clinical trials, and we will face an even greater risk if we commercially sell any products that we may develop. While we currently have no product candidates in clinical trials or that have been approved for commercial sale, the future use of product candidates by us in clinical trials, and the sale of any approved products in the future, may expose us to liability claims. These claims might be made by patients that use the product, healthcare providers, pharmaceutical companies or others selling such products. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend any related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any product candidates we may develop.

We will need to increase our insurance coverage if we commence clinical trials or if we commence commercialization of any product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise. If a successful clinical trial or product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

## **Risks relating to manufacturing**

***The manufacture of genetic medicine products is complex and difficult and is subject to a number of scientific and technical risks, some of which are common to the manufacture of drugs and biologics and others of which are unique to the manufacture of gene therapies. We could experience manufacturing problems that result in delays in our development or commercialization programs.***

Genetic medicine drug products are complex and difficult to manufacture. We have an established current Good Manufacturing Practices, or cGMP, -ready process at the 200-liter scale which we have successfully transferred to external contract development and manufacturing organizations, or CDMOs, to supply our gene therapies for IND-enabling preclinical studies and early clinical trials. We believe that we will be able to enter into arrangements with existing and/or additional CDMOs to provide commercial supply. We may also seek to eventually establish our own manufacturing facility for long-term commercial supply.

A number of factors common to the manufacturing of biologics and drugs could also cause production issues or interruptions for our gene therapies, including raw material or starting material variability in terms of quality, cell line viability, productivity or stability issues, shortages of any kind, shipping, distribution, storage and supply chain failures, growth media contamination, equipment malfunctions, operator errors, facility contamination, labor problems, natural disasters, disruption in utility services, terrorist activities, or acts of god that are beyond our or our contract manufacturer's control. It is often the case that early stage process development is conducted with materials that are not manufactured using cGMP starting materials, techniques or processes and which are not subject to the same level of analysis that would be required for clinical grade

material. We may encounter difficulties in translating the manufacturing processes used to produce research grade materials to cGMP compliant processes, and any changes in the manufacturing process may affect the safety and efficacy profile of our product candidates.

Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage. Some of the raw materials that we anticipate will be required in our manufacturing process, such as Sf9 cells, are derived from biologic sources. Such raw materials may be difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of any product candidates we may develop could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially harm our development timelines and our business, financial condition, results of operations and prospects.

***Our non-viral gene therapy platform is novel, and the combination of novel constructs with untested scaling may cause us to experience delays in satisfying regulatory authorities or production problems that result in delays in our development or commercialization programs, limit the supply of any product candidates we may develop or otherwise harm our business.***

Our non-viral gene therapy platform is novel and the manufacture of products on the basis of our platform is untested at a large scale. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims, insufficient inventory or potentially delay progression of our preclinical or clinical development of any product candidates we may develop. If we successfully develop product candidates, we may encounter problems achieving adequate quantities and quality that meet FDA, EMA or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs. The ability to scale our manufacturing and maintain the manufacturing process at the same levels of quality and efficacy that we are currently manufacturing is yet to be tested. If we or our CDMOs are unable to scale our manufacturing at the same levels of quality and efficiency, we may not be able to supply the required number of doses for clinical trials or commercial supply, and our business could be harmed.

***Manufacturing the ctLNP component of a potential product candidate may be complex and difficult, and we could experience delays in satisfying regulatory authorities or production problems that result in delays in our development or commercialization, limit the supply of any product candidates we may develop or otherwise harm our business.***

Many product candidates we may develop will require the manufacture of the ctLNP component, which may require processing steps that are more complex than those required for current products that utilize LNPs. In order to manufacture ctLNPs that are specialized for a given platform program, we may need to add biologic ligands to existing LNPs. This process is challenging and may pose a risk to our ability to manufacture on a scale sufficient to meet clinical and commercial needs.

***Testing of and changes to methods of product candidate manufacturing or formulation may result in additional costs or delay.***

As product candidates proceed through preclinical studies to clinical trials towards potential approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are tested and then altered along the way in an effort to optimize processes and results. Such changes carry the risk that they will not achieve these intended objectives.

An important part of the manufacturing of our potential product candidates is analytical testing. Analytical testing of gene therapies involves tests that are more numerous, more complex in scope and take a longer time

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to develop and to conduct as compared to traditional drugs. We and our CDMOs may need to expend considerable time and resources to develop assays and other analytical tests for our product candidates, including assays to assess the potency of our product candidates. Some assays may need to be outsourced to specialized testing laboratories. Even when assays are developed, they may need to be further tested, qualified and validated, which may take substantial time and resources. Because of the lagging nature of analytical testing, we may proceed with additional manufacturing and other development activities without having first fully characterized our manufactured materials. If the results of the testing fail to meet our expectations, we may need to delay or repeat certain manufacturing and development activities.

We may make changes to our manufacturing methods as part of our product development activities. Any such changes could cause any product candidates we may develop to perform differently and affect the results of clinical trials conducted with the materials manufactured using altered processes. Such changes may also require additional testing, FDA notification or FDA approval. This could delay completion of clinical trials, require the conduct of bridging clinical trials or the repetition of one or more clinical trials, increase clinical trial costs, delay approval of our product candidates and jeopardize our ability to commence sales and generate revenue.

In addition, the FDA, the EMA, and other regulatory authorities may require us to submit samples of any lot of and approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA, or other regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay product launches, which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

***We currently depend on a small number of third-party suppliers for our drug substance and drug product, and we expect to continue to depend on third-party suppliers for materials used in the manufacture of any product candidates we may develop, and the loss of these third-party suppliers or their inability to supply us with adequate materials, particularly those raw materials that are in short supply, could harm our business.***

We currently rely on a small number of third-party suppliers for our drug substance and drug product and expect to continue to rely on third-party suppliers for certain materials and components required for the production of any product candidates we may develop. Our dependence on these third-party suppliers and the challenges we may face in obtaining and maintaining adequate supplies of materials involve several risks, including limited control over pricing, availability and quality and delivery schedules. There is substantial demand and limited supply for certain of the raw materials used to manufacture genetic medicine products and these raw materials are usually sole-sourced, as there are a limited number of qualified suppliers. This limited supply, combined with any problems that may arise during the manufacturing process development, may create long lead times to manufacture or procure starting materials. The progress of our non-viral gene therapy platform is highly dependent on these suppliers providing us or our contract manufacturer with the necessary starting materials that meet our requirements in a timely manner. As a small company, our negotiation leverage is limited, and we are likely to get lower priority than our competitors that are larger than we are. We cannot be certain that our suppliers will continue to provide us with the quantities of these raw materials that we require or satisfy our anticipated specifications and quality requirements.

Any supply interruption in limited or sole-sourced raw materials could materially harm our ability to manufacture any product candidates we may develop until a new source of supply, if any, could be identified and qualified. We may be unable to find a sufficient alternative supply channel in a reasonable time or on commercially reasonable terms. Any performance failure on the part of our suppliers could delay the development and potential commercialization of any product candidates we may develop, including limiting

supplies necessary for clinical trials and regulatory approvals, which would have a material adverse effect on our business.

## **Risks related to our dependence on third parties**

***We rely, and expect to continue to rely, on third parties to conduct some or all aspects of our product manufacturing, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.***

We do not expect to independently conduct all aspects of our product manufacturing, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to many of these items, including CDMOs for the manufacturing of any product candidates we test in preclinical or clinical development, as well as contract research organizations, or CROs for the conduct of our animal testing and research. Any of these third parties may terminate their engagements with us at any time. If we need to enter into alternative arrangements, it could delay our product development activities. Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. For example, for product candidates that we develop and commercialize on our own, we will remain responsible for ensuring that each of our IND-enabling studies and clinical trials are conducted in accordance with the study plan and protocols.

Although we intend to design the clinical trials for any product candidates we may develop, CLROs will conduct some or all of the clinical trials. As a result, many important aspects of our development programs, including their conduct and timing, will be outside of our direct control. Our reliance on third parties to conduct future preclinical studies and clinical trials will also result in less direct control over the management of data developed through preclinical studies and clinical trials than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our preclinical studies and clinical trials and may subject us to unexpected cost increases that are beyond our control. If the CLROs and other third parties do not perform preclinical studies and future clinical trials in a satisfactory manner, breach their obligations to us or fail to comply with regulatory requirements, the development, regulatory approval and commercialization of any product candidates we may develop may be delayed, we may not be able to obtain regulatory approval and commercialize our product candidates or our development programs may be materially and irreversibly harmed. If we are unable to rely on preclinical and clinical data collected by our CLROs and other third parties, we could be required to repeat, extend the duration of or increase the size of any preclinical studies or clinical trials we conduct and this could significantly delay commercialization and require greater expenditures.

If third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical studies and clinical trials required to support future IND submissions and approval of any product candidates we may develop.



***We and our contract manufacturers are subject to significant regulation with respect to manufacturing our products. The manufacturing facilities on which we rely may not continue to meet regulatory requirements and have limited capacity.***

All entities involved in the preparation of therapeutics for clinical trials or commercial sale, including any contract manufacturers of any product candidates we may develop, are subject to extensive regulation. Components of a finished therapeutic product approved for commercial sale or used in late-stage clinical trials must be manufactured in accordance with cGMP. These regulations govern manufacturing processes and procedures (including record keeping) and the implementation and operation of quality systems to control and assure the quality of investigational products and products approved for sale. Poor control of production processes can lead to the introduction of adventitious agents or other contaminants or to inadvertent changes in the properties or stability of our product candidates that may not be detectable in final product testing. We or our contract manufacturer must supply all necessary documentation in support of a biologics license application, or BLA, on a timely basis and must adhere to the FDA's cGLP and cGMP regulations enforced through its facilities inspection program. Our facilities and quality systems and the facilities and quality systems of some or all of our third-party contractors must pass a pre-approval inspection for compliance with the applicable regulations as a condition of regulatory approval of any product candidates we may develop or any of our other potential products. In addition, the regulatory authorities may, at any time, audit or inspect a manufacturing facility involved with the preparation of our product candidates or our other potential products or the associated quality systems for compliance with the regulations applicable to the activities being conducted. If these facilities do not pass a pre-approval plant inspection, FDA approval of the products will not be granted.

The regulatory authorities also may, at any time following approval of a product for sale, audit our manufacturing facilities or those of our third-party contractors. If any such inspection or audit identifies a failure to comply with applicable regulations or if a violation of our product specifications or applicable regulations occurs independent of such an inspection or audit, we or the relevant regulatory authority may require remedial measures that may be costly and/or time-consuming for us or a third party to implement and that may include the temporary or permanent suspension of a clinical study or commercial sales or the temporary or permanent closure of a facility. Any such remedial measures imposed upon us or third parties with whom we contract could materially harm our business.

If we or any of our third-party manufacturers fail to maintain regulatory compliance, the FDA can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new drug product or biologic product, or revocation of a pre-existing approval. As a result, our business, financial condition and results of operations may be materially harmed.

Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms or at all if they are affiliated with our competitors;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities, particularly if they are under contract with our competitors;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

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Any of these events could lead to clinical trial delays or failure to obtain regulatory approval or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of production.

Additionally, if supply from one approved manufacturer is interrupted, there could be a significant disruption in supply. An alternative manufacturer would need to be qualified through a BLA supplement which could result in further delay. The regulatory agencies may also require additional studies or trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

These factors could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our products successfully. Furthermore, if our suppliers fail to meet contractual requirements, and we are unable to secure one or more replacement suppliers capable of production at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

***We may from time to time be dependent on single-source suppliers for some of the components and materials used in, and the processes required to develop, our development candidates and investigational medicines.***

We may from time to time depend on single-source suppliers for some of the components and materials used in, and manufacturing processes required to develop, our development candidates and investigational medicines. We cannot ensure that these suppliers or service providers will remain in business, have sufficient capacity or supply to meet our needs or that they will not be purchased by one of our competitors or another company that is not interested in continuing to work with us. Our use of single-source suppliers of raw materials, components, key processes and finished goods exposes us to several risks, including disruptions in supply, price increases or late deliveries. There are, in general, relatively few alternative sources of supply for substitute components. These vendors may be unable or unwilling to meet our future demands for our clinical trials or commercial sale. Establishing additional or replacement suppliers for these components, materials and processes could take a substantial amount of time and it may be difficult to establish replacement suppliers who meet regulatory requirements. Any disruption in supply from any single-source supplier or service provider could lead to supply delays or interruptions which would damage our business, financial condition, results of operations and prospects.

If we have to switch to a replacement supplier, the manufacture and delivery of our development candidates or investigational medicines could be interrupted for an extended period, which could adversely affect our business. Establishing additional or replacement suppliers for any of the components or processes used in our investigational medicines, if required, may not be accomplished quickly. If we are able to find a replacement supplier, the replacement supplier would need to be qualified and may require additional regulatory authority approval, which could result in further delay. While we seek to maintain adequate inventory of the single source components and materials used in our products, any interruption or delay in the supply of components or materials, or our inability to obtain components or materials from alternate sources at acceptable prices in a timely manner, could impair our ability to meet the demand for our investigational medicines.

***If we or any contract manufacturers and suppliers we engage fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.***

We and any contract manufacturers and suppliers we engage are subject to numerous federal, state and local environmental, health, and safety laws, regulations and permitting requirements, including those governing laboratory procedures; the generation, handling, use, storage, treatment and disposal of hazardous and

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regulated materials and wastes; the emission and discharge of hazardous materials into the ground, air and water; and employee health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biological and radioactive materials. Our operations also produce hazardous waste. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. Under certain environmental laws, we could be held responsible for costs relating to any contamination at third-party facilities. We also could incur significant costs associated with civil or criminal fines and penalties.

Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our research and product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws, regulations and permitting requirements. These current or future laws, regulations and permitting requirements may impair our research, development or production efforts. Failure to comply with these laws, regulations and permitting requirements also may result in substantial fines, penalties or other sanctions or business disruption, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Any third-party contract manufacturers and suppliers we engage will also be subject to these and other environmental, health and safety laws and regulations. Liabilities they incur pursuant to these laws and regulations could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

***We expect to rely on third parties to conduct, supervise and monitor IND-enabling studies and clinical studies, and if these third parties perform in an unsatisfactory manner, it may harm our business.***

We expect to rely on CROs and CLROs and research and clinical trial sites to ensure our IND-enabling studies and clinical trials are conducted properly and on time. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our CROs and CLROs' activities. Nevertheless, we will be responsible for ensuring that each of these studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs and CLROs does not relieve us of our regulatory responsibilities.

We and our CROs and CLROs will be required to comply with the FDA's GCPs for conducting, recording and reporting the results of IND-enabling studies and clinical trials to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. The FDA enforces these GCPs through periodic inspections of study sponsors, principal investigators and clinical trial sites. If we or our CROs or CLROs fail to comply with applicable GCPs, the preclinical and clinical data generated in our studies may be deemed unreliable and the FDA may require us to perform additional

studies before approving any marketing applications. Upon inspection, the FDA may determine that our studies did not comply with GCPs.

Our CLROs and CROs are not our employees, and we are therefore unable to directly monitor whether or not they devote sufficient time and resources to our clinical and nonclinical programs. These CLROs and CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our CROs or CLROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols or regulatory requirements, or for any other reasons, our studies may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize any product candidates we may develop. As a result, our financial results and commercial prospects would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

***We may enter into collaborations with third parties for the research, development and commercialization of certain of the product candidates we may develop. If any such collaborations are not successful, we may not be able to capitalize on the market potential of those product candidates.***

We may seek third-party collaborators for the research, development and commercialization of certain of the product candidates we may develop. If we enter into any such arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of any collaboration that we enter into.

Collaborations involving our research programs or any product candidates we may develop pose numerous risks to us, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not pursue development and commercialization of any product candidates we may develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;
- collaborators may delay programs, preclinical studies or clinical trials, provide insufficient funding for programs, preclinical studies or clinical trials, stop a preclinical study or clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with any product candidates we may develop if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- collaborators may be acquired by a third party having competitive products or different priorities;
- collaborators with marketing and distribution rights to one or more medicines may not commit sufficient resources to the marketing and distribution of such medicine or medicines;

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- collaborators may not properly obtain, maintain, enforce or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;
- disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our medicines or any product candidates we may develop or that result in costly litigation or arbitration that diverts management attention and resources;
- we may lose certain valuable rights under certain circumstances, including if we undergo a change of control;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates we may develop; and
- collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished or terminated.

If our collaborations do not result in the successful development and commercialization of product candidates, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this prospectus apply to the activities of our collaborators.

These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders, or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators, and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of several factors. If we license rights to any product candidates we or our collaborators may develop, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture.

***If conflicts arise between us and our collaborators or strategic partners, these parties may act in a manner adverse to us and could limit our ability to implement our strategies.***

If conflicts arise between our corporate or academic collaborators or strategic partners and us, the other party may act in a manner adverse to us and could limit our ability to implement our strategies. Some of our academic collaborators are conducting multiple product development efforts within each area that is the subject of the collaboration with us. Our collaborators or strategic partners, however, may develop, either alone or with others, products in related fields that are competitive with the product candidates we may develop that are the subject of these collaborations with us. Competing products, either developed by the collaborators or strategic partners or to which the collaborators or strategic partners have rights, may result in the withdrawal of partner support for our product candidates we may develop.

Some of our collaborators or strategic partners could also become our competitors in the future. Our collaborators or strategic partners could develop competing products, preclude us from entering into

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collaborations with their competitors, fail to obtain timely regulatory approvals, terminate their agreements with us prematurely, fail to devote sufficient resources to the development and commercialization of products, or merge with or be acquired by a third party who may do any of these things. Any of these developments could harm our product development efforts.

***If we are not able to establish collaborations on commercially reasonable terms, we may have to alter our development and commercialization plans.***

Our product development and research programs and the potential commercialization of any product candidates we may develop will require substantial additional cash to fund expenses. For some of the product candidates we may develop, we may decide to collaborate with other pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration, and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA, the EMA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization, reduce the scope of any sales or marketing activities, or increase our own expenditures on the development of the product candidate.

### **Risks related to commercialization**

***We face substantial competition, which may result in others discovering, developing or commercializing products before us or more successfully than we do.***

The development and commercialization of new drug products is highly competitive. We face competition with respect to any product candidates that we may develop from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. There are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of products for the treatment of many of the disorders for which we are conducting research programs. Some of these competitive products and therapies are based on scientific approaches that are the same as or similar to our approach, and others are based on entirely different approaches. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization.

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There are numerous companies that are selling or developing genetic medicines, including in indications for which we may develop our non-viral gene therapies. These companies include viral gene therapy companies such as BioMarin Pharmaceuticals, Inc., Homology Medicines, Inc., Adverum Biotechnologies, Inc. and Hoffmann La Roche Ltd; gene editing companies such as Crispr Therapeutics AG, Intellia Therapeutics, Inc. and Editas Medicine, Inc.; and mRNA companies such as Moderna, Inc. See “Business—Competition” for additional information regarding competition.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than our product candidates or that would render any product candidates that we may develop obsolete or non-competitive. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing any product candidates we may develop against competitors.

Many of the companies against which we are competing or against which we may compete in the future have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

***Even if any product candidate that we may develop receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payers and others in the medical community necessary for commercial success.***

If any product candidate we may develop receives marketing approval, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payers and others in the medical community. Sales of medical products depend in part on the willingness of physicians to prescribe the treatment, which is likely to be based on a determination by these physicians that the products are safe, therapeutically effective and cost-effective. In addition, the inclusion or exclusion of products from treatment guidelines established by various physician groups and the viewpoints of influential physicians can affect the willingness of other physicians to prescribe the treatment. We cannot predict whether physicians, physicians' organizations, hospitals, other healthcare providers, government agencies or private insurers will determine that our product is safe, therapeutically effective and cost-effective as compared with competing treatments. Efforts to educate the medical community and third-party payers on the benefits of any product candidates we may develop may require significant resources and may not be successful. If any product candidates we may develop do not achieve an adequate level of acceptance, we may not generate significant product revenues and we may not become profitable. The degree of market acceptance of any product candidates we may develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential advantages and limitations compared to alternative treatments;
- the effectiveness of sales and marketing efforts;

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- the cost of treatment in relation to alternative treatments;
- the clinical indications for which the product is approved;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of third-party coverage and adequate reimbursement;
- the prevalence and severity of any side effects; and
- any restrictions on the use of our products, if approved, together with other medications.

***The pricing, insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our future product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.***

The initial target platforms in our pipeline are indications with small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payers, the adoption of those product candidates and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect that coverage and reimbursement by third-party payers will be essential for most patients to be able to afford these treatments. Accordingly, sales of our future product candidates will depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payers. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement by government authorities for new products are typically made by the Centers for Medicare & Medicaid Services, or CMS, since CMS decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payers tend to follow CMS to a substantial degree. However, one payer's determination to provide coverage for a product does not assure that other payers will also provide coverage for the drug product. Further, a payer's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement agencies in the European Union may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries.

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of



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therapeutics such as any product candidates we may develop. In many countries, particularly the countries of the European Union, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay or might even prevent our commercial launch of the product, possibly for lengthy periods of time. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for product candidates. Accordingly, in markets outside the United States, the reimbursement for any product candidates we may develop may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payers, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for any product candidates we may develop. We expect to experience pricing pressures in connection with the sale of any product candidates we may develop due to the trend toward managed healthcare, the increasing influence of certain third-party payers, such as health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market. Recently there have been instances in which third-party payers have refused to reimburse treatments for patients for whom the treatment is indicated in the FDA-approved product label. Even if we are successful in obtaining FDA approvals to commercialize our product candidates, we cannot guarantee that we will be able to secure reimbursement for all patients for whom treatment with our product candidates is indicated.

In addition to CMS and private payers, professional organizations such as the American Medical Association, or the AMA, can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payers contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more product candidates for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

***If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution agreements with third parties, we may not be successful in commercializing any product candidates we may develop if and when they are approved.***

We do not have a sales or marketing infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any product for which we have obtained marketing approval, we will need to establish a sales, marketing and distribution organization, either ourselves or through collaborations or other arrangements with third parties.

In the future, we may build a sales and marketing infrastructure to market some of the product candidates we may develop if and when they are approved. There are risks involved with establishing our own sales, marketing and distribution capabilities. For example, recruiting and training a sales force is expensive and

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time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. These efforts may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our products on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales, marketing, coverage or reimbursement, customer service, medical affairs and other support personnel;
- the inability of sales personnel to educate adequate numbers of physicians on the benefits of any future products;
- the inability of reimbursement professionals to negotiate arrangements for formulary access, reimbursement and other acceptance by payers;
- the inability to price our products at a sufficient price point to ensure an adequate and attractive level of profitability;
- restricted or closed distribution channels that make it difficult to distribute our products to segments of the patient population;
- the lack of complementary products to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines; and
- unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we are unable to establish our own sales, marketing and distribution capabilities and we enter into arrangements with third parties to perform these services, our product revenues and our profitability, if any, are likely to be lower than if we were to market, sell and distribute any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute any product candidates we may develop or may be unable to do so on terms that are acceptable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing any product candidates we may develop.

***Any product candidates for which we intend to seek approval as biologic products may face competition sooner than anticipated.***

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Amendment, or the PPACA, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA, which created an abbreviated approval pathway for biological products that are biosimilar to or interchangeable with an FDA-licensed reference biological product. Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date that the reference product was first licensed by the FDA. In addition, the approval of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still market a competing version of the reference product if the FDA approves a full BLA for the competing product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The law is complex and is still being interpreted and implemented by the FDA. As a result, its ultimate impact, implementation, and meaning are subject to uncertainty. While it is uncertain when such processes

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intended to implement BPCIA may be fully adopted by the FDA, any such processes could have an adverse effect on the future commercial prospects for our biological products.

There is a risk that any product candidates we may develop that are approved as a biological product under a BLA would not qualify for the 12-year period of exclusivity or that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider any product candidates we may develop to be reference products for competing products, potentially creating the opportunity for generic competition sooner than anticipated. Other aspects of the BPCIA, some of which may impact the BPCIA exclusivity provisions, have also been the subject of recent litigation. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of our reference products in a way that is similar to traditional generic substitution for nonbiological products is not yet clear, and will depend on a number of marketplace and regulatory factors that are still developing.

***If the market opportunities for any product candidates we may develop are smaller than we believe they are, our potential revenues may be adversely affected, and our business may suffer. Because the target patient populations for many of the initial product candidates we may develop are small, we must be able to successfully identify patients and achieve a significant market share to maintain profitability and growth.***

We are focusing our initial research and product development on treatments for rare genetically defined diseases; as a result, the relevant patient population may be small. Our projections of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with product candidates we may develop, are based on estimates. These estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of these diseases. The number of patients in the United States, Europe and elsewhere may turn out to be lower than expected, and patients may not be amenable to treatment with our product candidates we may develop, or may become increasingly difficult to identify or gain access to, all of which would adversely affect our business, financial condition, results of operations and prospects. Additionally, because of the potential that any product candidates we may develop could cure a target disease, we may not receive recurring revenues from patients and may deplete the patient population prevalence through curative therapy.

### **Risks related to our intellectual property**

***Although we own and license a number of pending patent applications which have not yet issued as patents, we do not currently own or exclusively in-license any issued patents relating to any product candidates we may develop or technology, including with respect to our ceDNA constructs, ctLNP delivery system and manufacturing process. If we or our licensors are unable to obtain, maintain and defend patent and other intellectual property protection for our product candidates and technology, or if the scope of the patent or other intellectual property protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully develop and commercialize any product candidates we may develop or our technology may be adversely affected due to such competition.***

Our success depends in large part on our and our licensors' ability to obtain and maintain patent and other intellectual property protection in the United States and other jurisdictions with respect to any product candidates we may develop and our technology, including our ceDNA constructs, ctLNP delivery system, manufacturing processes and their respective components, formulations, combination therapies, methods of treatment, processes and development that are important to our business, as well as successfully defending these patents and other intellectual property against third-party challenges. We and our licensors have sought, and will seek, to protect our proprietary position by filing patent applications in the United States and abroad

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related to certain technologies and our platform that are important to our business. However, our patent portfolio is at an early stage and we currently do not own or exclusively license any issued patents in any jurisdiction. Moreover, there can be no assurance as to whether or when our patent applications will issue as granted patents. Our ability to stop third parties from making, using, selling, marketing, offering to sell, importing and commercializing any product candidates we may develop and our technology is dependent upon the extent to which we have rights under valid and enforceable patents and other intellectual property that cover our platform and technology. If we are unable to secure, maintain, defend and enforce patents and other intellectual property with respect to any product candidates we may develop and technology, it would have a material adverse effect on our business, financial condition, results of operations and prospects.

We own certain patent applications, and exclusively in-license from University of Massachusetts as represented by and solely on behalf of its Medical School, or UMass, certain other patent applications, which cover our ceDNA platform structure, use and/or function, our ctLNP platform and its use, and ceDNA manufacturing processes, as applicable. Our pending Patent Cooperation Treaty, or PCT, patent applications are not eligible to become issued patents until, among other things, we file a national stage patent application within 30 to 32 months, depending on the jurisdiction, from such application's priority date in the jurisdictions in which we are seeking patent protection. Similarly, our pending provisional patent applications are not eligible to become issued patents until, among other things, we file a non-provisional patent application within 12 months of such provisional patent application's filing date. If we do not timely file such national stage patent applications or non-provisional patent applications, we may lose our priority date with respect to such PCT or provisional patent applications, respectively, and any patent protection on the inventions disclosed in such PCT or provisional patent applications, respectively. While we and our licensors intend to timely file national stage and non-provisional patent applications relating to our PCT and provisional patent applications, respectively, we cannot predict whether any such patent applications will result in the issuance of patents. If we or our licensors do not successfully obtain issued patents, or, if the scope of any patent protection we or our licensors obtain is not sufficiently broad, we will be unable to prevent others from using any product candidates we may develop or our technology or from developing or commercializing technology and products similar or identical to ours or other competing products and technologies. Any failure to obtain or maintain patent protection with respect to our ceDNA constructs, ctLNP delivery system, manufacturing processes or our other product candidates and technology would have a material adverse effect on our business, financial condition, results of operations and prospects.

The patent prosecution process is expensive, time-consuming and complex, and we and our licensors may not be able to file, prosecute, maintain, defend, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. We and our licensors may not be able to obtain, maintain or defend patents and patent applications due to the subject matter claimed in such patents and patent applications being in the public domain. For example, in some cases, the work of certain academic researchers in the genetic medicine field has entered or will enter the public domain, which may compromise our and our licensors' ability to obtain patent protection for certain inventions related to or building upon such prior work. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach these agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection. Consequently, we would not be able to prevent any third party from using any of our technology that is in the public domain to compete with our product candidates.

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The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of patent rights are highly uncertain. Our pending and future owned and licensed patent applications may not result in patents being issued which protect our technology or product candidates, effectively prevent others from commercializing competitive technologies and product or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all, and even if such patent applications do issue as patents, they may not issue in a form, or with a scope of claims, that will provide us with any meaningful protection, prevent others from competing with us or otherwise provide us with any competitive advantage. In addition, the scope of claims of an issued patent can be reinterpreted after issuance, and changes in either the patent laws or interpretation of the patent laws in the United States and other jurisdictions may diminish the value of our patent rights or narrow the scope of our patent protection. Furthermore, our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

Third parties have developed technologies that may be related or competitive to our own technologies and product candidates and may have filed or may file patent applications, or may have obtained issued patents, claiming inventions that may overlap or conflict with those claimed in our owned or licensed patent applications or issued patents. We may not be aware of all third-party intellectual property rights potentially relating to our current and future product candidates and technology. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot know for certain whether the inventors of our owned or licensed patents and patent applications were the first to make the inventions claimed in any owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. If a third party can establish that we or our licensors were not the first to make or the first to file for patent protection of such inventions, our owned or licensed patent applications may not issue as patents and even if issued, may be challenged and invalidated or ruled unenforceable.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and other jurisdictions. For example, we may be subject to a third-party submission of prior art to the United States Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of our owned or licensed patents. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of our owned or licensed pending patent applications. We may become involved in opposition, derivation, re-examination, *inter partes* review, post-grant review or interference proceedings and similar proceedings in foreign jurisdictions (for example, opposition proceedings) challenging our owned or licensed patent rights. In addition, a third party may claim that our owned or licensed patent rights are invalid or unenforceable in a litigation. An adverse result in any litigation or patent office proceeding could put one or more of our owned or licensed patents at risk of being invalidated, ruled unenforceable or interpreted narrowly and could allow third parties to commercialize products identical or similar to any product candidates we may develop and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights. Moreover, we, or one of our licensors, may have to participate in interference proceedings declared by the USPTO to determine priority of invention or in post-grant challenge proceedings, such as oppositions in a foreign patent office, that challenge priority of invention or other features of patentability. Such challenges and proceedings may result in loss of patent rights, exclusivity, freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and any product candidates we may develop. Such challenges and proceedings may also result in substantial cost and require significant time from

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our scientists and management, even if the eventual outcome is favorable to us. Moreover, there could be public announcements of the results of hearings, motions or other interim proceedings or developments related to such challenges and proceedings and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Furthermore, patents have a limited lifespan. In the United States, the expiration of a patent is generally 20 years from the earliest date of filing of the first non-provisional patent application to which the patent claims priority. Patent term adjustments and extensions may be available; however, the overall term of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such product candidates might expire before or shortly after such product candidates are commercialized. As a result, our owned and licensed patent and other intellectual property rights may not provide us with sufficient rights to exclude others from commercializing products similar or identical to our technology and any product candidates we may develop. Any of the foregoing could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

***Our rights to develop and commercialize any product candidates are subject, in part, to the terms and conditions of licenses granted to us by third parties. If we fail to comply with our obligations under our current or future intellectual property license agreements or otherwise experience disruptions to our business relationships with our current or any future licensors, we could lose intellectual property rights that are important to our business.***

We are reliant upon licenses from third parties for certain patent and other intellectual property rights that are important or necessary to the development of our technology and product candidates. For example, we rely on a license from the National Institutes of Health, or NIH, and the Association Institut de Myologie, Universite Pierre et Marie Curie, Centre National de la Recherche Scientifique and Inserm Transfert SA, which we refer to as the French Institutions, pursuant to which we have been granted a non-exclusive, worldwide, royalty-bearing license to certain patent rights related to our ceDNA construct, to make and have made, research and have researched, use and have used, sell and have sold, offer to sell and to import products for the treatment, prevention or palliation of any human disease, disorder or condition. In addition, we rely on a license from UMass pursuant to which we have been granted an exclusive, worldwide, royalty-bearing license to certain patent rights related to our ceDNA construct to research, develop, manufacture, have manufactured, use, offer for sale, sell and import products in the treatment, prevention or palliation of any human disease, disorder or condition. Our existing license agreements, including our license agreements with NIH and UMass, impose, and we expect that future license agreements will impose, specified diligence, milestone payment, royalty, commercialization, development and other obligations on us and require us to meet development timelines, or to exercise diligent or commercially reasonable efforts to develop and commercialize licensed products, in order to maintain the licenses. For more information on the terms of the license agreements with NIH and UMass, see “Business—Intellectual property—License agreements.” We may enter into additional license agreements in the future.

Furthermore, the licensors of our license agreements have the right to terminate the agreement if we materially breach the agreement and fail to cure such breach within a specified period or in the event we undergo certain bankruptcy events. In spite of our best efforts, our current or any future licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements. If our license agreements are terminated, we may lose our rights to develop and commercialize any product candidates we may develop and technology, lose patent protection any product candidates we may develop and our technology, experience significant delays in the development and commercialization of our product candidates and technology and incur liability for damages. If these in-licenses are terminated, or if the

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underlying intellectual property fails to provide the intended exclusivity, our competitors or other third parties could have the freedom to seek regulatory approval of, and to market, products and technologies identical or competitive to ours and we may be required to cease our development and commercialization of certain of our product candidates and technology. In addition, we may seek to obtain additional licenses from our licensors and, in connection with obtaining such licenses, we may agree to amend our existing licenses in a manner that may be more favorable to the licensors, including by agreeing to terms that could enable third parties, including our competitors, to receive licenses to a portion of the intellectual property that is subject to our existing licenses and to compete with any product candidates we may develop and our technology. Any of the foregoing could have a material adverse effect on our competitive position, business, financial condition, results of operations and prospects.

Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- our or our licensors' ability to obtain, maintain and defend intellectual property and to enforce intellectual property rights against third parties;
- the extent to which our technology, product candidates and processes infringe, misappropriate or otherwise violate the intellectual property of the licensor that is not subject to the license agreement;
- the sublicensing of patent and other intellectual property rights under our license agreements;
- our diligence, development, regulatory, commercialization, financial or other obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship and ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our current or future licensors and us and our partners; and
- the priority of invention of patented technology.

In addition, our license agreements are, and future license agreements are likely to be, complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our diligence, development, regulatory, commercialization, financial or other obligations under the relevant agreement, either of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

If disputes over intellectual property that we have licensed or any other dispute described above related to our license agreements prevent or impair our ability to maintain our current license agreements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates and technology. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Our license agreement with NIH is, and other license agreements we may enter into in the future may be, non-exclusive. Accordingly, third parties may also obtain non-exclusive licenses from such licensors, including NIH, with respect to the intellectual property licensed to us under such license agreements, including our NIH license agreement. Accordingly, our NIH license agreement does not, and other license agreements may not, provide us with exclusive rights to use such licensed patent and other intellectual property rights, or may not provide us with exclusive rights to use such patent and other intellectual property rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our technology and any product candidates we may develop in the future.

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Moreover, some of our in-licensed patent and other intellectual property rights are, and may in the future be, subject to third party interests such as co-ownership. If we are unable to obtain an exclusive license to such third-party co-owners' interest, in such patent and other intellectual property rights, such third-party co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. We or our licensors may need the cooperation of any such co-owners of our licensed patent and other intellectual property rights in order to enforce them against third parties, and such cooperation may not be provided to us or our licensors.

Additionally, we do not have complete control over the preparation, filing, prosecution, maintenance, enforcement and defense of patents and patent applications that we license from third parties. For example, pursuant to each of our intellectual property licenses with NIH and UMass, our licensors retain control of preparation, filing, prosecution and maintenance, and, in certain circumstances, enforcement and defense of their patents and patent applications. It is possible that our licensors' filing, prosecution and maintenance of the licensed patents and patent applications, enforcement of patents against infringers or defense of such patents against challenges of validity or claims of enforceability may be less vigorous than if we had conducted them ourselves, and accordingly, we cannot be certain that these patents and patent applications will be prepared, filed, prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to file, prosecute, maintain, enforce and defend such patents and patent applications, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated, our right to develop and commercialize any of our technology and any product candidates we may develop that are the subject of such licensed rights could be adversely affected and we may not be able to prevent competitors or other third parties from making, using and selling competing products. Any of these events could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

Furthermore, our owned and in-licensed patent rights may be subject to a reservation of rights by one or more third parties. For example, inventions contained within some of our in-licensed patent rights may be made using U.S. government funding. When new technologies are developed with government funding, in order to secure ownership of patent rights related to the technologies, the recipient of such funding is required to comply with certain government regulations, including timely disclosing the inventions claimed in such patent rights to the U.S. government and timely electing title to such inventions. We rely on our licensors to ensure compliance with applicable obligations arising from such funding, including such timely disclosure and election of title. The failure of our licensors to meet their obligations may lead to a loss of rights or the unenforceability of relevant patents or patent applications. In addition, the U.S. government has certain rights in such in-licensed patent rights, including a non-exclusive license authorizing the U.S. government to use the invention or to have others use the invention on its behalf. If the U.S. government decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. The U.S. government's rights may also permit it to disclose the funded inventions and technology, which may include our confidential information, to third parties and to exercise march-in rights to use or allow third parties to use the technology we have licensed that was developed using U.S. government funding. The U.S. government may exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the U.S. government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such in-licensed U.S. government-funded inventions may be subject to certain requirements to manufacture any product candidates we may develop embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations and prospects significantly.



***We may not be able to protect our intellectual property rights throughout the world.***

Filing, prosecuting, maintaining, enforcing and defending patents and other intellectual property rights on our technology and any product candidates we may develop in all jurisdictions throughout the world would be prohibitively expensive, and accordingly, our intellectual property rights in some jurisdictions outside the United States could be less extensive than those in the United States. In some cases, we or our licensors may not be able to obtain patent or other intellectual property protection for certain technology and product candidates outside the United States. In addition, the laws of some foreign jurisdictions do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we and our licensors may not be able to obtain issued patents or other intellectual property rights covering any product candidates we may develop and our technology in all jurisdictions outside the United States and, as a result, may not be able to prevent third parties from practicing our and our licensors' inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Third parties may use our technologies in jurisdictions where we and our licensors have not pursued and obtained patent or other intellectual property protection to develop their own products and, further, may export otherwise infringing, misappropriating or violating products to territories where we have patent or other intellectual property protection, but enforcement is not as strong as that in the United States. These products may compete with any product candidates we may develop and our technology and our or our licensors' patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Additionally, many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain jurisdictions, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement, misappropriation or other violation of our patent and other intellectual property rights or marketing of competing products in violation of our intellectual property rights generally. For example, an April 2019 report from the Office of the United States Trade Representative identified a number of countries, including China, Russia, Argentina, Chile and India, where challenges to the procurement and enforcement of patent rights have been reported. Proceedings to enforce our or our licensors' patent and other intellectual property rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patent and other intellectual property rights at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We or our licensors may not prevail in any lawsuits that we or our licensors initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Many jurisdictions have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In addition, many jurisdictions limit the enforceability of patents against government agencies or government contractors. In these jurisdictions, the patent owner may have limited remedies, which could materially diminish the value of such patents. If we or any of our licensors is forced to grant a license to third parties with respect to any patents relevant to our business, our competitive position may be impaired, and our business, financial condition, results of operations and prospects may be adversely affected.

***Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or patent applications will be due to be paid to the USPTO and various government patent agencies outside of the

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United States over the lifetime of our owned or licensed patent rights. We rely on our outside counsel and other professionals or our licensing partners to pay these fees due to the USPTO and non-U.S. government patent agencies. The USPTO and various non-U.S. government patent agencies also require compliance with several procedural, documentary and other similar provisions during the patent application process. We rely on our outside counsel and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment, loss of priority or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our competitive position, business, financial conditions, results of operations and prospects.

### ***We may not be successful in obtaining necessary rights to product candidates we may develop through acquisitions and in-licenses.***

We currently have rights to certain intellectual property through licenses from third parties. Because our programs may require the use of additional intellectual property rights held by third parties, the growth of our business likely will depend, in part, on our ability to acquire, in-license or use these intellectual property rights. In addition, with respect to any patent or other intellectual property rights that we co-own with third parties, we may require exclusive licenses to such co-owners' interest in such patent or other intellectual property rights. However, we may be unable to secure such licenses or otherwise acquire or in-license any intellectual property rights related to compositions, methods of use, processes or other components from third parties that we identify as necessary for any product candidates we may develop and our technology on commercially reasonable terms, or at all. Even if we are able to in-license any such necessary intellectual property, it could be on non-exclusive terms, thereby giving our competitors and other third parties access to the same intellectual property licensed to us, and the applicable licensors could require us to make substantial licensing and royalty payments. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive or necessary. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to third parties, potentially blocking our ability to pursue our research program and develop and commercialize our product candidates.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have licensed, we may be required to expend significant time and resources to redesign any product candidates we may develop or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected product candidates, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

***Issued patents covering any product candidates we may develop could be found invalid or unenforceable if challenged in court or before administrative bodies in the United States or abroad.***

Our owned and licensed patent rights may be subject to priority, validity, inventorship and enforceability disputes. If we or our licensors are unsuccessful in any of these proceedings, such patent rights may be narrowed, invalidated or held unenforceable, we may be required to obtain licenses from third parties, which may not be available on commercially reasonable terms or at all, or we may be required to cease the development, manufacture and commercialization of one or more of our product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

If one of our licensing partners, one of our co-owners, we or our licensor's other licensees initiate legal proceedings against a third party to enforce a patent covering any of any product candidates we may develop or our technology, the defendant could counterclaim that the patent covering the product candidate or technology is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, lack of written description or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, interference proceedings, derivation proceedings, post grant review, *inter partes* review and equivalent proceedings such as opposition, invalidation and revocation proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover any product candidates we may develop or our technology or prevent third parties from competing with any product candidates we may develop or our technology. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a third party were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates or technology. Such a loss of patent protection could have a material adverse effect on our business, financial condition, results of operations and prospects.

***If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.***

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors, contractors and other parties who have access to such technology and processes. However, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements. Monitoring unauthorized uses and disclosures is difficult, and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the collaborators, scientific advisors, employees and consultants who are parties to these agreements breach or violate the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets and third parties could use our trade secrets to compete

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with any product candidates we may develop and our technology. Additionally, we cannot guarantee that we have entered into such agreements with each party that may have or has had access to our trade secrets or proprietary technology and processes.

We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems; however, such systems and security measures may be breached, and we may not have adequate remedies for any breach.

In addition, our trade secrets may otherwise become known or be independently discovered by competitors or other third parties. Competitors or third parties could purchase any product candidates we may develop or our technology and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our intellectual property rights or develop their own competitive technologies that fall outside the scope of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor or other third party, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products, our business, financial condition, results of operations and prospects could be materially and adversely affected.

***Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could harm our business.***

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property rights of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may become party to, or be threatened with, adversarial proceedings or litigation in which third parties may assert infringement, misappropriation or other violation claims against us, alleging that any product candidates we may develop, manufacturing methods, formulations or administration methods are covered by their patents. Given the vast number of patents and other intellectual property in our field of technology, we cannot be certain or guarantee that we do not infringe, misappropriate or otherwise violate patents or other intellectual property. Other companies and institutions have filed, and continue to file, patent applications that may be related to our technology and, more broadly, to gene therapy and related manufacturing methods. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. If a patent holder believes the manufacture, use, sale or importation of any product candidates we may develop or our technology infringes its patent, the patent holder may sue us even if we have licensed other patent rights for our technology.

It is also possible that we have failed to identify relevant third-party patents or applications. Because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending which may later result in issued patents that may be infringed by the manufacture, use, sale or importation of any product candidates we may develop or our technology and we may not be aware of such patents. Furthermore, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States may remain confidential until a patent issues. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to any product candidates we may develop and our technologies

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because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our technology. In addition, we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our technologies, any product candidates we may develop or the use of any product candidates we may develop.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize any product candidates we may develop or any other of our product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue developing, manufacturing and marketing any product candidates we may develop and our technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from manufacturing and commercializing any product candidates we may develop or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business, financial condition, results of operations and prospects.

### ***Intellectual property litigation or other proceedings could cause us to spend substantial resources and distract our personnel from their normal responsibilities.***

Competitors may challenge the validity and enforceability of our patent rights or those of our licensing partners, infringe, misappropriate or otherwise violate our or our licensors' patent and other intellectual property rights, or we may be required to defend against claims of infringement, misappropriation or other violation. Litigation and other proceedings in connection with any of the foregoing claims can be unpredictable, expensive and time consuming. Even if resolved in our favor, litigation or other proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our scientific, technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

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We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors or other third parties may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could adversely affect our ability to compete in the marketplace and could have a material adverse effect on our business, financial condition, results of operations and prospects.

***We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.***

Many of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or be required to obtain licenses to such intellectual property rights, which may not be available on commercially reasonable terms or at all. An inability to incorporate such intellectual property rights would harm our business and may prevent us from successfully commercializing any product candidates we may develop or at all. In addition, we may lose personnel as a result of such claims and any such litigation or the threat thereof may adversely affect our ability to hire employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize any product candidates we may develop and our technology, which would have a material adverse effect on our business, results of operations, financial condition and prospects. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to our scientific and management personnel.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. Moreover, even when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Furthermore, individuals executing agreements with us may have pre-existing or competing obligations to a third party, such as an academic institution, and thus an agreement with us may be ineffective in perfecting ownership of inventions developed by that individual. Disputes about the ownership of intellectual property that we own may have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, we or our licensors may in the future be subject to claims by former employees, consultants or other third parties asserting an ownership right in our owned or licensed patent rights. An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar technology and therapeutics, without payment to us, or could limit the duration of the patent protection covering our technology and any product candidates we may develop. Such challenges may also result in our inability to develop, manufacture or commercialize our technology and product candidates without infringing third-party patent rights. In addition, if the breadth or

strength of protection provided by our owned or licensed patent rights are threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future technology and product candidates. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

***Changes in patent law in the United States or worldwide could diminish the value of patents in general, thereby impairing our ability to protect any product candidates we may develop and our technology.***

Changes in either the patent laws or interpretation of patent laws in the United States and worldwide, including patent reform legislation such as the Leahy-Smith America Invents Act, or the Leahy-Smith Act, could increase the uncertainties and costs surrounding the prosecution of any owned or in-licensed patent applications and the maintenance, enforcement or defense of any current in-licensed issued patents and issued patents we may own or in-license in the future. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These changes include provisions that affect the way patent applications are prosecuted, redefine prior art, provide more efficient and cost-effective avenues for competitors to challenge the validity of patents, and enable third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent at USPTO-administered post-grant proceedings, including post-grant review, *inter partes* review, and derivation proceedings. Assuming that other requirements for patentability are met, prior to March 2013, in the United States, the first to invent the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith Act, the United States transitioned to a first-to-file system in which, assuming that the other statutory requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the claimed invention. As such, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our in-licensed issued patents and issued patents we may own or in-license in the future, all of which could have a material adverse effect on our business, financial condition, results of operations and prospects.

In addition, the patent positions of companies in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. As one example, in the case *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the U.S. Supreme Court held that certain claims to DNA molecules are not patentable simply because they have been isolated from surrounding material. Moreover, in 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the claim amounts to significantly more than the natural principle itself should be rejected as directed to patent-ineligible subject matter. Accordingly, in view of the guidance memo, there can be no assurance that claims in our patent rights covering any product candidates we may develop or our technology will be held by the USPTO or equivalent foreign patent offices or by courts in the United States or in foreign jurisdictions to cover patentable subject matter. This combination of events has created uncertainty with respect to the validity and enforceability of patents once obtained. Depending on future actions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that could have a material adverse effect on our patent rights and our ability to protect, defend and enforce our patent rights in the future.

***If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be harmed.***

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop and our technology, one or more of our U.S. patents that we license or may own in the future may be eligible for limited patent term extension under Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved product, a method for using it or a method for manufacturing it may be extended. The application for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

***We may be subject to claims challenging the inventorship or ownership of our patent and other intellectual property rights.***

We or our licensors may be subject to claims that former employees, collaborators or other third parties have an interest in our owned or in-licensed patent rights, trade secrets or other intellectual property as an inventor or co-inventor. For example, we or our licensors may have inventorship disputes arise from conflicting obligations of employees, consultants or others who are involved in developing our product candidates or technology. Litigation may be necessary to defend against these and other claims challenging inventorship or our or our licensors' ownership of our owned or in-licensed patent rights, trade secrets or other intellectual property. If we or our licensors fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights, such as exclusive ownership of or right to use intellectual property that is important to any product candidates we may develop or our technology. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management and other employees. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

***If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be adversely affected.***

We have filed trademark applications with the USPTO for the mark "Generation Bio" and the Generation Bio logo. Our current and future trademark applications in the United States and other foreign jurisdictions may not be allowed or may be subsequently opposed. Once filed and registered, our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. As a means to enforce our trademark rights and prevent infringement, we may be required to file trademark claims against third parties or initiate trademark opposition proceedings. This can be expensive and time-consuming, particularly for a company of our size. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of



interest. At times, third parties may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations or prospects.

***Intellectual property rights do not necessarily address all potential threats.***

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to any product candidates we may develop but that are not covered by the intellectual property, including the claims of the patents, that we own or license currently or in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we own or license currently or in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing, misappropriating or otherwise violating our owned or licensed intellectual property rights;
- it is possible that our or our licensors' current or future pending patent applications will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by third parties;
- third parties might conduct research and development activities in jurisdictions where we do not have patent or other intellectual property rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could significantly harm our business, financial condition, results of operations and prospects.

***Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor or other third party will discover our trade secrets or that our trade secrets will be misappropriated or disclosed.***

Because we currently rely on certain third parties to manufacture all or part of our drug product and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our product engine and pipeline, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements and other similar agreements with our collaborators, advisors, employees, consultants and contractors prior to beginning research or disclosing any proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors or other third parties, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's or other third party's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

## **Risks related to regulatory approval and other legal compliance matters**

***Regulatory requirements governing genetic medicine products, and in particular any novel gene therapy products we may develop, have changed frequently and may continue to change in the future.***

Regulatory requirements governing gene and cell therapy products, and in particular any novel gene therapy products we may develop, have changed frequently and may continue to change in the future. We are aware of a limited number of gene therapy products that have received marketing authorization from the FDA and EMA. Even with respect to more established products in the gene therapy field, the regulatory landscape is still developing. For example, the FDA has established the Office of Tissues and Advanced Therapies (formerly the Office of Cellular, Tissue and Gene Therapies) within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, also are potentially subject to review by the Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC; however, the NIH announced that the RAC will soon only publicly review clinical trials if the trials cannot be evaluated by standard oversight bodies and pose unusual risks.

The same applies in the European Union. The EMA's Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety and efficacy of advanced-therapy medicinal products. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the Committee for Medicinal Products for Human Use, or CHMP, before CHMP adopts its final opinion. In the European Union, the development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant European Union guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. As a result, the procedures and standards applied to gene therapy

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products and cell therapy products may be applied to any product candidates we may develop, but that remains uncertain at this point.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of any product candidates we may develop or lead to significant post-approval limitations or restrictions. As we advance any product candidates we may develop, we will be required to consult with these regulatory and advisory groups and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of these product candidates. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue to maintain our business.

Although the FDA decides whether individual genetic medicine protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution to conduct a clinical trial, that institution's institutional biosafety committee, or IBC, as well as its IRB would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of genetic medicine products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any product candidates we may develop. Similarly, the EMA may issue new guidelines concerning the development and marketing authorization for genetic medicine products and require that we comply with these new guidelines.

As we are initially seeking to identify and develop product candidates to treat diseases using novel technologies, there is heightened risk that the FDA, the EMA or other regulatory authority may not consider the clinical trial endpoints that we propose to provide clinically meaningful results. Even if the endpoints are deemed clinically meaningful, we may not achieve these endpoints to a degree of statistical significance, particularly because many of the diseases we are targeting with our platform have small patient populations, making development of large and rigorous clinical trials more difficult.

Adverse developments in post-marketing experience or in clinical trials conducted by others of gene therapy products or cell therapy products may cause the FDA, the EMA, and other regulatory bodies to revise the requirements for development or approval of any product candidates we may develop or limit the use of products utilizing non-viral gene therapy technologies, either of which could materially harm our business. In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as the product candidates we may develop can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Regulatory agencies administering existing or future regulations or legislation may not allow production and marketing of products utilizing non-viral gene therapy technology in a timely manner or under technically or commercially feasible conditions. In addition, regulatory action or private litigation could result in expenses, delays or other impediments to our research programs or the commercialization of resulting products.

In addition, ethical, social and legal concerns about genetic medicine, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that any product candidates we may develop are unsafe or

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pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of any product candidates we may develop under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

As we advance any product candidates we may develop through clinical development, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of any product candidates we may develop or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue.

***Even if we complete the necessary preclinical studies and clinical trials, the marketing approval process is expensive, time-consuming and uncertain and may prevent us from obtaining approvals for the commercialization of any product candidates we may develop. If we are not able to obtain, or if there are delays in obtaining, required regulatory approvals, we will not be able to commercialize, or will be delayed in commercializing, product candidates we may develop, and our ability to generate revenue will be materially impaired.***

Any product candidates we may develop and the activities associated with their development and commercialization, including their design, testing, manufacture, safety, efficacy, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other regulatory authorities in the United States and by comparable authorities in other countries. Failure to obtain marketing approval for a product candidate we may develop will prevent us from commercializing the product candidate in a given jurisdiction. We have not received approval to market any product candidates from regulatory authorities in any jurisdiction. We have only limited experience in filing and supporting the applications necessary to gain marketing approvals and expect to rely on third-party CLROs to assist us in this process. Securing regulatory approval requires the submission of extensive preclinical and clinical data and supporting information to the various regulatory authorities for each therapeutic indication to establish the biologic product candidate's safety, purity and potency. Securing regulatory approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the relevant regulatory authority. Any product candidates we may develop may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities, or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use.

The process of obtaining marketing approvals, both in the United States and abroad, is expensive, may take many years if additional clinical trials are required, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Changes in marketing approval policies during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. The FDA and comparable authorities in other countries have substantial discretion in the approval process and may refuse to accept any application or may decide that our data is insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we ultimately obtain may be

limited or subject to restrictions or post-approval commitments that render the approved medicine not commercially viable.

If we experience delays in obtaining approval or if we fail to obtain approval of any product candidates we may develop, the commercial prospects for those product candidates may be harmed, and our ability to generate revenues will be materially impaired.

***Negative public opinion of gene therapy and increased regulatory scrutiny of gene therapy and genetic research may adversely impact public perception of our future product candidates.***

Our potential therapeutic products involve introducing genetic material into patients' cells. The clinical and commercial success of our potential products will depend in part on public acceptance of the use of gene therapy and gene regulation for the prevention or treatment of human diseases. Public attitudes may be influenced by claims that gene therapy and gene regulation are unsafe, unethical or immoral, and, consequently, our products may not gain the acceptance of the public or the medical community. Adverse public attitudes may adversely impact our ability to enroll clinical trials. Moreover, our success will depend upon physicians prescribing, and their patients being willing to receive, treatments that involve the use of product candidates we may develop in lieu of, or in addition to, existing treatments with which they are already familiar and for which greater clinical data may be available.

More restrictive government regulations or negative public opinion would have a negative effect on our business or financial condition and may delay or impair the development and commercialization of our product candidates or demand for any products once approved. For example, in 2003, trials using early versions of murine gamma-retroviral vectors, which integrate with, and thereby alter, the host cell's DNA, have led to several well-publicized adverse events, including reported cases of leukemia. Although our delivery system is non-viral, any product candidates we may develop may be associated with such viral delivery systems as a gene therapy platform. Adverse events in our clinical trials, even if not ultimately attributable to our product candidates, and the resulting publicity could result in increased governmental regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates. The risk of cancer remains a concern for gene therapy and we cannot assure that it will not occur in any of our planned or future clinical trials. In addition, there is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biological activity of the genetic material or other components of products used to carry the genetic material. If any such adverse events occur, commercialization of our product candidates or further advancement of our clinical trials could be halted or delayed, which would have a negative impact on our business and operations.

***Failure to obtain marketing approval in foreign jurisdictions would prevent any product candidates we may develop from being marketed in such jurisdictions, which, in turn, would materially impair our ability to generate revenue.***

In order to market and sell any product candidates we may develop in the European Union and many other foreign jurisdictions, we or our collaborators must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We or these third parties may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in

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other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our medicines in any jurisdiction, which would materially impair our ability to generate revenue.

Political and socioeconomic factors can adversely affect our business. For example, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the European Union on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020 (extendable up to two years). Discussions between the United Kingdom and the European Union have so far mainly focused on finalizing withdrawal issues and transition agreements but have been extremely difficult. To date, only an outline of a trade agreement has been reached. Much remains open but the Prime Minister has indicated that the United Kingdom will not seek to extend the transitional period beyond the end of 2020. If no trade agreement has been reached before the end of the transitional period, there may be significant market and economic disruption. The Prime Minister has also indicated that the United Kingdom will not accept high regulatory alignment with the European Union.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for any product candidates we may develop, which could significantly and materially harm our business.

***Fast track designation by the FDA may not actually lead to a faster development or regulatory review or approval process and does not assure FDA approval of any product candidates we may develop.***

If any product candidate we may develop is intended for the treatment of a serious or life-threatening condition and the product candidate demonstrates the potential to address unmet medical need for this condition, the sponsor may apply for FDA fast track designation. However, a fast track designation does not ensure that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive fast track designation for any product candidates we may develop, we may not experience a faster development process, review or approval compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

***Breakthrough or RMAT therapy designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of any product candidates we may develop.***

If any product candidate we may develop is intended, either alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development, the sponsor may apply for FDA breakthrough designation or a regenerative medicine advanced therapy, or RMAT, designation. However, neither a breakthrough designation nor an RMAT designation ensures that the product candidate will receive marketing approval or that approval will be granted within any particular timeframe. As a result, while we may seek and receive breakthrough or RMAT designation for any product candidates we may develop, we may not experience a faster development process, review or approval compared to conventional

FDA procedures. In addition, the FDA may withdraw breakthrough or RMAT designation if it believes that the designation is no longer supported by data from our clinical development program. Neither breakthrough nor RMAT designation alone guarantees qualification for the FDA's priority review procedures.

***Priority review designation by the FDA may not lead to a faster regulatory review or approval process and, in any event, does not assure FDA approval of any product candidates we may develop.***

If the FDA determines that a product candidate we may develop offers major advances in treatment or provides a treatment where no adequate therapy exists, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for any product candidates we may develop. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate we may develop is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster regulatory review process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or thereafter.

***We may not be able to obtain orphan drug exclusivity for any product candidates we may develop, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.***

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan products by the EMA in the European Union. Generally, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for the same product for the same therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

In order for the FDA to grant orphan drug exclusivity to one of our products, the agency must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually in the United States. The FDA may conclude that the condition or disease for which we seek orphan drug exclusivity does not meet this standard. Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition. In particular, the concept of what constitutes the "same drug" for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA has issued recent draft guidance suggesting that it would not consider two genetic medicine products to be different drugs solely based on minor differences in the transgenes or vectors. In addition, even after an orphan drug is approved, the FDA can subsequently approve the same product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition.

In 2017, the Congress passed the FDA Reauthorization Act of 2017, or the FDARA. FDARA, among other things, codified the FDA's pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same

rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

***Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when or if we will obtain regulatory approval to commercialize a product candidate we may develop or the approval may be for a more narrow indication than we expect.***

We cannot commercialize a product until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if any product candidates we may develop demonstrate safety and efficacy in clinical trials, the regulatory agencies may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory agency policy during the period of product development, clinical trials and the review process. Regulatory agencies also may approve a product candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. In addition, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of any product candidates we may develop. For example, our development of any product candidates for pediatric use is an important part of our current business strategy, and if we are unable to obtain regulatory approval for the desired age ranges, our business may suffer.

***Even if we, or any collaborators we may have, obtain marketing approvals for any product candidates we may develop, the terms of approvals and ongoing regulation of our products could require the substantial expenditure of resources and may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.***

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post-approval clinical data, labeling, advertising and promotional activities for such medicine, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents, and requirements regarding the distribution of samples to physicians and recordkeeping. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The FDA typically advises that patients treated with genetic medicine undergo follow-up observations for potential adverse events for a 15-year period. The holder of an approved BLA must also submit new or supplemental applications and obtain FDA approval for certain changes to the approved product, product labeling or manufacturing process. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the medicine may be marketed or to the conditions of approval, or contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the medicine.

Accordingly, assuming we, or any collaborators we may have, receive marketing approval for one or more product candidates we may develop, we, and such collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we and such collaborators are not able to comply with



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post-approval regulatory requirements, we and such collaborators could have the marketing approvals for our products withdrawn by regulatory authorities and our, or such collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our business, operating results, financial condition and prospects.

If we fail to comply with applicable regulatory requirements following approval of any product candidates we may develop, a regulatory agency may:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or supplements to a BLA submitted by us;
- seize product; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we may develop and generate revenues.

***Any product candidate we may develop for which we obtain marketing approval could be subject to restrictions or withdrawal from the market, and we may be subject to substantial penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our medicines, when and if any of them are approved.***

The FDA and other regulatory agencies closely regulate the post-approval marketing and promotion of medicines to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA and other regulatory agencies impose stringent restrictions on manufacturers' communications regarding off-label use, and if we do not market our medicines for their approved indications, we may be subject to enforcement action for off-label marketing by the FDA and other federal and state enforcement agencies, including the Department of Justice. Violation of the Federal Food, Product, and Cosmetic Act and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription products may also lead to investigations or allegations of violations of federal and state healthcare fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown problems with our medicines, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such medicines, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a medicine;
- restrictions on the distribution or use of a medicine;
- requirements to conduct post-marketing clinical trials;
- receipt of warning or untitled letters;
- withdrawal of the medicines from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of medicines;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals;
- suspension of any ongoing clinical trials;
- refusal to permit the import or export of our medicines;

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- product seizure; and
- injunctions or the imposition of civil or criminal penalties.

Any government investigation of alleged violations of law could require us to expend significant time and resources in response and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize any product candidates we develop and adversely affect our business, financial condition, results of operations and prospects.

Additionally, if any product candidates we may develop receive marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to healthcare practitioners. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

***We are affected by the political environment and changes that may be made to regulatory regimes. The efforts of the current presidential administration to pursue regulatory reform may limit the FDA's ability to engage in oversight and implementation activities in the normal course, and that could negatively impact our business.***

The current presidential administration has taken several executive actions, including the issuance of a number of executive orders, that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance and review and approval of marketing applications. On January 30, 2017, the president issued an executive order, applicable to all executive agencies, including the FDA, that required that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the "two-for-one" provisions. This executive order includes a budget neutrality provision that required the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the executive order requires agencies to identify regulations to offset any incremental cost of a new regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within the Office of Management on February 2, 2017, the administration indicated that the "two-for-one" provisions may apply not only to agency regulations, but also to significant agency guidance documents. It is difficult to predict how these requirements will be implemented, and the extent to which they will impact the FDA's ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

***Our relationships with healthcare providers, physicians and third-party payers will be subject to applicable anti-kickback, fraud and abuse, and other healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.***

Healthcare providers, physicians and third-party payers play a primary role in the recommendation and prescription of any product candidates that we develop for which we obtain marketing approval. Our future arrangements with third-party payers and customers may expose us to broadly applicable fraud and abuse and

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other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute our medicines for which we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal healthcare anti-kickback statute prohibits, among other things, persons from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order, or recommendation of, any good or service, for which payment may be made under federal and state healthcare programs such as Medicare and Medicaid;
- the federal False Claims Act imposes criminal and civil penalties, including civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting or causing to be presented, to the federal government, claims for payment or approval from Medicare, Medicaid or other government payers that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties, currently set at \$11,181 to \$22,363 per false claim;
- the federal Health Insurance Portability and Accountability Act of 1996, as further amended by the Health Information Technology for Economic and Clinical Health Act, which imposes certain requirements, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, healthcare clearinghouses and healthcare providers;
- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements under the federal Physician Payment Sunshine Act, which requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services, or HHS, information related to payments and other transfers of value to physicians and teaching hospitals and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and
- analogous state laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payers, including private insurers, and certain state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring drug manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our business, financial condition, results of operations and prospects.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the

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European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business are found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Liabilities they incur pursuant to these laws could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

***Recently enacted and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.***

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of any product candidates we may develop, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or the Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payers.

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The PPACA, which became law in 2010, contains provisions of importance to our business, including, without limitation, our ability to commercialize and the prices we may obtain for any product candidates we may develop and that are approved for sale, the following:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers' Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the PPACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2029 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the PPACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the PPACA-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Further, the Bipartisan Budget Act of 2018, among other things, amended the PPACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." The Congress may consider other legislation to replace elements of the PPACA during the next Congressional session.

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Further, each chamber of the Congress has put forth multiple bills designed to repeal or repeal and replace portions of the PPACA. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the PPACA. The Congress will likely consider other legislation to replace elements of the PPACA, during the next Congressional session. It is possible that repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. While the timing and scope of any potential future legislation to repeal and replace PPACA provisions is highly uncertain in many respects, it is also possible that some of the PPACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with PPACA coverage expansion provision.

We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our potential products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payers.

The current presidential administration has also taken executive actions to undermine or delay implementation of the PPACA. Since January 2017, the president has signed two executive orders designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. One executive order directs federal agencies with authorities and responsibilities under the PPACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the PPACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers or manufacturers of pharmaceuticals or medical devices. The second executive order terminates the cost-sharing subsidies that reimburse insurers under the PPACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the PPACA for plans sold through such marketplaces. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than \$12 billion in PPACA risk corridor payments to third-party payers who argued were owed to them. This decision is under review by the U.S. Supreme Court during its current term. The full effects of this gap in reimbursement on third-party payers, the viability of the PPACA marketplace, providers, and potentially our business, are not yet known.

The costs of prescription pharmaceuticals have also been the subject of considerable discussion in the United States, and members of Congress and the executive branch have stated that they will address such costs through new legislative and administrative measures. To date, there have been several recent U.S. congressional inquiries and proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, the current presidential administration has pressed for drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the current presidential administration have

each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. In addition, on December 23, 2019, the Trump Administration published a proposed rulemaking that, if finalized, would allow states or certain other non-federal government entities to submit importation program proposals to FDA for review and approval. Applicants would be required to demonstrate their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. At the same time, the FDA issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for any product candidates we may develop or additional pricing pressures.

***Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.***

We are exposed to the risk of fraud or other misconduct by our employees, consultants and partners, and, if we commence clinical trials, our principal investigators. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately, or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

***Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain product candidates outside of the United States and require us to develop and implement costly compliance programs.***

We are subject to numerous laws and regulations in each jurisdiction outside the United States in which we operate. The creation, implementation and maintenance of international business practices compliance programs is costly and such programs are difficult to enforce, particularly where reliance on third parties is required.

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The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations. The anti-bribery provisions of the FCPA are enforced primarily by the Department of Justice. The Securities and Exchange Commission, or SEC, is involved with enforcement of the books and records provisions of the FCPA.

Compliance with the FCPA and other anti-corruption laws potentially applicable to our business is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, compliance with the FCPA and other anti-corruption laws presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. Our expansion outside of the United States has required, and will continue to require, us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing or selling certain drugs and drug candidates outside of the United States, which could limit our growth potential and increase our development costs. The failure to comply with laws governing international business practices may result in substantial penalties, including suspension or debarment from government contracting. Violation of the FCPA can result in significant civil and criminal penalties. Indictment alone under the FCPA can lead to suspension of the right to do business with the U.S. government until the pending claims are resolved. Conviction of a violation of the FCPA can result in long-term disqualification as a government contractor. The termination of a government contract or relationship as a result of our failure to satisfy any of our obligations under laws governing international business practices would have a negative impact on our operations and harm our reputation and ability to procure government contracts. The SEC also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

***We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.***

We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.



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The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the European Union General Data Protection Regulation (EU) 2016/679, or the GDPR, which took effect across all member states of the European Economic Area, or EEA, in May 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States and, as a result, increases the scrutiny that such rules should apply to transfers of personal data from clinical trial sites located in the EEA to the United States. The GDPR also permits data protection authorities to require destruction of improperly gathered or used personal information and/or impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or 20 million Euros, whichever is greater, and confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR provides that European Union member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with the GDPR's requirements are rigorous and time intensive and require significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data collected in the European Union. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

Similar privacy and data security requirements are either in place or underway in the United States. There are a broad variety of data protection laws that may be applicable to our activities, and a range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act of 2018, or the CCPA, which became effective on January 1, 2020, requires companies that process information on California residents to make new disclosures to consumers about their data collection, use and sharing practices, allow consumers to opt out of certain data sharing with third parties and provide a new cause of action for data breaches. Many other states are considering similar legislation, and a broad range of legislative measures also have been introduced at the federal level.

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There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information, including certain laws that regulate the use and disclosure of personal health information. In particular, regulations promulgated pursuant to the Health Insurance Portability and Accountability Act of 1996, or HIPAA, establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. These provisions may be applicable to our business in the future. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation.

If we are unable to properly protect the privacy and security of protected health information, we could be found to have violated these privacy and security laws and/or breached certain contracts with our business partners. Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems.

It is possible that new and existing laws may be interpreted and applied in a manner that is inconsistent with our practices and our efforts to comply with the evolving data protection rules may be unsuccessful. If so, this could result in government-imposed fines or penalties or orders requiring that we change our practices, which could adversely affect our business. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with federal, state and international laws regarding privacy and security of personal information could expose us to fines and penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. We also face a threat of consumer class actions related to these laws and the overall protection of personal data. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business, financial condition, results of operations or prospects.

### **Risks related to employee matters and managing growth**

***Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.***

We are highly dependent on the research and development, clinical, financial, operational and other business expertise of our executive officers, as well as the other principal members of our management, scientific and clinical teams. Although we have entered into employment offer letters with our executive officers, each of them may terminate their employment with us at any time. We do not maintain “key person” insurance for any of our executives or other employees. Recruiting and retaining qualified scientific, clinical, manufacturing, accounting, legal and sales and marketing personnel will also be critical to our success.

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The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize products. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. Our success as a public company also depends on implementing and maintaining internal controls and the accuracy and timeliness of our financial reporting. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

***We expect to expand our development and regulatory capabilities and potentially implement sales, marketing and distribution capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.***

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, clinical, regulatory affairs and, if any product candidate we may develop receives marketing approval, sales, marketing and distribution. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

As a growing biotechnology company, we are actively pursuing new platforms and product candidates in many therapeutic areas and across a wide range of diseases. Successfully developing product candidates for and fully understanding the regulatory and manufacturing pathways to all of these therapeutic areas and disease states requires a significant depth of talent, resources and corporate processes in order to allow simultaneous execution across multiple areas. Due to our limited resources, we may not be able to effectively manage this simultaneous execution and the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure, give rise to operational mistakes, legal or regulatory compliance failures, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of our product candidates. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than expected, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to compete effectively and commercialize our product candidates, if approved, will depend in part on our ability to effectively manage the future development and expansion of our company.

***Future acquisitions or strategic alliances could disrupt our business and harm our financial condition and results of operations.***

We may acquire additional businesses or drugs, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new drugs resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with strategic partners as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into our organization;
- the need to implement or improve controls, procedures and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;
- liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities and other known liabilities;
- unanticipated write-offs or charges; and
- litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or results of operations.

***Our internal information technology systems, or those of our third-party vendors, collaborators or other contractors or consultants, may fail or suffer security breaches, loss or leakage of data and other disruptions, which could result in a material disruption of our product development programs, compromise sensitive information related to our business or prevent us from accessing critical information, potentially exposing us to liability or otherwise adversely affecting our business.***

We are increasingly dependent upon information technology systems, infrastructure and data to operate our business. In the ordinary course of business, we collect, store and transmit confidential information (including but not limited to intellectual property, proprietary business information and personal information). It is critical that we do so in a secure manner to maintain the confidentiality and integrity of such confidential information.

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Despite the implementation of security measures, given the size and complexity of our internal information technology systems and those of our current and any future third-party vendors, collaborators and other contractors and consultants, and the increasing amounts of confidential information that they maintain, such information technology systems are vulnerable to damage or interruption from computer viruses, computer hackers, malicious code, employee theft or misuse, denial-of-service attacks, sophisticated nation-state and nation-state-supported actors, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. The risk of a security breach or disruption, particularly through cyber-attacks or cyber intrusion, including by computer hackers, foreign governments and cyber terrorists, has generally increased as the number, intensity and sophistication of attempted attacks and intrusions from around the world have increased. We may not be able to anticipate all types of security threats, and we may not be able to implement preventive measures effective against all such security threats. The techniques used by cyber criminals change frequently, may not be recognized until launched, and can originate from a wide variety of sources, including outside groups such as external service providers, organized crime affiliates, terrorist organizations or hostile foreign governments or agencies.

While we seek to protect our information technology systems from system failure, accident and security breach, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary or confidential information or other disruptions. For example, the loss of clinical trial data from future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. If we were to experience a significant cybersecurity breach of our information systems or data, the costs associated with the investigation, remediation and potential notification of the breach to counterparties and data subjects could be material. In addition, our remediation efforts may not be successful. Moreover, if the information technology systems of our third-party vendors, collaborators and other contractors and consultants become subject to disruptions or security breaches, we may have insufficient recourse against such third parties and we may have to expend significant resources to mitigate the impact of such an event, and to develop and implement protections to prevent future events of this nature from occurring. If we do not allocate and effectively manage the resources necessary to build and sustain the proper technology and cybersecurity infrastructure, we could suffer significant business disruption, including transaction errors, supply chain or manufacturing interruptions, processing inefficiencies, data loss or the loss of or damage to intellectual property or other proprietary information.

To the extent that any disruption or security breach were to result in a loss of, or damage to, our or our third-party vendors', collaborators' or other contractors' or consultants' data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability including litigation exposure, penalties and fines, we could become the subject of regulatory action or investigation based primarily on the laws and regulations discussed above in the privacy discussion, our competitive position and reputation could be harmed and the further development and commercialization of our product candidates could be delayed. Furthermore, any such event that leads to unauthorized access, use, or disclosure of personal information, including personal information regarding our customers or employees, could harm our reputation, compel us to comply with federal and/or state breach notification laws and foreign law equivalents, subject us to mandatory corrective action, and otherwise subject us to liability under laws and regulations that protect the privacy and security of personal information, which could result in significant legal and financial exposure and reputational damages. Any of the above could have a material adverse effect on our business, financial condition, results of operations or prospects.

***Our operations or those of the third parties upon whom we depend might be affected by the occurrence of a natural disaster, pandemic or other catastrophic event.***

We depend on our employees, consultants, CDMOs, CLROs, as well as regulatory agencies and other parties, for the continued operation of our business. While we maintain disaster recovery plans, they might not adequately protect us. Despite any precautions we take for natural disasters or other catastrophic events, these events, including terrorist attack, pandemics, hurricanes, fire, floods and ice and snowstorms, could result in significant disruptions to our research and development, preclinical studies, clinical trials, and, ultimately, commercialization of our products. Long-term disruptions in the infrastructure caused by events, such as natural disasters, the outbreak of war, the escalation of hostilities and acts of terrorism or other “acts of God,” particularly involving cities in which we have offices, manufacturing or clinical trial sites, could adversely affect our businesses. Although we carry business interruption insurance policies and typically have provisions in our contracts that protect us in certain events, our coverage might not respond or be adequate to compensate us for all losses that may occur. Any natural disaster or catastrophic event affecting us, our CDMOs, our CLROs, regulatory agencies or other parties with which we are engaged could have a significant negative impact on our operations and financial performance.

**Risks related to this offering, ownership of our common stock and our status as a public company**

***We do not know whether a market will develop for our common stock or what the market price of our common stock will be, and, as a result, it may be difficult for you to sell your shares of our common stock.***

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock will be determined through negotiations with the underwriters. Although we intend to apply to have our common stock approved for listing on the Nasdaq Global Market, an active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop, it may be difficult for you to sell shares you purchase in this offering without depressing the market price for the shares or at all.

***If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.***

The initial public offering price of our common stock will be substantially higher than the pro forma as adjusted net tangible book value per share of our common stock after this offering. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our pro forma as adjusted net tangible book value per share after this offering. Based on an assumed initial public offering price of \$ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, you will experience immediate dilution of \$ per share. To the extent outstanding options are exercised, you will incur further dilution.

***If securities analysts do not publish or cease publishing research or reports or publish misleading, inaccurate or unfavorable research about our business or if they publish negative evaluations of our stock, the price and trading volume of our stock could decline.***

The trading market for our common stock will rely, in part, on the research and reports that industry or financial analysts publish about us or our business. We do not currently have, and may never obtain, research coverage by industry or financial analysts. If no, or few, analysts commence coverage of us, the trading price of our stock would likely decrease. Even if we do obtain analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock or publish inaccurate or unfavorable research about our business, or provide more favorable relative recommendations about our competitors, the price of our stock

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could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price and trading volume to decline.

***The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock in this offering.***

Our stock price is likely to be volatile. The stock market in general and the market for smaller biopharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. The market price for our common stock may be influenced by many factors, including:

- results of or developments in preclinical studies and clinical trials of any product candidates we may develop or those of our competitors or potential collaborators;
- timing of the results of our preclinical studies and clinical trials or those of our competitors;
- our success in commercializing any product candidates we may develop, if and when approved;
- the success of competitive products or technologies;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other intellectual property or proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any product candidates we may develop;
- the results of our efforts to discover, develop, acquire or in-license products, product candidates, technologies or data referencing rights, the costs of commercializing any such products and the costs of development of any such product candidates or technologies;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- variations in our financial results or the financial results of companies that are perceived to be similar to us;
- sales of common stock by us, our executive officers, directors or principal stockholders or others;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry, political and market conditions; and
- the other factors described in this “Risk factors” section.

In the past, following periods of volatility in the market price of a company’s securities, securities class-action litigation has often been instituted against that company. Any lawsuit to which we are a party, with or without merit, may result in an unfavorable judgment. We also may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. Such litigation may also cause us to incur other substantial costs to defend such claims and divert management’s attention and resources.

***The COVID-19 pandemic, which began in late 2019 and has spread worldwide, may affect our ability to initiate and complete preclinical studies, delay the initiation of our planned clinical trial or future clinical trials, disrupt regulatory activities, or have other adverse effects on our business and operations. In addition, this pandemic has caused substantial disruption in the financial markets and may adversely impact economies worldwide, each of which could result in adverse effects on our business, on raising capital and on our operations.***

The COVID-19 pandemic, which began in December 2019 and has spread worldwide, is causing many governments to implement measures to slow the spread of the outbreak through quarantines, travel restrictions, heightened border scrutiny and other measures. The outbreak and government measures taken in response have also had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. The future progression of the outbreak and its effects on our business and operations are uncertain. We and our CDMOs, and CROs, have experienced a reduction in the capacity to undertake research-scale production and to execute some preclinical studies, and we may face disruptions that affect our ability to initiate and complete preclinical studies, and disruptions in procuring items that are essential for our research and development activities, such as raw materials used in the manufacture of any product candidates we may develop, laboratory supplies used in our preclinical studies, or animals that are used for preclinical testing for which there are shortages because of ongoing efforts to address the outbreak. We and our CROs and CDMOs may face disruptions related to our future IND-enabling studies and clinical trials arising from delays in preclinical studies, manufacturing disruptions, and the ability to obtain necessary IRB, IBC or other necessary site approvals, as well as other delays at clinical trial sites. The response to the COVID-19 pandemic may redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions. The pandemic has already caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could impact our ability to raise additional funds through public offerings and may also impact the volatility of our stock price and trading in our stock. Moreover, it is possible the pandemic will significantly impact economies worldwide, which could result in adverse effects on our business and operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to adversely affect our business, financial condition, results of operations and prospects.

***Unfavorable global economic conditions could adversely affect our business, financial condition, stock price and results of operations.***

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the 2008 global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the 2008 global financial crisis, could result in a variety of risks to our business, including, weakened demand for any product candidates we may develop and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive such difficult economic times, which could directly affect our ability to attain our operating goals on schedule and on budget. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial



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market conditions could adversely impact our business. Furthermore, our stock price may decline due in part to the volatility of the stock market and any general economic downturn.

***After this offering, our executive officers, directors and principal stockholders, if they choose to act together, will continue to have the ability to control all matters submitted to stockholders for approval.***

Upon the closing of this offering, based on the number of shares outstanding as of \_\_\_\_\_, our executive officers and directors and our stockholders who owned more than 5% of our outstanding common stock before this offering will, in the aggregate, beneficially own shares representing approximately \_\_\_\_\_% of our common stock. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these stockholders, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

This concentration of ownership control may:

- delay, defer or prevent a change in control;
- entrench our management and board of directors; or
- delay or prevent a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

***We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.***

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

***Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.***

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

***A significant portion of our total outstanding shares is restricted from immediate resale but may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is doing well.***

Sales of a substantial number of shares of our common stock in the public market, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. After this offering, we will have \_\_\_\_\_ shares of common stock outstanding based on the number of shares outstanding as of \_\_\_\_\_, 2020. This includes the shares that we are selling in this offering, which may be resold in the public market immediately without restriction, unless purchased by our affiliates. The remaining \_\_\_\_\_ shares are currently restricted as a result of securities laws or lock-up agreements but will become eligible to be sold at various times after the offering as described in the section of this prospectus titled "Shares eligible for future sale." The representatives of the underwriters may release some or all of the shares of common stock subject to lock-up agreements at any time and without notice, which would allow for earlier sales of shares in the public market.

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Moreover, beginning 180 days after the completion of this offering, holders of an aggregate of \_\_\_\_\_ shares of our common stock will have rights, along with holders of an additional shares of our common stock issuable upon exercise of outstanding options, subject to specified conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also intend to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described in the "Underwriters" section of this prospectus.

***We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.***

We are an "emerging growth company," or EGC, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. We may remain an EGC until the end of the fiscal year in which the fifth anniversary of this offering occurs, although if the market value of our common stock that is held by non-affiliates exceeds \$700.0 million as of any June 30 before that time or if we have annual gross revenues of \$1.07 billion or more in any fiscal year, we would cease to be an EGC as of December 31 of the applicable year. We also would cease to be an EGC if we issue more than \$1.0 billion of non-convertible debt over a three-year period. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include:

- being permitted to provide only two years of audited financial statements in this prospectus, in addition to any required unaudited interim financial statements, with correspondingly reduced "Management's discussion and analysis of financial condition and results of operations" disclosure;
- not being required to comply with the auditor attestation requirements in the assessment of our internal control over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We have taken advantage of reduced reporting obligations in this prospectus. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an EGC.

We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

In addition, the JOBS Act permits an EGC to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected to take advantage of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we will adopt the new or revised standard at the time private companies adopt the new or revised standard and will do so until such time that we either irrevocably elect to "opt out" of such extended transition period or no longer qualify as an EGC. We may choose to early adopt any new or revised accounting standards whenever such early adoption is permitted for private companies.

***We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.***

As a public company, and particularly after we are no longer an EGC, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs, particularly as we hire additional financial and accounting employees to meet public company internal control and financial reporting requirements and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, which in turn could make it more difficult for us to attract and retain qualified members of our board of directors.

We are evaluating these rules and regulations and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, in our second annual report due to be filed with the SEC after becoming a public company, we will be required to furnish a report by our management on our internal control over financial reporting. However, while we remain an EGC, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, including through hiring additional financial and accounting personnel, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses in our internal control over financial reporting, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

***If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results or prevent fraud. As a result, stockholders could lose confidence in our financial and other public reporting, which would harm our business and the trading price of our common stock.***

Effective internal control over financial reporting is necessary for us to provide reliable financial reports and, together with adequate disclosure controls and procedures, is designed to prevent fraud. Any failure to implement required new or improved controls, or difficulties encountered in their implementation could cause us to fail to meet our reporting obligations. In addition, any testing by us conducted in connection with Section 404, or any subsequent testing by our independent registered public accounting firm, may reveal

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deficiencies in our internal control over financial reporting that are deemed to be material weaknesses or that may require prospective or retroactive changes to our financial statements or identify other areas for further attention or improvement. Inferior internal controls could also cause investors to lose confidence in our reported financial information, which could harm our business and have a negative effect on the trading price of our stock.

We will be required to disclose changes made in our internal controls and procedures on a quarterly basis and our management will be required to assess the effectiveness of these controls annually. However, for as long as we are an EGC under the JOBS Act, our independent registered public accounting firm will not be required to attest to the effectiveness of our internal control over financial reporting pursuant to Section 404. We could be an EGC for up to five years. An independent assessment of the effectiveness of our internal control over financial reporting could detect problems that our management's assessment might not. Undetected material weaknesses in our internal control over financial reporting could lead to financial statement restatements and require us to incur the expense of remediation, which could have a negative effect on the trading price of our stock.

### ***Our disclosure controls and procedures may not prevent or detect all errors or acts of fraud.***

Upon completion of this offering, we will become subject to certain reporting requirements of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Our disclosure controls and procedures are designed to reasonably assure that information required to be disclosed by us in reports we file or submit under the Exchange Act is accumulated and communicated to management, recorded, processed, summarized and reported within the time periods specified in the rules and forms of the SEC. We believe that any disclosure controls and procedures or internal controls and procedures, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control system are met. These inherent limitations include the realities that judgments in decision-making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people or by an unauthorized override of the controls. Accordingly, because of the inherent limitations in our control system, misstatements or insufficient disclosures due to error or fraud may occur and not be detected.

### ***Changes in tax laws or in their implementation or interpretation may adversely affect our business and financial condition.***

Recent changes in tax law may adversely affect our business or financial condition. On December 22, 2017, the U.S. government enacted the Tax Act, which significantly reformed the Code. The Tax Act, among other things, contained significant changes to corporate taxation, including a reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, the limitation of the tax deduction for net interest expense to 30% of adjusted taxable income (except for certain small businesses), the limitation of the deduction for NOLs arising in taxable years beginning after December 31, 2017 to 80% of current year taxable income and elimination of NOL carrybacks for losses arising in taxable years ending after December 31, 2017 (though any such NOLs may be carried forward indefinitely), the imposition of a one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, the elimination of U.S. tax on foreign earnings (subject to certain important exceptions), the allowance of immediate deductions for certain new investments instead of deductions for depreciation expense over time, and the modification or repeal of many business deductions and credits.

As part of Congress' response to the COVID-19 pandemic, the Families First Coronavirus Response Act, or FFCR Act, was enacted on March 18, 2020, and the CARES Act was enacted on March 27, 2020. Both contain numerous tax provisions. In particular, the CARES Act retroactively and temporarily (for taxable years

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beginning before January 1, 2021) suspends application of the 80%-of-income limitation on the use of NOLs, which was enacted as part of the Tax Act. It also provides that NOLs arising in any taxable year beginning after December 31, 2017, and before January 1, 2021 are generally eligible to be carried back up to five years. The CARES Act also temporarily (for taxable years beginning in 2019 or 2020) relaxes the limitation of the tax deductibility for net interest expense by increasing the limitation from 30 to 50% of adjusted taxable income.

Regulatory guidance under the Tax Act, the FFCR Act and the CARES Act is and continues to be forthcoming, and such guidance could ultimately increase or lessen impact of these laws on our business and financial condition. It is also likely that Congress will enact additional legislation in connection with the COVID-19 pandemic, some of which could have an impact on our company. In addition, it is uncertain if and to what extent various states will conform to the Tax Act, the FFCR Act or the CARES Act.

***Provisions in our corporate charter documents and under Delaware law could make an acquisition of our company, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current directors and members of management.***

Provisions in our certificate of incorporation and our bylaws that will become effective upon the closing of this offering may discourage, delay or prevent a merger, acquisition or other change in control of our company that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that only one of three classes of directors is elected each year;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from our board of directors;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal specified provisions of our certificate of incorporation or bylaws that will become effective upon the closing of this offering.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

***Our certificate of incorporation that will become effective upon the closing of this offering designates the state courts in the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against the company and our directors, officers and employees.***

Our certificate of incorporation that will become effective upon the closing of this offering provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery of the State of Delaware does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for the following types of proceedings:

- any derivative action or proceeding brought on our behalf;
- any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders;
- any action asserting a claim arising pursuant to any provision of the DGCL or as to which the DGCL confers jurisdiction on the Court of Chancery of the State of Delaware; or
- any action asserting a claim arising pursuant to any provision of our certificate of incorporation or bylaws (in each case, as they may be amended from time to time) or governed by the internal affairs doctrine.

These choice of forum provisions will not apply to suits brought to enforce a duty or liability created by the Securities Act of 1933, as amended, the Exchange Act or any other claim for which federal courts have exclusive jurisdiction.

These exclusive forum provisions may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees. Alternatively, if a court were to find the choice of forum provisions contained in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could materially adversely affect our business, financial condition and operating results.

## Cautionary note regarding forward-looking statements and industry data

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical fact, contained in this prospectus, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management, are forward-looking statements. The words “anticipate,” “believe,” “continue” “could,” “estimate,” “expect,” “intend,” “may,” “might,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

The forward-looking statements in this prospectus include, among other things, statements about:

- the initiation, timing, progress and results of our research and development programs and preclinical studies and clinical trials;
- our estimates regarding expenses, future revenue, capital requirements, need for additional financing and the period over which we believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient to fund our operating expenses and capital expenditure requirements;
- our plans to develop and, if approved, subsequently commercialize any product candidates we may develop;
- the timing of and our ability to submit applications for, obtain and maintain regulatory approvals for any product candidates we may develop;
- the potential advantages of our non-viral gene therapy platform;
- our estimates regarding the potential addressable patient populations for our programs;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our expectations regarding our ability to obtain and maintain intellectual property protection;
- our intellectual property position;
- our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our commercial objectives;
- our expectations related to the use of proceeds from this offering;
- the impact of government laws and regulations;
- our competitive position and expectations regarding developments and projections relating to our competitors and any competing therapies that are or become available;
- developments and expectations regarding developments and projections relating to our competitors and our industry;
- our ability to maintain and establish collaborations or obtain additional funding; and
- our expectations regarding the time during which we will be an emerging growth company under the JOBS Act.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could

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differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this prospectus, particularly in the "Risk factors" section, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments we may make or enter into.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this prospectus are made as of the date of this prospectus, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

This prospectus includes statistical and other industry and market data that we obtained from independent industry publications and research, surveys and studies conducted by independent third parties as well as our own estimates of the prevalence of certain diseases and conditions. The market data used in this prospectus involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Our estimates of the patient population with the potential to benefit from treatment with any product candidates we may develop include several key assumptions based on our industry knowledge, industry publications, third-party research and other surveys, which may be based on a small sample size and may fail to accurately reflect the addressable patient population. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions.



## Use of proceeds

We estimate that the net proceeds to us from our issuance and sale of \_\_\_\_\_ shares of our common stock in this offering will be approximately \$ \_\_\_\_\_ million, assuming an initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise their option to purchase additional shares in full, we estimate that our net proceeds will be approximately \$ \_\_\_\_\_ million.

A \$1.00 increase (decrease) in the assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the net proceeds to us from this offering by approximately \$ \_\_\_\_\_ million, assuming the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the net proceeds to us from this offering by approximately \$ \_\_\_\_\_ million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

As of December 31, 2019, we had cash and cash equivalents of \$15.1 million, and in January 2020, we received gross proceeds of \$111.5 million from the sale of our Series C preferred stock. We currently estimate that we will use the net proceeds from this offering, together with our existing cash and cash equivalents, as follows:

- approximately \$ \_\_\_\_\_ million for continued research and development of our programs, including preclinical and IND-enabling studies;
- approximately \$ \_\_\_\_\_ million for continued development and enhancement of our platform technologies; and
- the remainder for working capital and other general corporate purposes.

Our expected use of net proceeds from this offering and our existing cash and cash equivalents represents our intentions based upon our current plans and business conditions, which could change in the future as our plans and business conditions evolve. We believe opportunities may exist from time to time to expand our current business through acquisitions of complementary companies, products or technologies. While we have no current agreements, commitments or understandings for any specific acquisitions at this time, we may use a portion of the net proceeds for these purposes. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including results from our research and development efforts for each program, the timing and success of our preclinical studies and the timing and outcome of regulatory submissions, as well as any collaborations that we may enter into with third parties for any product candidates we may develop, and any unforeseen cash needs. Our management will retain broad discretion over the allocation of the net proceeds from this offering.

Based on our current plans, we believe that anticipated net proceeds from this offering, together with our existing cash and cash equivalents, will be sufficient to enable us to fund our operating expenses and capital expenditure requirements through \_\_\_\_\_. We have based our estimates as to how long we expect we will be able to fund our operations on assumptions that may prove to be wrong. We could use our available capital resources sooner than we currently expect, in which case we would be required to obtain additional financing, which may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

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Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments and U.S. government securities.

## **Dividend policy**

We have never declared or paid cash dividends on our common stock since our inception. We currently intend to retain all available funds and any future earnings to fund the development and expansion of our business and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination to declare and pay dividends will be made at the discretion of our board of directors and will depend on then-existing conditions, including our results of operations, financial condition, contractual restrictions, capital requirements, business prospects and other factors our board of directors may deem relevant.

## Capitalization

The following table sets forth our cash and cash equivalents and our capitalization as of December 31, 2019:

- on an actual basis;
- on a pro forma basis to give effect to the conversion of all outstanding shares of our preferred stock into an aggregate of 47,856,346 shares of common stock upon the closing of this offering, and the filing and effectiveness of our amended and restated certificate of incorporation; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of \_\_\_\_\_ shares of our common stock in this offering at an assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The pro forma as adjusted information below is illustrative only, and our capitalization following the closing of this offering will change based on the actual initial public offering price and other terms of this offering determined at pricing. You should read the information in this table, together with our consolidated financial statements and the related notes appearing elsewhere in this prospectus and the “Selected consolidated financial data” and “Management’s discussion and analysis of financial condition and results of operations” sections of this prospectus.

| (in thousands, except share and per share data)  | As of December 31, 2019 |           |                       |
|--|-------------------------|-----------|-----------------------|
|  | Actual                  | Pro forma | Pro forma as adjusted |
| Cash and cash equivalents  | \$ 15,076               | \$        | \$                    |
| Convertible preferred stock (Series A and B), \$0.0001 par value; 26,425,664 shares authorized, 26,425,664 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted   | 115,593                 |           |                       |
| Stockholders' equity (deficit)   |                         |           |                       |
| Preferred stock, \$0.0001 par value; no shares authorized, issued or outstanding, actual; _____ shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted   | —                       |           |                       |
| Common stock, \$0.0001 par value; 46,750,000 shares authorized, 12,321,881 shares issued and 9,310,006 shares outstanding at December 31, 2019, actual; _____ shares authorized, _____ shares issued and _____ shares outstanding, pro forma; _____ shares authorized, _____ shares issued and _____ shares outstanding, pro forma as adjusted | 1                       |           |                       |
| Additional paid-in capital   | 9,859                   |           |                       |
| Accumulated deficit  | (108,452)               |           |                       |
| Total stockholders' equity (deficit)   | (98,592)                |           |                       |
| Total capitalization   | \$ 17,001               | \$        | \$                    |

A \$1.00 increase (decrease) in the assumed initial public offering price of \$ \_\_\_\_\_ per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, additional paid-in capital, total stockholders' equity and total capitalization by \$ \_\_\_\_\_ million, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts

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and commissions and estimated offering expenses payable by us. An increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents, additional paid-in capital, total stockholders' equity and total capitalization by \$        million, assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The table above is based on 12,321,881 shares of common stock outstanding as of December 31, 2019, which includes 3,011,875 shares of unvested restricted stock subject to repurchase by us, and excludes:

- 5,959,602 shares of common stock issuable upon the exercise of stock options outstanding as of December 31, 2019 under our 2017 Stock Incentive Plan, or the 2017 Plan, at a weighted average exercise price of \$2.69 per share (which does not include options to purchase an aggregate of 3,052,950 shares of common stock, at a weighted average exercise price of \$2.92 per share, that were granted subsequent to December 31, 2019);
- 1,366,502 shares of common stock available for future issuance as of December 31, 2019 under our 2017 Plan; and
- and            additional shares of common stock that will become available for issuance under our 2020 Stock Incentive Plan and our 2020 Employee Stock Purchase Plan, respectively, each of which will become effective immediately prior to the effectiveness of the registration statement of which this prospectus is a part, as well as any automatic increases in the number of shares of common stock reserved for future issuance under these plans.

## Dilution

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock immediately after this offering.

Our historical net tangible book value (deficit) as of December 31, 2019 was \$(98.7) million, or \$(8.01) per share of common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities and the carrying value of our preferred stock, which is not included within stockholders' equity (deficit). Historical net tangible book value (deficit) per share represents historical net tangible book value (deficit) divided by the 12,321,881 shares of common stock outstanding as of December 31, 2019, including 3,011,875 shares of unvested restricted stock subject to repurchase by us.

Our pro forma net tangible book value as of December 31, 2019 was \$        million, or \$        per share of common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to the conversion of all outstanding shares of our convertible preferred stock into an aggregate of 47,856,346 shares of common stock upon the closing of this offering. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of December 31, 2019, after giving effect to the pro forma adjustment described above.

After giving further effect to our issuance and sale of        shares of our common stock in this offering at an assumed initial public offering price of \$        per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of December 31, 2019 would have been \$        million, or \$        per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of \$        to existing stockholders and immediate dilution of \$        in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the assumed initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

|  |          |
|--|----------|
| Assumed initial public offering price per share  | \$       |
| Historical net tangible book value (deficit) per share as of December 31, 2019   | \$       |
| Increase per share attributable to the pro forma adjustment described above  | _____    |
| Pro forma net tangible book value per share as of December 31, 2019  | _____    |
| Increase in pro forma as adjusted net tangible book value per share attributable to new investors purchasing common stock in this offering | _____    |
| Pro forma as adjusted net tangible book value per share after this offering  | _____    |
| Dilution per share to new investors purchasing common stock in this offering   | \$ _____ |

The dilution information discussed above is illustrative only and will change based on the actual initial public offering price and other terms of this offering determined at pricing. A \$1.00 increase (decrease) in the assumed initial public offering price of \$        per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) our pro forma as adjusted net tangible book value per share after this offering by \$        and dilution per share to new investors purchasing common stock in this offering by \$       , assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. An increase of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase our pro forma as adjusted net tangible

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book value per share after this offering by \$ [redacted] and decrease the dilution per share to new investors purchasing common stock in this offering by \$ [redacted], assuming no change in the assumed initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. A decrease of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would decrease our pro forma as adjusted net tangible book value per share after this offering by \$ [redacted] and increase the dilution per share to new investors purchasing common stock in this offering by \$ [redacted], assuming no change in the assumed initial public offering price and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

If the underwriters exercise their option to purchase additional shares in full, our pro forma as adjusted net tangible book value per share after this offering would be \$ [redacted], representing an immediate increase in pro forma as adjusted net tangible book value per share of \$ [redacted] to existing stockholders and immediate dilution in pro forma as adjusted net tangible book value per share of \$ [redacted] to new investors purchasing common stock in this offering, assuming an initial public offering price of \$ [redacted] per share, which is the midpoint of the price range set forth on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The following table summarizes, as of December 31, 2019, on the pro forma as adjusted basis described above, the total number of shares of common stock purchased from us on an as converted to common stock basis, the total consideration paid or to be paid and the average price per share paid or to be paid by existing stockholders and by new investors in this offering at an assumed initial public offering price of \$ [redacted] per share, which is the midpoint of the price range set forth on the cover page of this prospectus, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. As the table shows, new investors purchasing common stock in this offering will pay an average price per share substantially higher than our existing stockholders paid.

|  | <u>Shares purchased</u> |                | <u>Total consideration</u> |                   | <u>Average price per share</u> |
|--|-------------------------|----------------|----------------------------|-------------------|--------------------------------|
|  | <u>Number</u>           | <u>Percent</u> | <u>Amount</u>              | <u>Percentage</u> |                                |
| Existing stockholders                    |                         | %              | \$                         | %                 | \$                             |
| Investors participating in this offering |                         |                |                            |                   | \$                             |
| <b>Total</b>                             |                         | <b>100%</b>    | <b>\$</b>                  | <b>100%</b>       |                                |

A \$1.00 increase (decrease) in the assumed initial public offering price of \$ [redacted] per share, which is the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the total consideration paid by new investors by \$ [redacted] million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by [redacted] percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by [redacted] percentage points, assuming that the number of shares offered by us, as set forth on the cover page of this prospectus, remains the same. An increase (decrease) of 1,000,000 shares in the number of shares offered by us, as set forth on the cover page of this prospectus, would increase (decrease) the total consideration paid by new investors by \$ [redacted] million and, in the case of an increase, would increase the percentage of total consideration paid by new investors by [redacted] percentage points and, in the case of a decrease, would decrease the percentage of total consideration paid by new investors by [redacted] percentage points, assuming no change in the assumed initial public offering price.

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is exercised in full, the number of shares of our common stock held by existing stockholders would be reduced to [redacted] % of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors

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purchasing common stock in this offering would be increased to \_\_\_\_\_ % of the total number of shares of our common stock outstanding after this offering.

The tables and discussion above are based on 12,321,881 shares of common stock outstanding as of December 31, 2019, which includes 3,011,875 shares of unvested restricted stock subject to repurchase by us, and excludes:

- 5,959,602 shares of common stock issuable upon the exercise of stock options outstanding as of December 31, 2019 under our 2017 Plan at a weighted average exercise price of \$2.69 per share (which does not include options to purchase an aggregate of 3,052,950 shares of common stock, at a weighted average exercise price of \$2.92 per share, that were granted subsequent to December 31, 2019);
- 1,366,502 shares of common stock available for future issuance as of December 31, 2019 under our 2017 Plan; and
- \_\_\_\_\_ and \_\_\_\_\_ additional shares of common stock that will become available for issuance under our 2020 Stock Incentive Plan and our 2020 Employee Stock Purchase Plan, respectively, each of which will become effective immediately prior to the effectiveness of the registration statement of which this prospectus is a part, as well as any automatic increases in the number of shares of common stock reserved for future issuance under these plans.

To the extent that outstanding stock options are exercised, new stock options or warrants are issued, or we issue additional shares of common stock in the future, there will be further dilution to new investors. In addition, we may choose to raise additional capital because of market conditions or strategic considerations, even if we believe that we have sufficient funds for our current or future operating plans. If we raise additional capital through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders.



## Selected consolidated financial data

You should read the following selected consolidated financial data together with our consolidated financial statements and the related notes appearing elsewhere in this prospectus and the "Management's discussion and analysis of financial condition and results of operations" section of this prospectus. We have derived the consolidated statement of operations data for the years ended December 31, 2018 and 2019 and the consolidated balance sheet data as of December 31, 2018 and 2019 from our audited consolidated financial statements appearing elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that may be expected in the future.

| (in thousands, except per share data)  | Year ended<br>December 31, |            |
|--|----------------------------|------------|
|  | 2018                       | 2019       |
| <b>Consolidated statement of operations data:</b>                                      |                            |            |
| Revenue  | \$ 36                      | \$ —       |
| Operating expenses:  |                            |            |
| Research and development   | 28,152                     | 50,134     |
| General and administrative   | 9,178                      | 12,168     |
| Total operating expenses   | 37,330                     | 62,302     |
| Loss from operations   | (37,294)                   | (62,302)   |
| Other income (expense):  |                            |            |
| Interest income and other income (expense), net  | 1,491                      | 985        |
| Net loss and net loss attributable to common stockholders                              | \$(35,803)                 | \$(61,317) |
| Net loss per share attributable to common stockholders, basic and diluted              | \$ (6.05)                  | \$ (7.34)  |
| Weighted average common shares outstanding, basic and diluted                          | 5,918                      | 8,357      |
| Pro forma net loss per share attributable to common stockholders, basic and diluted(1) |                            | \$ (1.76)  |
| Pro forma weighted average common shares outstanding, basic and diluted(1)             |                            | 34,783     |

(1) See Notes 2 and 12 to our consolidated financial statements appearing elsewhere in this prospectus for details on the calculation of unaudited pro forma net loss per share attributable to common stockholders.

| (in thousands)                          | As of December 31, |           |
|---|--------------------|-----------|
|   | 2018               | 2019      |
| <b>Consolidated Balance Sheet Data:</b> |                    |           |
| Cash and cash equivalents               | \$ 7,305           | \$ 15,076 |
| Marketable securities                   | 67,565             | —         |
| Working capital(1)                      | 70,371             | 8,998     |
| Total assets                            | 83,519             | 42,140    |
| Convertible preferred stock             | 115,593            | 115,593   |
| Total stockholders' deficit             | (41,591)           | (98,592)  |

(1) We define working capital as current assets less current liabilities.

# Management's discussion and analysis of financial condition and results of operations

*You should read the following discussion and analysis of our financial condition and results of operations together with the "Selected consolidated financial data" section of this prospectus and our consolidated financial statements and the related notes and other financial information included elsewhere in of this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.*

## Overview

We are an innovative genetic medicines company creating a new class of gene therapy utilizing our proprietary non-viral gene therapy platform to provide durable, redosable treatments for millions of patients living with rare and prevalent diseases. Our non-viral gene therapy platform incorporates our high-capacity DNA construct called closed-ended DNA, or ceDNA; our cell-targeted lipid nanoparticle delivery system, or ctLNP; and our established, scalable capsid-free manufacturing process. Using our approach, we are developing novel gene therapies to provide targeted delivery of genetic payloads that include large and multiple genes to a range of tissues across a broad array of diseases. We are also engineering our gene therapies to be redosable, which may enable individualized patient titration to reach the desired level of therapeutic expression and to maintain efficacy throughout a patient's life.

Since our inception in October 2016, we have focused substantially all of our resources on building our non-viral gene therapy platform, establishing and protecting our intellectual property portfolio, conducting research and development activities, developing our manufacturing process, organizing and staffing our company, business planning, raising capital and providing general and administrative support for these operations. We do not have any products approved for sale and have not generated any revenue from product sales. To date, we have funded our operations with proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017) and the sales of convertible preferred stock. Through December 31, 2019, we had received gross proceeds of \$115.8 million from sales of preferred stock and instruments convertible into preferred stock. In addition, in January 2020, we received gross proceeds of \$111.5 million from the sale of 19,936,296 shares of Series C preferred stock. Since our inception, we have incurred significant operating losses. Our ability to generate any product revenue or product revenue sufficient to achieve profitability will depend on the successful development and eventual commercialization of one or more product candidates we may develop. For the years ended December 31, 2018 and 2019, we reported net losses of \$35.8 million and \$61.3 million, respectively. As of December 31, 2019, we had an accumulated deficit of \$108.5 million. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect that our expenses and capital requirements will increase substantially in connection with our ongoing activities, particularly if and as we:

- continue our current research programs and conduct additional research programs;
- advance any product candidates we identify into preclinical and clinical development;
- expand the capabilities of our non-viral gene therapy platform;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- obtain, expand, maintain, enforce and defend our intellectual property portfolio;

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- hire additional clinical, regulatory and scientific personnel;
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- establish a commercial manufacturing source and secure supply chain capacity sufficient to provide commercial quantities of any product candidates for which we may obtain regulatory approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development, future commercialization efforts and operations as a public company.

We will not generate revenue from product sales unless and until we successfully complete clinical development and obtain regulatory approval for any product candidates we may develop. If we obtain regulatory approval for any product candidates we may develop, we expect to incur significant expenses related to developing our commercial capability to support product sales, marketing and distribution. Further, following the completion of this offering, we expect to incur additional costs associated with operating as a public company.

As a result, we will need substantial additional funding to support our continuing operations and pursue our growth strategy. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. We may be unable to raise additional funds or enter into such other agreements or arrangements when needed on favorable terms, or at all. If we fail to raise capital or enter into such agreements when needed or on terms acceptable to us, we would be required to delay, limit, reduce or terminate our product development or future commercialization of one or more of our product candidates.

Because of the numerous risks and uncertainties associated with pharmaceutical product development, we are unable to accurately predict the timing or amount of increased expenses or when, or if, we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

We believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital expenditures through . We have based our estimates as to how long we expect we will be able to fund our operations on assumptions that may prove to be wrong. We could use our available capital resources sooner than we currently expect, in which case we would be required to obtain additional financing, which may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. See “—Liquidity and capital resources.”

## **Components of our results of operations**

### **Revenue**

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from the sale of products in the near future. If our development efforts for our product candidates are successful and result in regulatory approval or license or collaboration agreements with third parties, we may generate revenue in the future from product sales, payments from collaboration or license agreements that we may enter into with third parties, or any combination thereof.

We have previously generated a small amount of revenue by providing services to pharmaceutical and life sciences companies. We did not generate any such revenue in 2019.

## **Operating expenses**

### *Research and development expenses*

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts, and the development of our programs, which include:

- personnel-related costs, including salaries, benefits and stock-based compensation expense, for employees engaged in research and development functions;
- expenses incurred in connection with our research programs, including under agreements with third parties, such as consultants and contractors and contract research organizations, or CROs;
- the cost of developing and scaling our manufacturing process and manufacturing drug substance and drug product for use in our research and preclinical studies, including under agreements with third parties, such as consultants and contractors and contract development and manufacturing organizations, or CDMOs;
- laboratory supplies and research materials;
- facilities, depreciation and amortization and other expenses, which include direct and allocated expenses for rent and maintenance of facilities and insurance; and
- payments made under third-party licensing agreements.

We expense research and development costs as incurred. Advance payments that we make for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

Our external research and development expenses consist of costs that include fees and other costs paid to consultants, contractors, CDMOs and CROs in connection with our preclinical and manufacturing activities. We do not allocate our research and development costs to specific programs because costs are deployed across multiple programs and our platform and, as such, are not separately classified. We expect that our research and development expenses will increase substantially as we advance our programs into clinical development and expand our discovery, research and preclinical activities in the near term and in the future. At this time, we cannot accurately estimate or know the nature, timing and costs of the efforts that will be necessary to complete the preclinical and clinical development of any product candidates we may develop. The successful development of any of our product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with product development, including the following:

- the timing and progress of preclinical studies, including investigational new drug, or IND, -enabling studies;
- the number and scope of preclinical and clinical programs we decide to pursue;
- raising additional funds necessary to complete preclinical and clinical development of our product candidates;
- the timing of filing and acceptance of INDs or comparable foreign applications that allow commencement of future clinical trials for our product candidates;
- the successful initiation, enrollment and completion of clinical trials, including under current good clinical practices;
- our ability to achieve positive results from our future clinical programs that support a finding of safety and effectiveness and an acceptable risk-benefit profile in the intended patient populations of any product candidates we may develop;

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- the availability of specialty raw materials for use in production of our product candidates;
- our ability to establish arrangements with third-party manufacturers for clinical supply;
- our ability to establish new licensing or collaboration arrangements;
- the receipt and related terms of regulatory approvals from the U.S. Food and Drug Administration, or FDA, and other applicable regulatory authorities;
- our ability to establish, obtain, maintain, enforce and defend patent, trademark, trade secret protection and other intellectual property rights or regulatory exclusivity for any product candidates we may develop and our technology; and
- our ability to maintain a continued acceptable safety, tolerability and efficacy profile of our product candidates following approval.

A change in the outcome of any of these variables with respect to any product candidates we may develop could significantly change the costs and timing associated with the development of that product candidate. We may never succeed in obtaining regulatory approval for any product candidates we may develop.

### *General and administrative expenses*

General and administrative expenses consist primarily of personnel-related costs, including salaries, benefits and stock-based compensation, for employees engaged in executive, legal, finance and accounting and other administrative functions. General and administrative expenses also include professional fees for legal, patent, consulting, investor and public relations and accounting and audit services as well as direct and allocated facility-related costs.

We anticipate that our general and administrative expenses will increase in the future as we increase our headcount to support our continued research activities and development of our programs and platform. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance, director and officer insurance costs and investor and public relations expenses associated with operating as a public company.

### **Other income (expense)**

#### *Interest income and other income (expense), net*

Interest income consists of interest earned on our invested cash balances. We expect our interest income to increase as we invest the cash received from the sale of Series C preferred stock in January 2020 and the net proceeds from this offering.

Other income (expense) consists of miscellaneous income and expense unrelated to our core operations.

### **Income taxes**

Since our inception, we have not recorded any income tax benefits for the net losses we have incurred or for the research and development tax credits earned in each year, as we believe, based upon the weight of available evidence, that it is more likely than not that all of our net operating loss carryforwards and tax credit carryforwards will not be realized.

As of December 31, 2019, we had federal net operating loss carryforwards of \$91.9 million, which may be available to offset future taxable income, of which \$8.2 million of the total net operating loss carryforwards begin to expire in 2036, while the remaining \$83.7 million do not expire but may be limited in their usage to an annual deduction equal to 80% of annual taxable income. In addition, as of December 31, 2019, we had state

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net operating loss carryforwards of \$90.6 million, which may be available to offset future taxable income and expire at various dates beginning in 2036. As of December 31, 2019, we also had federal and state research and development tax credit carryforwards of \$4.0 million and \$2.5 million, respectively, which may be available to reduce future tax liabilities and expire at various dates beginning in 2036 and 2032, respectively. Due to our history of cumulative net losses since inception and uncertainties surrounding our ability to generate future taxable income, we have recorded a full valuation allowance against our net deferred tax assets at each balance sheet date.

## Results of operations

### Comparison of the years ended December 31, 2018 and 2019

The following table summarizes our results of operations for the years ended December 31, 2018 and 2019:

| (in thousands)                                  | Year ended<br>December 31, |            | Change     |
|---|----------------------------|------------|------------|
|   | 2018                       | 2019       |            |
| Revenue   | \$ 36                      | \$ —       | \$ (36)    |
| Operating expenses:                             |                            |            |            |
| Research and development                        | 28,152                     | 50,134     | 21,982     |
| General and administrative                      | 9,178                      | 12,168     | 2,990      |
| Total operating expenses                        | 37,330                     | 62,302     | 24,972     |
| Loss from operations                            | (37,294)                   | (62,302)   | (25,008)   |
| Other income (expense):                         |                            |            |            |
| Interest income and other income (expense), net | 1,491                      | 985        | (506)      |
| Net loss  | \$(35,803)                 | \$(61,317) | \$(25,514) |

#### Revenue

We recorded less than \$0.1 million of revenue for the year ended December 31, 2018 for services rendered. We did not generate revenue in 2019.

#### Research and development expenses

The following table summarizes our research and development expenses for the years ended December 31, 2018 and 2019:

| (in thousands)                          | Year Ended<br>December 31, |          | Change   |
|---|----------------------------|----------|----------|
|   | 2018                       | 2019     |          |
| Personnel related                       | \$ 8,705                   | \$12,847 | \$ 4,142 |
| Stock-based compensation                | 2,093                      | 2,753    | 660      |
| Preclinical and manufacturing           | 7,359                      | 15,027   | 7,668    |
| Facilities                              | 2,835                      | 9,553    | 6,718    |
| Lab supplies                            | 3,200                      | 4,124    | 924      |
| Consulting and professional services    | 2,377                      | 2,629    | 252      |
| Other                                   | 1,583                      | 3,201    | 1,618    |
| Total research and development expenses | \$28,152                   | \$50,134 | \$21,982 |

Research and development expenses were \$28.2 million for the year ended December 31, 2018, compared to \$50.1 million for the year ended December 31, 2019. The increase in personnel-related costs of \$4.1 million was

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primarily due to increased headcount in our research and development function. The increase in preclinical and manufacturing expense of \$7.7 million was primarily due to developing and scaling our manufacturing process, advancing our lead programs towards development candidates and advancing our discovery efforts. The increase in facility-related expenses and lab supplies of \$6.7 million and \$0.9 million, respectively, was primarily due to rent expense for our new facility in Cambridge, Massachusetts, for which we recorded a full year of rent expense in 2019 compared to a partial year in 2018, and the increased costs of supporting a larger group of research and development personnel and their research efforts. The increase in other expense of \$1.6 million was primarily due to an increase in technology licensing fees.

### *General and administrative expenses*

The following table summarizes our general and administrative expenses for the years ended December 31, 2018 and 2019:

| <b>(in thousands)</b>                     | <b>Year ended</b>   |             | <b>Change</b> |
|---|---------------------|-------------|---------------|
|   | <b>December 31,</b> |             |               |
|   | <b>2018</b>         | <b>2019</b> |               |
| Personnel related                         | \$2,997             | \$ 4,279    | \$ 1,282      |
| Stock-based compensation                  | 1,395               | 1,454       | 59            |
| Professional and consultant fees          | 3,319               | 4,465       | 1,146         |
| Facilities                                | 623                 | 1,217       | 594           |
| Other                                     | 844                 | 753         | (91)          |
| Total general and administrative expenses | \$9,178             | \$12,168    | \$ 2,990      |

General and administrative expenses for the year ended December 31, 2018 were \$9.2 million, compared to \$12.2 million for the year ended December 31, 2019. The increase in personnel-related costs of \$1.3 million was a result of an increase in headcount in our general and administrative function. Professional and consultant fees increased by \$1.1 million primarily due to professional fees relating to accounting, audit and legal services as well as costs associated with ongoing business activities and our preparations to operate as a public company. The increase in facility-related expenses of \$0.6 million was primarily due to rent expense for our new facility in Cambridge, Massachusetts.

### *Other income and expense, net*

Other income and expense, net for the year ended December 31, 2018 was \$1.5 million, compared to \$1.0 million for the year ended December 31, 2019. The decrease was primarily due to a reduction in interest income as we had a lower average invested balance during 2019.

Other expense was not significant for either of the years ended December 31, 2018 or 2019.

## **Liquidity and capital resources**

Since our inception in October 2016, we have incurred significant operating losses. We expect to incur significant expenses and operating losses for the foreseeable future as we support our continued research activities and development of our programs and platform. To date, we have funded our operations with proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017) and the sale of convertible preferred stock. Through December 31, 2019, we had received gross proceeds of \$115.8 million from sales of convertible preferred stock and instruments convertible into convertible preferred stock. As of December 31, 2019, we had cash and cash equivalents of \$15.1 million. In January 2020, we received gross proceeds of \$111.5 million from the sale of 19,936,296 shares of Series C preferred stock.

[Table of Contents](#)**Cash flows**

The following table summarizes our sources and uses of cash for each of the periods presented:

| <b>(in thousands)</b>                                      | <b>Year ended<br/>December 31,</b> |             |
|--|------------------------------------|-------------|
|  | <b>2018</b>                        | <b>2019</b> |
| Cash used in operating activities                          | \$ (28,119)                        | \$ (40,346) |
| Cash provided by (used in) investing activities            | (69,761)                           | 47,985      |
| Cash provided by financing activities                      | 100,189                            | 78          |
| Net increase in cash, cash equivalents and restricted cash | \$ 2,309                           | \$ 7,717    |

**Operating activities**

During the year ended December 31, 2019, operating activities used \$40.3 million of cash, primarily resulting from our net loss of \$61.3 million, partially offset by net non-cash charges of \$5.7 million and net cash provided by changes in our operating assets and liabilities of \$15.2 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2019 consisted primarily of a \$13.0 million increase in deferred rent and a \$3.2 million increase in accounts payable and accrued expenses and other current liabilities, partially offset by a \$1.9 million increase in prepaid expenses and other current assets.

During the year ended December 31, 2018, operating activities used \$28.1 million of cash, primarily resulting from our net loss of \$35.8 million, partially offset by net non-cash charges of \$3.0 million and net cash provided by changes in our operating assets and liabilities of \$4.7 million. Net cash provided by changes in our operating assets and liabilities for the year ended December 31, 2018 consisted primarily of a \$3.0 million increase in deferred rent and a \$3.5 million increase in accounts payable and accrued expenses and other current liabilities, partially offset by a \$1.3 million increase in tenant receivable and a \$0.5 million increase in prepaid expenses and other current assets.

Changes in accounts payable, accrued expenses and other current liabilities and prepaid expenses and other current assets in both periods were generally due to growth in our business, the advancement of our research programs and the timing of vendor invoicing and payments. The increase in deferred rent in both periods primarily related to a tenant improvement allowance from our landlord.

**Investing activities**

During the year ended December 31, 2019, net cash provided by investing activities was \$48.0 million, due primarily to net sales and maturities of marketable securities, partially offset by the acquisition of property and equipment during the year. Property and equipment purchases during the year ended December 31, 2019 primarily related to leasehold improvements and lab equipment for our new facility in Cambridge, Massachusetts.

During the year ended December 31, 2018, net cash used in investing activities was \$69.8 million, due primarily to net purchases of marketable securities and property and equipment. Property and equipment purchases during the year ended December 31, 2018 primarily related to leasehold improvements and lab equipment for our new facility in Cambridge, Massachusetts.

**Financing activities**

During the year ended December 31, 2019, net cash provided by financing activities was \$0.1 million, consisting primarily of proceeds from the exercise of common stock options.



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During the year ended December 31, 2018, net cash provided by financing activities was \$100.2 million, consisting of net proceeds from the sale of our Series B preferred stock.

### **Funding requirements**

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance the preclinical activities and initiate clinical trials for our product candidates in development. The timing and amount of our operating expenditures will depend largely on:

- the identification of additional research programs and additional product candidates;
- the scope, progress, costs and results of preclinical and clinical development for any product candidates we may develop;
- the costs, timing and outcome of regulatory review of any product candidates we may develop;
- the cost and timing of completion of commercial-scale manufacturing activities;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any product candidates we may develop for which we receive marketing approval;
- the costs and scope of the continued development of our non-viral gene therapy platform;
- the costs of satisfying any post-marketing requirements;
- the revenue, if any, received from commercial sales of product candidates we may develop for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting applications for patents, obtaining, maintaining, defending and enforcing our intellectual property rights and defending against any intellectual property-related claims, including claims of infringement, misappropriation or other violation of third-party intellectual property;
- the costs of operational, financial and management information systems and associated personnel;
- the associated costs in connection with any acquisition of in-licensed products, intellectual property and technologies; and
- the costs of operating as a public company.

We believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital expenditure requirements through . We have based our estimates as to how long we expect we will be able to fund our operations on assumptions that may prove to be wrong. We could use our available capital resources sooner than we currently expect, in which case we would be required to obtain additional financing, which may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We do not have any committed external source of funds. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. If we raise additional funds by issuing equity securities, our stockholders may experience dilution. Any future debt financing into which we enter would result in fixed payment obligations and may involve agreements that include grants of security interests on our assets and restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, granting liens over our assets, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. Any debt financing or additional equity that we raise may contain terms that could adversely affect the holdings or the rights of our common stockholders.

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If we are unable to raise sufficient capital as and when needed, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product candidate we may develop, or be unable to expand our operations or otherwise capitalize on our business opportunities. If we raise additional funds through collaborations or licensing arrangements with third parties, we may have to relinquish valuable rights to future revenue streams or product candidates or grant licenses on terms that may not be favorable to us.

See "Risk factors" for additional risks associated with our substantial capital requirements.

### **Contractual obligations and commitments**

The following table summarizes our contractual obligations as of December 31, 2019 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

| (in thousands)                 | Total    | Payments due by period |              |              |                   |
|--------------------------------|----------|------------------------|--------------|--------------|-------------------|
|                                |          | Less than 1 year       | 1 to 3 years | 4 to 5 years | More than 5 years |
| Operating lease commitments(1) | \$71,742 | \$ 6,867               | \$ 14,304    | \$ 15,120    | \$ 35,451         |
| Total                          | \$71,742 | \$ 6,867               | \$ 14,304    | \$ 15,120    | \$ 35,451         |

(1) Amounts in table reflect payments due for our lease of office and laboratory space in Cambridge, Massachusetts under an operating lease agreement that expires in April 2029.

We enter into contracts in the normal course of business with CROs, CDMOs and other third parties for preclinical research studies and manufacturing services. These contracts do not contain minimum purchase commitments and are cancelable by us upon prior written notice. Payments due upon cancellation consist only of payments for services provided or expenses incurred, including noncancelable obligations of our service providers, up to the date of cancellation. These payments are not included in the table of contractual obligations above as the amount and timing of such payments are not known.

We have also entered into license agreements under which we are obligated to make specified milestone and royalty payments. We have not included future payments under these agreements in the table of contractual obligations above since the payment obligations under these agreements are contingent upon future events, such as our achievement of specified development, regulatory and commercial milestones, or generating product sales. As of December 31, 2019, we were unable to estimate the timing or likelihood of achieving these milestones or generating future product sales. For additional information about our licenses agreements and amounts that could become payable in the future under such agreements, see "Business—Intellectual Property—License Agreements" and Note 10 to our consolidated financial statements appearing elsewhere in this prospectus.

### **Critical accounting policies and significant judgments and estimates**

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States, or GAAP. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue, costs and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions or conditions.

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While our significant accounting policies are described in more detail in Note 2 to our consolidated financial statements appearing elsewhere in this prospectus, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

### ***Accrued research and development expenses***

As part of the process of preparing our consolidated financial statements, we are required to estimate certain accrued research and development expenses. This process involves estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual costs. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of the estimates with the service providers and make adjustments if necessary. Examples of estimated accrued research and development expenses include those related to fees paid to:

- vendors in connection with discovery and preclinical development activities;
- CROs in connection with preclinical studies and testing; and
- CDMOs in connection with the process development and scale up activities and the production of materials.

We base the expense recorded related to contract research and manufacturing on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CROs and CDMOs that conduct services and supply materials. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from the estimate, we adjust the accrual accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in reporting amounts that are too high or too low in any particular period. To date, there have not been any material adjustments to our prior estimates of accrued research and development expenses. While the majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; some require advance payments. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. We record these as prepaid expenses on our consolidated balance sheet.

### ***Stock-based compensation***

We measure all stock-based awards granted to employees, non-employees and directors based on their fair value on the date of the grant using the Black-Scholes option-pricing model for options or the difference between the purchase price, if any, and the fair value of our common stock for restricted stock awards. Compensation expense for awards with service-based vesting is generally recognized over the vesting period of the award using the straight-line method to record the expense. We use the graded-vesting method to record the expense of awards with both service-based and performance-based vesting conditions, commencing once achievement of the performance condition becomes probable. We account for forfeitures of share-based awards as they occur.

The Black-Scholes option-pricing model uses as inputs the fair value of our common stock and assumptions we make for the expected volatility of our common stock, the expected term of stock options, the risk-free interest rate for a period that approximates the expected term of our common stock options and our expected dividend yield.

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### *Determination of fair value of common stock*

As there has been no public market for our common stock to date, the estimated fair value of our common stock has been determined by our board of directors as of the date of each option grant, with input from management, considering our most recently available third-party valuations of common stock, and our board of directors' assessment of additional objective and subjective factors that it believed were relevant and which may have changed from the date of the most recent valuation through the date of the grant. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Our common stock valuations were prepared using either an option pricing method, or OPM, or a hybrid method, both of which used market approaches to estimate our enterprise value. The OPM treats common stock and preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceeded the value of the preferred stock liquidation preferences at the time of the liquidity event, such as a strategic sale or a merger. A discount for lack of marketability of the common stock is then applied to arrive at an indication of value for the common stock. The hybrid method is a probability-weighted expected return method, or PWERM, where the equity value in one or more scenarios is calculated using an OPM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for the company, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock. These third-party valuations were performed at various dates, which resulted in valuations of our common stock of \$2.60 per share as of February 21, 2018, \$3.60 per share as of May 31, 2019, \$4.11 per share as of August 31, 2019, \$2.92 as of January 31, 2020, and \$2.97 as of March 25, 2020. In addition to considering the results of these third-party valuations, our board of directors considered various objective and subjective factors to determine the fair value of our common stock as of each grant date, including:

- the prices at which we sold shares of preferred stock and the superior rights and preferences of the preferred stock relative to our common stock at the time of each grant;
- the progress of our research and development programs, including the status and results of preclinical studies in our programs;
- our stage of development and our business strategy;
- external market conditions affecting the biotechnology industry and trends within the biotechnology industry;
- our financial position, including cash on hand, and our historical and forecasted performance and operating results;
- the lack of an active public market for our common stock and our preferred stock;
- the likelihood of achieving a liquidity event, such as an initial public offering, or IPO, or sale of our company in light of prevailing market conditions; and
- the analysis of IPOs and the market performance of similar companies in the biotechnology industry.

The assumptions underlying these valuations represented management's best estimate, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly

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different assumptions or estimates, the fair value of our common stock and our stock-based compensation expense could have been materially different.

Once a public trading market for our common stock has been established in connection with the completion of this offering, it will no longer be necessary for our board of directors to estimate the fair value of our common stock in connection with our accounting for granted stock options and other such awards we may grant, as the fair value of our common stock will be determined based on the quoted market price of our common stock.

### *Awards granted*

The following table summarizes by grant date the number of stock-based awards granted between January 1, 2019 and April 9, 2020, the per share exercise price of options, the fair value of common stock on each grant date, and the per share estimated fair value of the awards:

| <b>Grant date</b> | <b>Number of shares subject to options granted</b> | <b>Per share exercise price of options</b> | <b>Per share fair value of common stock on grant date</b> | <b>Per share estimated fair value of options</b> |
|-------------------|--|--|---|--|
| February 21, 2019 | 894,000  | \$ 2.60                                    | \$ 3.60(1)  | \$ 2.41  |
| August 12, 2019   | 523,750  | \$ 3.60                                    | \$ 3.60   | \$ 2.34  |
| November 25, 2019 | 494,500  | \$ 4.11                                    | \$ 4.11   | \$ 2.70  |
| December 6, 2019  | 209,000  | \$ 4.11                                    | \$ 4.11   | \$ 3.04  |
| March 5, 2020     | 2,940,200  | \$ 2.92                                    | \$ 2.92   | \$ 1.96  |
| March 31, 2020    | 112,750  | \$ 2.97                                    | \$ 2.97   | \$ 1.92  |

(1) At the time of the option grant on February 21, 2019, our board of directors determined that the fair value of our common stock of \$2.60 per share calculated in the valuation as of February 21, 2018 reasonably reflected the per share fair value of our common stock as of the grant date. However, the fair value of common stock as of February 21, 2019 was adjusted for financial reporting purposes based, in part, upon our third-party valuation of our common stock prepared as of May 31, 2019.

### **Off-balance sheet arrangements**

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the Securities and Exchange Commission.

### **Recently issued accounting pronouncements**

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our consolidated financial statements appearing elsewhere in this prospectus.

### **Emerging growth company status**

The Jumpstart Our Business Startups Act of 2012 permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have elected not to “opt out” of such extended transition period, which means that when a standard is issued or revised and it has different application dates for public or private companies, we can adopt the new or revised standard at the time private companies adopt the new or revised standard and may do so until such time that we either (1) irrevocably elect to “opt out” of such extended transition period or (2) no longer qualify as an emerging growth company.

## **Quantitative and qualitative disclosures about market risks**

As of December 31, 2019, we had cash and cash equivalents of \$15.1 million, which consisted of cash and money market funds. Interest income is sensitive to changes in the general level of interest rates; however, due to the nature of these investments, an immediate 10% change in market interest rates would not have a material effect on the fair market value of our investment portfolio. We are not currently exposed to significant market risk related to changes in foreign currency exchange rates. Our operations may be subject to fluctuations in foreign currency exchange rates in the future. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the years ended December 31, 2018 and 2019.

# Business

## Overview

We are an innovative genetic medicines company creating a new class of gene therapy utilizing our proprietary non-viral gene therapy platform to provide durable, redosable treatments for millions of patients living with rare and prevalent diseases. Our non-viral gene therapy platform incorporates our high-capacity DNA construct called closed-ended DNA, or ceDNA; our cell-targeted lipid nanoparticle delivery system, or ctLNP; and our established, scalable capsid-free manufacturing process. Using our approach, we are developing novel gene therapies to provide targeted delivery of genetic payloads that include large and multiple genes to a range of tissues across a broad array of diseases. We are also engineering our gene therapies to be redosable, which may enable individualized patient titration to reach the desired level of therapeutic expression and to maintain efficacy throughout a patient's life.

We are advancing an initial portfolio of eight programs for rare and prevalent diseases of the liver and retina. We are focused on diseases with significant unmet need for which our non-viral gene therapy platform may substantially improve clinical efficacy relative to current gene therapy approaches. We are prioritizing programs for rare monogenic diseases of the liver and retina, which are diseases that result from mutations in a single gene, that have well-established biomarkers and clear clinical and regulatory pathways.

We plan to expand our portfolio by pursuing additional programs in rare and prevalent diseases of the liver and retina, as well as in the skeletal muscle, the central nervous system, or CNS, and oncology by developing discrete ctLNPs, each engineered to reach a different tissue.

We believe our non-viral gene therapy platform may allow patients to produce antibody therapies from their own cells for years at a time from a single dose, and plan to advance antibody gene therapy programs across multiple therapeutic areas. The combination of the expected multi-year durability of a single dose of ceDNA, tissue-specific delivery and manufacturing capacity may provide dosing for millions of patients living with prevalent diseases.

By creating our new class of gene therapy, we believe we can reach previously untreatable or under-treated patients and address new indications, including those with large patient populations, thereby unlocking the full potential of genetic medicine. Specifically, we believe that our platform has the potential to provide durable and redosable therapies that will enable:

- expanded patient access, including the ability to treat children;
- delivery of large genetic payload, including large and multiple genes;
- native gene regulation;
- targeted delivery to a range of tissues;
- large-scale cost-effective production;
- treatment for millions of patients across the globe; and
- a sustainable payer model.

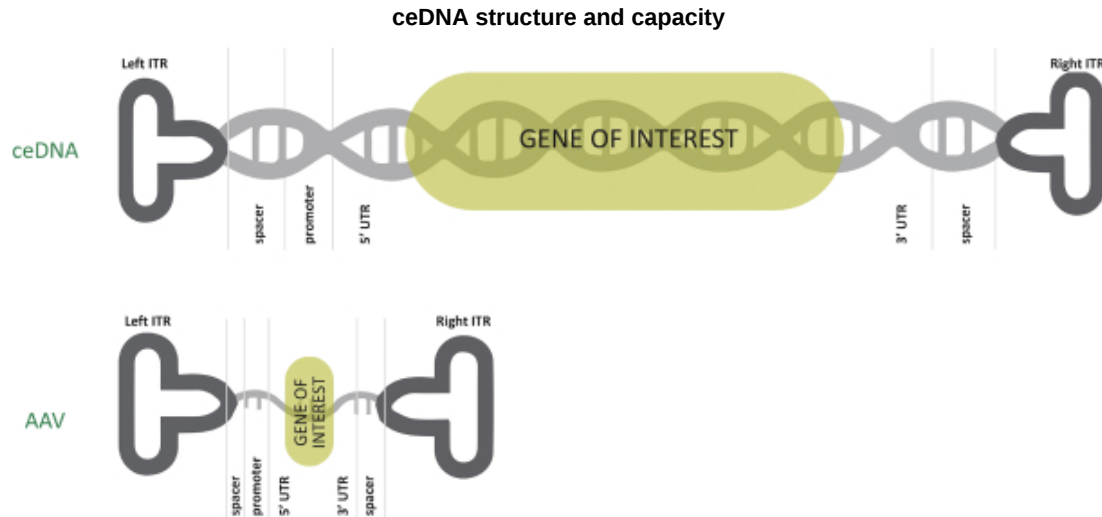
### ***Our non-viral gene therapy platform***

Our non-viral gene therapy platform is comprised of three essential components: our high-capacity ceDNA construct, which can accommodate large or multiple genes as well as native regulatory elements; our ctLNP delivery system, which enables highly specific delivery of ceDNA to a range of tissues; and our established, scalable capsid-free manufacturing process, which uses a cost-effective biologics infrastructure that has the potential to reach patients with rare diseases and to expand access to patients with prevalent diseases, requiring millions of doses, on a sustainable basis.

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### *ceDNA*

Our high-capacity ceDNA is an engineered, double-stranded, linear, covalently closed-ended DNA construct that includes the gene of interest and associated regulatory sequences. We have produced ceDNA constructs of 12 kilobases, or kb, which have almost three times the 4.7 kb capacity of adeno-associated virus, or AAV, gene therapy approaches. The structure and relative size of ceDNA as compared to AAV is shown in the figure below. We believe ceDNA can deliver a significant majority of the human coding sequences known to be relevant for the treatment of diseases that result from mutations in a single gene or in multiple genes. ceDNA may address many diseases, including prevalent diseases and diseases requiring more than one type of genetic correction that are beyond the scope of current gene therapy. This capacity can also accommodate native or engineered regulatory elements, potentially enabling a powerful new dimension of gene therapy that responds to the body's own signals.



### *ctLNP*

Our ctLNP delivery system builds upon clinically validated lipid nanoparticles, or LNPs, and is designed to allow for repeat dosing of a genetic payload without stimulating an immune response, such as antibody production. We have taken a significant step beyond current LNP technology by adding a biological targeting molecule, called a ligand, on the surface of our LNPs to direct their biodistribution to specific tissues. Different targeting ligands may enable our ctLNPs to actively target specific cell receptors in the liver, retina, skeletal muscle, CNS and tumors. In addition, our ctLNP delivery system may confer the advantages of predictable behavior across species, minimal off-target effects and a foundational platform approach that unlocks the ability to target the widest possible spectrum of diseases with our therapies.

### *Manufacturing process*

Our established, scalable manufacturing employs a capsid-free process and utilizes standard biologics infrastructure, unlike traditional viral gene therapy manufacturing. This highly efficient and reproducible process includes rigorous industrial-scale purification that consistently yields greater than 99% pure ceDNA. We have an established current Good Manufacturing Practices, or cGMP, -ready process at the 200-liter scale, which we have successfully transferred to external contract development and manufacturing organizations, or CDMOs, for production of clinical supply.



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We believe that our ability to conduct our manufacturing process at the 200-liter scale with high product quality suggests that further scaling to thousands of liters per batch is feasible using standard biological production equipment and engineering methods.

We believe the combination of the expected multi-year durability of a single dose of ceDNA, tissue-specific delivery and manufacturing capacity may provide dosing for millions of patients living with prevalent diseases. We also believe these features will allow the cost of production for our non-viral gene therapy platform to compare favorably to the cost of production of current biologic products.

### ***Our integrated research and development approach***

We have established a highly efficient eight-week research cycle to rapidly design, produce and screen ceDNA constructs in order to evaluate disease correction in relevant animal model studies. Our research cycle utilizes *in vitro* activity screens of novel designs initially as plasmid DNA, followed by *in vivo* activity screens of select designs as ceDNA. This allows us to simultaneously optimize disease-specific elements in the gene of interest region and expression elements in the rest of the construct. At any point in this cycle, additional constructs can be designed and screened to explore new functionality.

Our development strategy is differentiated and informed by our extensive experience in rare disease drug development, regulatory engagement and commercialization. We are focused on achieving early human proof of concept for our most advanced rare disease programs, and in parallel, on developing the constructs and capacity for programs to efficiently address prevalent diseases.

### ***Our portfolio***

We plan to expand our portfolio by pursuing additional programs in rare and prevalent diseases of the liver and retina, as well as in the skeletal muscle, the CNS and oncology by developing discrete ctLNPs, each engineered to reach a different tissue. By leveraging a common ctLNP for each tissue, we believe we can reduce the risk and accelerate the speed of development for subsequent indications in each tissue.



in the development of rare neuroscience programs with more than 15 years of industry experience. Matthew Stanton, Ph.D., our Chief Scientific Officer, is an expert in nucleic acid therapeutic development and delivery platforms with over 20 years of experience across pharmaceutical and biotechnology companies. Mark Angelino, Ph.D., our Chief Operating Officer, has more than 25 years of experience in drug development and deep expertise in building and scaling gene therapy manufacturing processes and infrastructure.

In the aggregate, our management team has been involved in the filing of over 40 INDs and contributed to the development of 20 approved products, including Biogen's SPINRAZA (nusinersen); Sanofi Genzyme's FABRAZYME (agalsidase beta), ALDURAZYME (laronidase) and MYOZYME (alglucosidase alfa); Sobi and Biogen's ELOCTATE and ALPROLIX; Vertex Pharmaceuticals Inc.'s KALYDECO (ivacaftor), ORKAMBI (lumacaftor/ivacaftor) and SYMDEKO (tezacaftor/ivacaftor); bluebird bio, Inc.'s ZYNTEGLO (autologous CD34+ cells encoding bA-T87Q-globin gene); and Moderna, Inc.'s mRNA-1944, an investigational therapy for the chikungunya virus.

Our organization is comprised of more than 85 talented individuals with significant experience across discovery, preclinical research, manufacturing and clinical development. We have also established scientific and clinical advisory boards comprised of leading experts in the fields of human genetics, rare disease drug discovery and development and global regulatory engagement, who share our mission of providing sustainable, life-long treatment for millions of patients living with rare and prevalent diseases.

## **The genetic medicine industry**

### ***Background***

The human genome is made up of approximately 25,000 genes, which act as a set of instructions to influence and determine every aspect of how the body functions. A genetic disease is caused by a change, or a mutation, in an individual's DNA sequence. Genetic diseases can be caused by a mutation in a single gene, known as a monogenic disorder, or by mutations in multiple genes, known as a multifactorial inheritance disorder. Current estimates suggest that there are more than 10,000 monogenic diseases. Many of these are rare, affecting hundreds or thousands of patients worldwide, such as PKU or hemophilia A. There are an even greater number of prevalent diseases whose genetics are multifactorial, affecting millions of people on a global scale, such as many types of metabolic disease and cancer.

Genetic medicines are designed to correct disease-causing dysfunction at the genetic level and utilize recombinant nucleic acids to regulate, repair, replace, add or delete a genetic sequence to achieve the desired therapeutic effect. Viral gene therapy, gene editing and messenger RNA, or mRNA, are genetic medicine modalities that specifically aim to replace the function of disease-causing genes by either inserting a gene, modifying the DNA, or inserting mRNA into a patient's cell.

Early gene therapy clinical trials in the 1990's used adenovirus to deliver genetic material. However, developers have moved away from using adenoviruses because they can trigger a strong immune reaction and their effect is short lived. Another form of viral gene therapy uses retroviral vectors, including lentiviral vectors, to incorporate DNA directly into a cell's chromosome upon infection, typically through *ex vivo* delivery. In *ex vivo* delivery, genetic modification of isolated patient or donor cells are conducted outside of the patient and then re-introduced to the patient. As an integrating virus, these vectors pose additional safety risks, and create the potential for disrupting genes or activating cancer-causing genes. In addition, *ex vivo* delivery poses a significant operational challenge and higher cost relative to *in vivo* therapies. As a result, AAV has become a preferred viral vector for gene therapy.

Significant progress has been made in the field of genetic medicine over the last decade with products approved in viral gene therapy, and several gene editing and mRNA programs in clinical development. While we

expect that there will be further advancements in these modalities, each possesses distinct clinical and commercial limitations due to known safety, efficacy, therapeutic delivery and manufacturing scale challenges.

### **Current and emerging genetic medicines and their limitations**

#### *Viral gene therapy*

Viral gene therapy, in which viral vectors are employed to deliver therapeutic genes to defective cells or tissues, has made significant progress in the past decade. The most advanced system for systemic administration is AAV gene therapy, which has demonstrated durable transduction of cells in several organ systems, with long-lasting expression in non-dividing cells. Several AAV gene therapy products have been approved, including LUXTURNA (voretigene neparvovec-rzyl) for the treatment of the rare inherited blindness disorder biallelic RPE65 mutation-associated retinal dystrophy and ZOLGENSMA (onasemnogene abeparvovec-xioi) for spinal muscular atrophy, or SMA.

However, current AAV gene therapy has demonstrated limitations, including:

- **Single dose administration only:** Following a single dose of AAV, antibodies are induced against the AAV capsid, the protein shell of the virus used for delivery. Because of these antibodies, AAV can only be dosed once, and it is typically dosed at the upper end of its therapeutic index to maximize potential efficacy across all treated patients.
  - **Variable expression:** Administration of a single fixed dose to all patients prevents repeat dosing to adjust the expression level in each patient, called titration, and leads to variable levels of expression, with many patients expressing the therapeutic protein above or below target levels.
  - **Inefficient clinical development:** Because patients cannot be redosed, those who do not achieve a therapeutic effect in early clinical trials cannot benefit from AAV therapy or contribute to further clinical development.
  - **Inability to extend expression:** The antibodies formed following a single dose of AAV prevent re-treatment to increase or extend efficacy for patients with inadequate initial response or declining levels of expression.
  - **Exclusion of pediatric patients:** The inability to redose precludes treatment of pediatric patients, whose organ growth and dividing cells would dilute expression over time.
- **Pre-existing immunity:** Up to half of patients have antibodies against AAV due to naturally acquired infections. These antibodies prevent them from receiving AAV gene therapy due to pre-existing AAV immunity to the capsid.
- **Payload capacity:** AAV constructs are limited to 4.7 kb in length, restricting both the size of genes and complexity of regulatory sequences that can be delivered. This restricts the diseases that can be addressed to those requiring single genes that can fit within this limited capacity and prevents the use of native regulatory sequences that may respond to the body's own signals.
- **Off-target, multi-tissue delivery:** Due to the inherent features of AAV, off-target delivery to unintended tissues and cell-types can lead to adverse events.
- **Quality control:** A lack of process and analytic control over the composition of AAV vectors leads to batch-to-batch variation in potency and potentially contributes to inconsistency in patients' responses.
- **Manufacturing scale:** The production systems for AAV gene therapies are limited in scale to 2,000 liters per batch or less. In general, the high doses required by AAV gene therapies and the low productivity of these

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systems combine to limit treatment to rare disease populations at a higher cost relative to other treatment modalities.

- **Payer coverage:** The relatively high cost of AAV gene therapies, combined with uncertain clinical durability and the inability to redose to extend expression, make it challenging for payers to predict clinical outcomes and, as a result, payers may be less inclined to pay for AAV gene therapies within the current reimbursement paradigm.

### *Gene editing*

Gene editing is the process of deleting, modifying or replacing defective DNA directly in the native genomic location. Zinc finger nuclease, TALEN and CRISPR-based gene editing are the most advanced approaches to gene editing and are currently in early clinical trials. CRISPR uses a combination of a nuclease to make a double-stranded break in the DNA and a guide RNA to direct the nuclease to the correct location for editing. Given insertion of full-length genes into the chromosome has remained relatively inefficient for gene editing, particularly *in vivo*, these approaches have primarily focused on *ex vivo* therapeutic applications.

Moreover, viral vectors are used widely to deliver gene editing nucleases *in vivo*, thereby conveying many of the same clinical and commercial challenges as viral gene therapy. Additional limitations for gene editing include the potential for unwanted DNA modifications related to double-stranded DNA breaks, the inability to control the level and duration of protein expression and low efficiency of precise gene correction.

### *Messenger RNA therapies*

Messenger RNA therapies are designed to increase mRNA levels by exogenous delivery of modified mRNA. However, the use of modified mRNA is limited by a lack of durable expression due to the half-life, or stability profile, of an mRNA transcript in the cell, which is approximately 10 hours. Due to the lack of durable expression for mRNA and the resulting requirement of frequent dosing, clinical development of mRNA therapies has focused primarily on novel vaccines. The safety of this frequent repeat dosing has yet to be proven clinically. In addition, the standard LNP approaches used for mRNA do not enable precise targeting of tissues.

*Summary of limitations of current approaches*

The advancements in gene therapy have demonstrated the potential of these modalities to replace full genes, while also highlighting important existing limitations, as shown below:

**The advantages of ceDNA compared to current approaches**



| Feature                   | AAV gene therapy | Gene editing* | mRNA | Generation Bio |
|---------------------------|------------------|---------------|------|----------------|
| Durable                   | ✓                | ✓             | ✗    | ✓              |
| Redosable                 | ✗                | ✗             | ✓    | ✓              |
| Titratable                | ✗                | ✗             | ✗    | ✓              |
| Large genetic payload     | ✗                | ✗             | ✓    | ✓              |
| Native gene regulation    | ✗                | ✓             | ✗    | ✓              |
| Tissue specificity        | ✗                | ✗             | ✗    | ✓              |
| Large scale manufacturing | ✗                | ✗             | ✓    | ✓              |

\*Refers to only whole gene homologous insertion

**Advantages of our non-viral gene therapy platform**

Our non-viral gene therapy platform, comprised of our ceDNA construct, our ctLNP delivery system and our established, scalable capsid-free manufacturing process, is designed to overcome the limitations of current gene therapy approaches and disrupt the field of genetic medicine. Specifically, our platform may provide the following significant advantages for patients, physicians and payers:

- **Durable expression:** Our ceDNA is highly stable and establishes extra-chromosomal episomes in the nucleus of cells to drive durable expression of the gene of interest. This durability has the potential to enable years-long expression for patients with each dose, minimizing the treatment burden for patients.
- **Redosable administration:** Our ctLNP system has been designed to avoid stimulating an antibody response in patients, thereby enabling redosing. The ability to redose provides several advantages:
  - **Individualized patient titration to reach desired expression level:** We expect our gene therapies will enable individualized patient titration, allowing each patient to be redosed until they reach the expression level required to address their specific needs. Patients and physicians may achieve individual therapeutic goals in a predictable manner.

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- *Greater opportunity to demonstrate efficacy in first-in-human trials:* Because we may be able to redose, we expect that a greater proportion of patients participating in our early clinical trials may achieve the desired level of gene expression.
- *Extending expression:* If expression of the gene of interest begins to wane for an individual patient, we expect that redosing could restore effective expression levels, prolonging the benefits of our therapies throughout the patient's life.
- *Reach pediatric patients:* Rapidly waning expression due to organ growth and dividing cells in children limits the utility of a single administration of gene therapy. We expect that the properties of our therapies may allow us to initiate treatment in childhood and allow the patient to prolong the benefits thereafter. Early intervention near the onset of disease may enable a greater therapeutic benefit throughout the patient's life.
- *Address untreated or under-treated patients:* Our therapies may be used to treat patients with pre-existing immunity to the AAV viral capsid, and therefore, not able to receive AAV gene therapy treatments, as well as patients whose efficacy outcomes on current gene therapy were insufficient in level or duration of expression.
- *Delivery of large genetic payloads:* The large payload capacity of ceDNA enables our constructs to carry large genes, multiple genes or combinations of genes with regulatory elements. This capacity arises from the lack of capsid packaging constraints that limit DNA incorporation in AAV gene therapy to less than 4.7 kb. Our established, scalable manufacturing process routinely prepares constructs of up to 12 kb without loss in yield or quality. We have not identified an upper limit of construct length.
  - *More potent constructs:* Our ceDNA constructs have the potential to improve upon existing gene therapy modalities by utilizing novel expression elements that exceed the capacity of AAV gene therapy.
  - *Larger genes:* Our ceDNA constructs may enable treatment of monogenic diseases requiring larger genes that are not possible to deliver with current gene therapy.
  - *Multiple genes:* Our ceDNA constructs have the potential to include novel multi-gene constructs to produce complex biologics such as monoclonal antibodies, or to address conditions that need more than one type of genetic correction.
  - *Native gene regulation:* The large capacity of our constructs provides us the ability to incorporate native regulatory elements that are naturally associated with the gene we are replacing. We believe this will allow for activity of the replaced gene to increase or decrease in response to the body's own signals.
- *Targeted, multi-tissue delivery:* Our ctLNP delivery system has been engineered to use biological ligands to reach receptors in a specific tissue. We believe that highly specific targeting to the key cell type for therapeutic benefit with limited delivery to off-target cells will improve the safety profile of our products.
- *Expected scale to reach millions of patients:* The combination of the expected multi-year durability of a single dose of ceDNA, tissue-specific delivery and manufacturing capacity may provide dosing for millions of patients living with prevalent diseases.
- *Sustainable payer model:* Our cost-effective manufacturing process combined with the potential to redose patients to extend expression may allow payers to better predict clinical outcomes and, as a result, to cover our therapies within the current reimbursement paradigm.

By creating this new class of gene therapy, we believe we can reach previously untreatable or under-treated patients and address new indications, including those with large patient populations, thereby unlocking the full potential of genetic medicine.

## Our strategy

Our goal is to become an industry leader in the field of genetic medicine, advancing our non-viral gene therapy platform to discover, develop, manufacture and globally commercialize a new class of gene therapy that is durable, redosable and specifically deliverable to a range of tissues for the treatment of diseases caused by single, large or multiple gene defects. Our mission is to provide sustainable, life-long treatment for millions of patients living with rare and prevalent diseases.

Key components of our strategy include:

- **Establish ceDNA as a new class of gene therapy, demonstrating its potential across rare monogenic diseases of the liver and retina.** We are prioritizing rare monogenic diseases of the liver and retina, including PKU, hemophilia A, Leber's Congenital Amaurosis, or LCA10, and Stargardt disease with significant unmet need for which our non-viral gene therapy platform may substantially improve clinical efficacy relative to current gene therapy approaches. We believe our initial focus on these rare indications, which have well-established biomarkers and clear clinical and regulatory pathways, may enable us to achieve rapid human proof of concept, regulatory approval and eventually, commercialization. We expect to submit our first IND applications beginning in .
- **Leverage our non-viral gene therapy platform to advance additional programs for diseases of the liver and retina and to expand quickly into additional tissues.** We are advancing additional programs for the liver and retina by making minimal changes to our ceDNA construct and by using the same ctLNP delivery system for each tissue. We believe this process can reduce the risk and accelerate the speed of development for subsequent indications in these tissues. We also plan to apply this approach as we develop biological ligands for our ctLNP delivery system to reach skeletal muscle, the CNS and tumors.
- **Expand manufacturing scale to access previously unattainable markets for gene therapy.** We have established a capsid-free manufacturing process for ceDNA that is scalable, cost-effective and yields a high-quality product using standard biological production equipment and engineering methods. We intend to further develop our internal manufacturing capabilities and to continue to expand our high-quality network of suppliers to increase capacity. We believe that our ability to conduct our manufacturing process at the 200-liter scale with high product quality suggests that further scaling to thousands of liters per batch is feasible using standard biological production equipment and engineering methods. We believe the combination of the expected multi-year durability of a single dose of ceDNA, tissue-specific delivery and manufacturing capacity may provide dosing for millions of patients living with prevalent diseases.
- **Leverage our eight-week research cycle to rapidly design, produce and screen ceDNA constructs in order to evaluate disease correction.** We have established a highly efficient eight-week research cycle to rapidly design, produce and screen ceDNA constructs in order to evaluate disease correction in relevant animal model studies. We intend to invest in technologies to further accelerate our research cycle and create additional efficiency and scope for this process.
- **Expand patient access to our non-viral gene therapies through a high-value network of alliances and collaborations.** We are developing a broad and expandable portfolio of gene therapies that have the distinct opportunity to address rare monogenic diseases, as well as prevalent diseases. To help us realize the full breadth of opportunities and to expedite patient access to our gene therapies, we have established and plan to continue to explore a limited set of innovative collaborations and strategic alliances with biopharmaceutical companies whose capabilities and resources are additive or complementary to our own.
- **Build a sustainable leadership position in non-viral gene therapy as a fully integrated innovative biotechnology company.** We have established a leading position in non-viral gene therapy for gene transfer

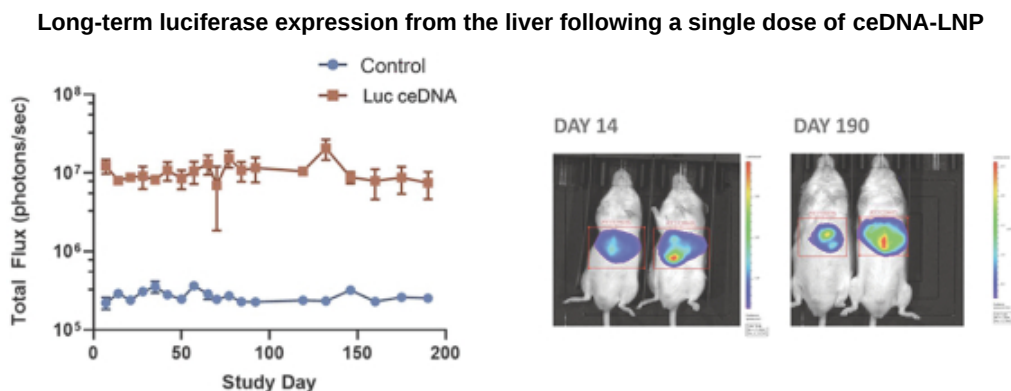


by investing in our ceDNA, ctLNP and manufacturing technologies and capabilities, and by rigorously protecting our innovations through an expanding intellectual property portfolio. We intend to evaluate and invest in new technologies that may further de-risk and accelerate the development of our new class of gene therapy, and to build out our capabilities to commercialize our gene therapies on our own.

## Our non-viral gene therapy platform

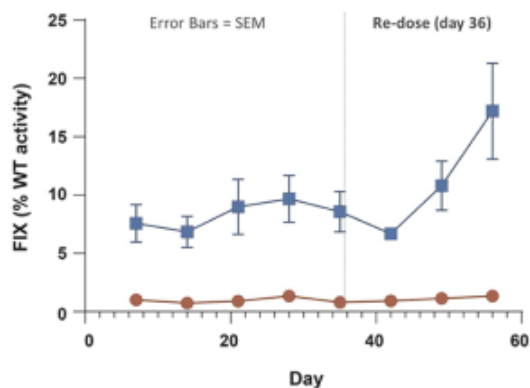
Our non-viral gene therapy platform is comprised of three essential components: our high-capacity ceDNA construct, which can accommodate large or multiple genes as well as native regulatory elements; our ctLNP delivery system, which enables highly specific delivery of ceDNA to a range of tissues; and our established, scalable capsid-free manufacturing process, which uses a cost-effective biologics infrastructure that has the potential to reach patients with rare diseases and to expand access to patients with prevalent diseases, requiring millions of doses on a sustainable basis.

The three components of our platform are designed to enable critical features that address key limitations of existing genetic medicines. The ceDNA construct is designed to enable durable expression with a single dose. As shown in the figure below, in an *in vivo* mouse study, a single intravenous dose of ceDNA formulated in an LNP provided months-long expression in the liver as exemplified by the reporter protein luciferase, measured as total bioluminescence.



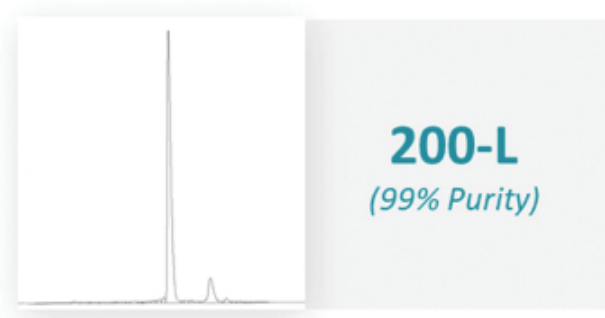
As shown in the figure below, ceDNA delivered in an LNP could be redosed in mice with a normal immune system because the first dose did not induce neutralizing antibodies. In the study, we administered ceDNA formulated in an LNP that encodes for human Factor IX, or FIX, the protein that is missing in hemophilia B. After the first administration, the mice demonstrated expression of FIX in the 5% to 10% of normal activity levels. Upon re-administration of ceDNA/LNP at day 36, a boost in expression occurred that led these mice to express FIX in the 10% to 20% of normal activity levels. These results support our belief that our platform may enable us to titrate every patient to the desired level of protein expression.

### Increased Factor IX expression upon redose of ceDNA-LNP



Our manufacturing process is analogous to biologics manufacturing in that ceDNA accumulates in Sf9 cells, which are then lysed to isolate and purify ceDNA. We have successfully completed runs of one liter, 50 liters and 200 liters. With the process and controls we have developed to purify ceDNA, we have consistently achieved greater than 99% purity, as demonstrated for 200 liters in the figure below.

### ceDNA purity at 200-liter manufacturing scale



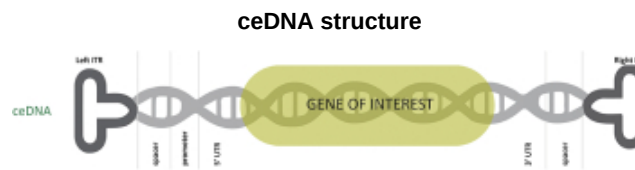
### Closed-ended DNA (ceDNA) construct

ceDNA was discovered by our scientific co-founder Robert Kotin, who during his two decades as a senior investigator at the National Institutes of Health also invented the Sf9 AAV production system and discovered the insertion site for AAV on chromosome 19. We have continued to build upon Dr. Kotin's work by assembling an expert team of leaders in molecular biology in order to deepen our understanding of ceDNA biology. In addition, we are expanding on Dr. Kotin's early work to create a broad palette of structural and sequence motifs for ceDNA constructs to explore novel mechanisms and to address multiple diseases. We are also deepening our expertise in computational biology, virology and cell biology to exploit the potential of large genomic datasets to elucidate the mechanisms underlying large subsets of prevalent and complex diseases.

ceDNA is an engineered, double-stranded, linear, covalently closed-ended DNA construct, with no free ends or viral capsid. As shown in the figure below, ceDNA includes the target gene or genes of interest, along with key components of the expression cassette, which is the full genetic sequence necessary to derive transcription including spacers, promoters and untranslated regions flanked by two inverted terminal repeat, or ITR,

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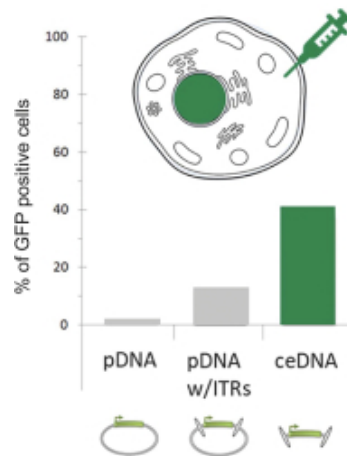
sequences. By gene of interest, we specifically mean the transcribed region of the target gene. ITRs, which are also present in AAV technology, are thought to be critical for durable expression in the nucleus of cells. To date, we have designed, produced and purified ceDNA constructs of up to 12 kb and have not identified an upper limit of construct length.



### *Nuclear entry*

In an AAV system, it is thought that the capsid mediates nuclear entry. For our capsid-free non-viral gene therapy, we believe our ceDNA accesses the nucleus due to a previously unrecognized function of the ITR structure. In an *in vitro* study, we injected plasmid lacking ITR sequences, plasmid containing ITR sequences and ceDNA into the cytoplasm of individual cells. As shown in the figure below, the plasmid lacking ITR sequences was not able to access the nucleus, and the plasmid containing ITR sequences resulted in low but measurable expression. By contrast, ceDNA displayed positive green fluorescent protein, or GFP, expression, indicating that the ceDNA had effectively translocated to the nucleus.

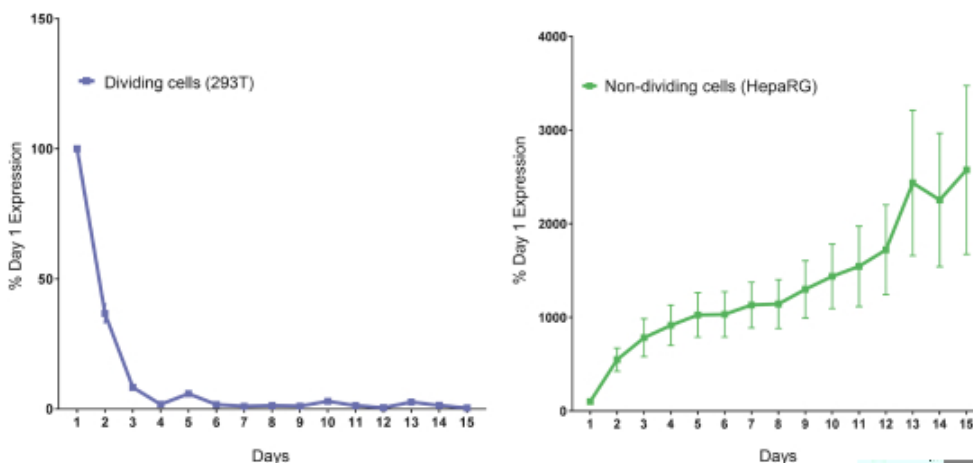
### **ceDNA's ITR structure drives translocation to the nucleus**



### *Episomal expression*

ceDNA-derived expression has been observed in *in vivo* and *in vitro* studies to be episomal, meaning that it can deliver genetic material outside of the chromosome without being directly incorporated into or altering the cell's genome. This characteristic of ceDNA is a potentially important safety feature of our redosable gene therapy platform. In cell culture studies, as depicted in the figure below, the expression in dividing cells transfected with ceDNA encoding GFP decayed rapidly as the cells divided, consistent with lack of genomic integration as each successive cell division effectively diluted the amount of ceDNA in new generations of cells. In contrast, in cells transfected with ceDNA encoding GFP which are post-mitotic and therefore not dividing, we observed sustained and durable GFP expression over the course of two weeks.

**ceDNA-derived GFP expression**



**ceDNA capacity**

The large payload capacity of ceDNA enables our constructs to carry large genes, multiple genes, or combinations of genes with regulatory elements. This capacity arises from the lack of capsid packaging constraints that limit DNA incorporation in AAV gene therapy to less than 4.7 kb. Our established, scalable manufacturing process routinely prepares constructs of up to 12 kb in length without loss in yield or quality. We have not identified an upper limit of construct length. We believe ceDNA can deliver a significant majority of the human coding sequences known to be relevant for the treatment of diseases that result from mutations in a single gene or in multiple genes. The practical applications of increased capacity include the ability:

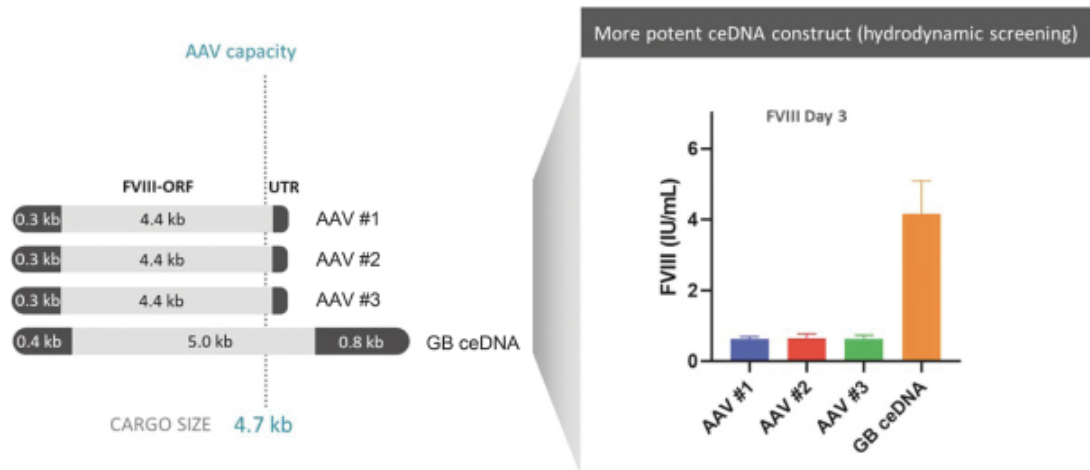
- to improve expression of existing targets of viral gene therapy and mRNA;
- to incorporate genes too large to be packaged in viral vectors;
- to include multiple genes, or to produce more than one transcript, which opens the possibility of creating several therapeutic molecules per ceDNA to address conditions that need more than one type of genetic correction; and
- to add native regulatory elements that are naturally associated with the gene we are replacing, which may allow for activity of the replaced gene to increase or decrease in response to the body's own signals.

We have observed the impact of the increased payload capacity of our constructs on improved expression efficiency in *in vitro* and *in vivo* studies. We undertook studies of Factor VIII, a protein involved in the coagulation cascade. Defects in Factor VIII are known to give rise to hemophilia A. Factor VIII is a large protein having a minimal gene of interest domain accounting for 4.4 kb (in the B-domain deleted format) which is almost the entire AAV capsid capacity. For this reason, AAV Factor VIII constructs currently in clinical development contain the minimal, B-domain deleted coding region along with a small core promoter of 0.3 kb and limited transcriptional enhancer regions.

We have designed ceDNA constructs that have larger promoter elements and more substantial transcriptional enhancer regions than can be accommodated within the payload capacity of AAV. We have observed in a mouse model that a ceDNA construct, labeled as GB ceDNA in the figure below, showed six-fold improved expression of Factor VIII relative to ceDNA constructs with the expression cassette of each of three AAV Factor VIII product

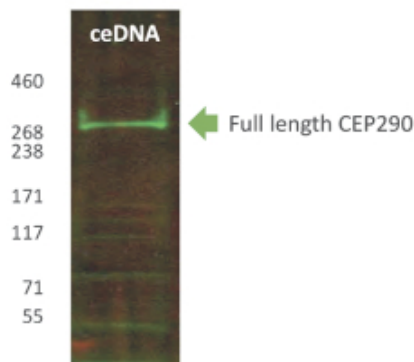
candidates currently in clinical development. The identification of constructs with improved potency may enable us to lower the dose required to achieve therapeutic efficacy.

### Increased ceDNA payload capacity enables the design of more potent constructs



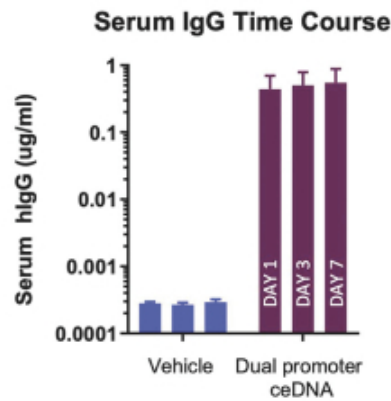
In addition to improving the activity of typical AAV target genes like Factor VIII, the capacity of ceDNA allows us to address monogenic diseases caused by large genes whose coding region is outside of the packaging capacity of AAV. One such example is LCA10, the most common genetic cause of childhood vision loss. LCA10 is caused by mutations in the CEP290 gene, which has a gene of interest region of 7.4 kb. Since this exceeds the payload capacity of AAV, there are no current transfer-based gene therapy programs in clinical trials for LCA10. By replacing the entire gene, gene therapy can potentially restore vision to patients with all forms of LCA10, irrespective of which mutation is present. We have created a ceDNA construct encoding the CEP290 gene that has demonstrated expression of full-length protein after transfection in cells, as shown in the immunoblot below.

### CEP290 Immunoblot



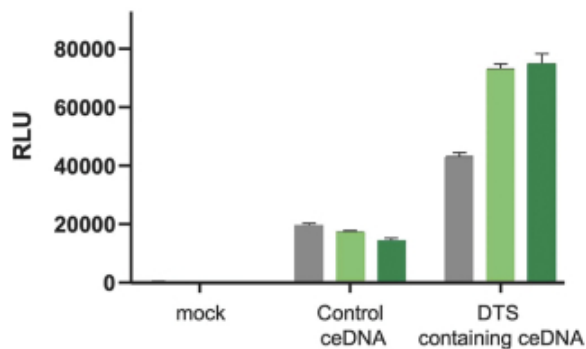
In *in vivo* studies, we have encoded and expressed two different proteins independently within the same ceDNA construct. To do this, we created a ceDNA that independently expressed heavy and light chain fragments of the antibody immunoglobulin, or IgG, with unique promoters for each, a requirement for producing distinct and separate proteins off one construct. Each promoter, a unique sequence that defines where DNA transcription

starts, governs the independent production of its own transcript. This construct was able to produce fully formed and secreted IgG *in vivo*, as shown in the figure below. This ability to include novel multi-gene constructs to produce complex biologics, such as monoclonal antibodies, also opens the potential to address conditions that need more than one type of genetic correction, such as one that could knock down a gene and another that replaces a missing or defective gene.



We have also shown in *in vitro* studies that our constructs can establish context-dependent expression by inclusion of regulatory sequences. The inclusion of regulatory sequences may enable expression of the therapeutic protein to adjust in response to changes in the patient's body, an example of which might be an anti-inflammatory antibody that is only expressed when the patient is in an inflammatory state. For these studies, we created a construct that contained multiple simian virus 40, or SV40, -derived nuclear factor kappa-light-chain-enhancer of activated B cells, or NF $\kappa$ b, and DNA-targeting sequences, or DTS, that are known to be responsive to tumor necrosis factor alpha, or TNF $\alpha$ . As shown in the figure below, expression in HepG2 cells, as measured in reflected light units, or RLU, was significantly increased in the presence of TNF $\alpha$  relative to control.

**Regulatory elements increased expression with TNF $\alpha$  stimulus**



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We believe this finding supports the potential to develop context-dependent expression cassettes and to take advantage of the sequence capacity necessary to accomplish this. Another example of such an application would be the inclusion of the native promoter in a construct expressing ABCB4 for progressive familial intrahepatic cholestasis type III where the native promoter is approximately 3.0 kb in size, which restricts its application in viral gene therapy. We have prepared this ceDNA and demonstrated that it expresses protein in cell culture.

Our research and development in ceDNA has dramatically increased our understanding of the construct's properties, capabilities and *in vivo* functioning. We have filed numerous patent applications on our innovations in ceDNA structure, applications of the technology and specific classes of therapeutic ceDNA. We intend to continue investing in both ceDNA development and its intellectual property protection.

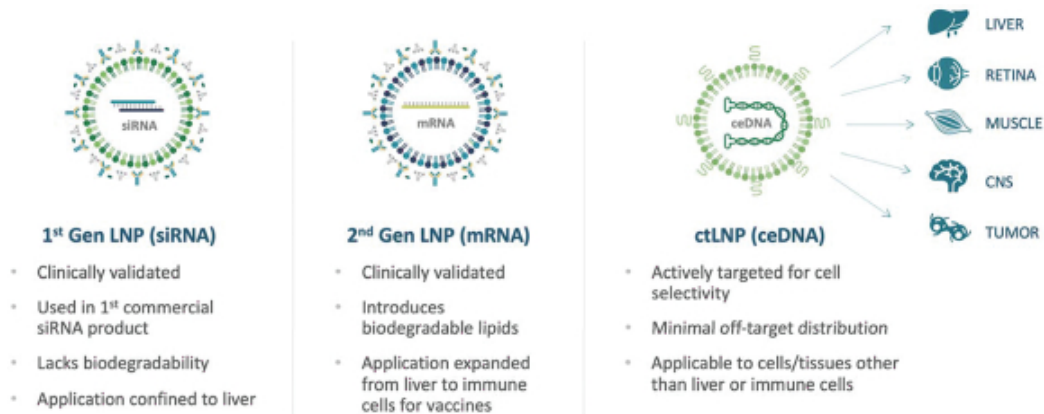
### **Cell-targeted LNP (ctLNP) delivery system**

Lipid nanoparticle technology has been developed over the past two decades for the effective delivery of nucleic acids to the liver, culminating in the first approved small interfering RNA, or siRNA, therapy for the treatment of transthyretin amyloidosis. Second-generation LNP technologies incorporate novel lipid components that improve potency, increase tolerability and are biodegradable. A second-generation LNP was recently clinically validated in a Phase 1 study of a passive immunization approach to Chikungunya virus.

Our Chief Scientific Officer Matthew Stanton has been a leader in LNP technology for the past 12 years. Prior to joining us, he led nucleic acid delivery research at Merck and Co., Inc. and Moderna, Inc., resulting in the discovery and development of four distinct classes of second-generation LNPs currently in clinical development. We have hired a team of chemists and formulation scientists along with cellular biologists and pharmacologists to continue to advance our LNP technology and expertise.

Building on the attributes of both the first- and second-generation LNP technology, we have designed our ctLNP delivery platform so that its biodistribution can be selectively controlled through the addition of a targeting molecule, or ligand. In addition, our ctLNP may confer the advantages of predictable behavior across species, minimal off-target effects, and a foundational platform approach that unlocks the ability to delivery to other tissues, including retina, skeletal muscle, the CNS and tumors. The graphic below illustrates the evolution from first generation LNPs to ctLNP.

### **Evolution from first-generation LNPs to ctLNP**



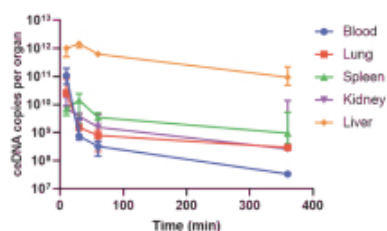
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A key feature of our ctLNP technology is the use of biological targeting ligands. This is a strategy that is shared with antibody-drug conjugates and with oligonucleotide conjugates, both of which have demonstrated clinical success. The application of targeting ligand technology has historically been unavailable to nanoparticles. This is primarily due to the non-specific and efficient uptake of nanoparticles by cells of the reticuloendothelial system, or RES, a system of cells that removes immune complexes and foreign particulates from circulation in healthy persons, and historically overwhelms the effectiveness of the targeting ligand. We have applied our chemistry and formulation capabilities to identify LNPs that avoid RES-mediated clearance, which has enabled the use of biological targeting ligands. We have achieved proof of concept for tissue-specific delivery with ctLNP *in vivo* for liver and retina and *in vitro* for skeletal muscle.

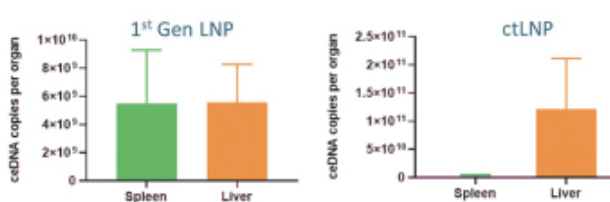
### Liver

We have employed N-acetyl galactosamine, or GalNAc, as the ligand of choice for targeting the majority of liver cells, called hepatocytes, specifically in the liver through the asialoglycoprotein receptor, or ASGPr, a well validated, selective ligand-receptor pair for delivery to hepatocytes. GalNAc targeted oligonucleotides have also demonstrated broad distribution to all hepatocytes. When we dosed GalNAc targeted ctLNPs in mice, we observed a profound selectivity for ceDNA delivery to the liver, as shown in the left figure below, as ceDNA was distributed to more than 97% of the sum of copies/tissue. By comparison, the distribution of the gold standard, first-generation LNP, MC3, in mice was more evenly distributed to liver and spleen at six hours, as shown in the right figure below. This selectivity enhancement of ctLNP relative to first-generation LNP minimized off-target effects and enabled well-tolerated dosing up to 5.0 mg/kg in mice. When we examined the liver tissue by *in situ* hybridization, we observed up to 100% of hepatocytes transduced with ceDNA at early time points, highlighting the breadth of distribution of ctLNP.

ctLNP Longitudinal PK in mice



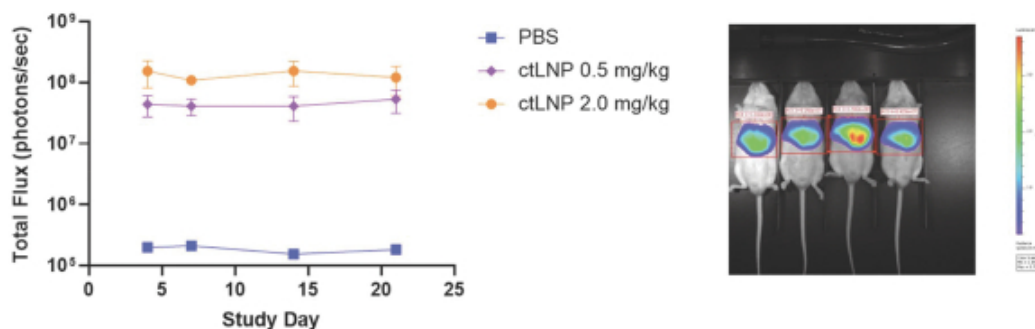
Biodistribution by tissue at 6hr for 1<sup>st</sup> Gen LNP



We have demonstrated in mice both high-level and dose-dependent expression from ceDNA incorporating our ctLNP system using a reporter ceDNA that expresses firefly luciferase after a single intravenous administration on day one. The expression was also determined to be dose proportional as we increased the ctLNP from 0.5 mg/kg to 2.0 mg/kg, as shown in the left figure below. The protein expression was specific to the liver region as determined by *in-life* imaging, as shown in the right figure below. We continue to apply chemistry and formulation optimization efforts to improve the activity of ctLNP, with our latest ctLNPs providing three to five times improvements in expression over our first iteration. We plan to continue to make further improvements in this technology.



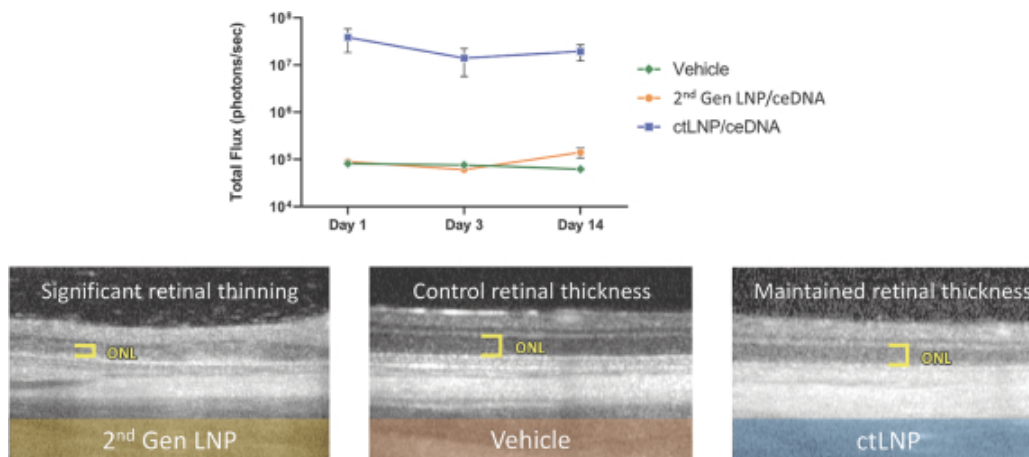
### Luciferase Expression



### Retina

In addition to assessing the specificity and activity of ctLNP established for systemic administration to the liver, we have also assessed ctLNP in local administration to the retina in the eye. Second-generation LNPs containing ceDNA, when dosed subretinally *in vivo* in mice, led to significant inflammation and retinal degeneration and failed to express protein. By contrast, as shown in the figure below, administration of ctLNP subretinally *in vivo* in mice led to high levels of expression and lack of retinal degeneration at day 21. This degeneration is evident by measuring the thickness of the outer nuclear layer, or ONL, by optical coherence tomography imaging, shown in the yellow labels in the figures below. We believe this improved retinal tolerability profile is due to ctLNP avoiding off-target delivery to local immune cells.

### Subretinal injection of ceDNA-ctLNP led to high levels of expression and lack of retinal degeneration

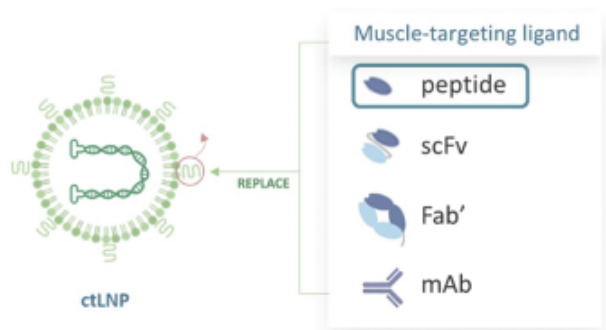


### Skeletal muscle

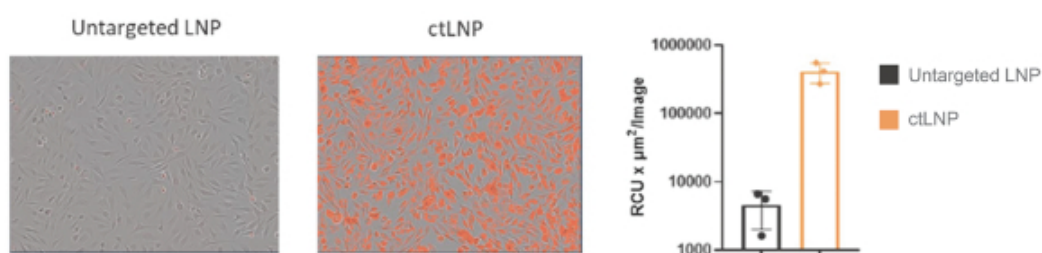
We have identified a number of potential ligands for skeletal muscle and have been able to conjugate peptide-based ligands to ctLNP that have enhanced uptake into a differentiated skeletal muscle cell line, or myocyte, *in vitro*. These peptide-based ligands target known receptors, such as the transferrin receptor that is present on the surface of skeletal muscle. ctLNPs that include these peptide-based ligands on the surface of the particle show high levels of uptake in muscle cells in culture. We have observed this in *in vitro* studies in which we

included a red dye in the ctLNP enabling quantification of uptake into these cells, as shown in the figure below. We are pursuing muscle-targeted ctLNPs for *in vivo*, systemic administration to target skeletal muscle and replace missing or defective genes, such as dystrophin in Duchenne Muscular Dystrophy.

### ctLNP targeting skeletal muscle using peptide ligand



### Enhanced uptake in c2c12 myocytes



LNPs are one of the most advanced non-viral delivery solutions for nucleic acid therapeutics. We have built on the attributes of the second generation of clinical LNPs, which include improved tolerability through utilization of biodegradable lipids. Specifically, we have engineered our ctLNP to allow for active targeting of specific cell types *in vivo*. Targeted delivery of DNA *in vivo* without incurring innate immune reactions has long been a challenge in the field. The profile of our ctLNP has led to further improvements in tolerability, increased delivery efficiency and application to tissues beyond liver and immune cells. Our ctLNP delivery system has shown proof of concept *in vivo* in liver and retina with *in vitro* proof of concept for skeletal muscle. We plan to continue to seek to optimize ctLNP for use in liver, retina and skeletal muscle and to expand its application across a range of tissues, including the CNS and tumors. We have filed numerous patent applications to solidify our leadership in this area, including ones covering fundamental LNP technology as well as methods to avoid or reduce immune response.

### **Established, scalable manufacturing**

Since our founding, we have invested in internalizing core development capabilities to build our manufacturing processes and analytical testing as a point of strength. Our co-founder and Chief Operating Officer, Mark Angelino, has led successful development and manufacturing organizations across several novel modalities, including complex biologics, oligonucleotide and viral gene therapy for the past 15 years. He has recruited a seasoned team of development and manufacturing experts who have enabled more than 20 INDs and negotiated multiple product launches, including for both a cell and a gene therapy product.

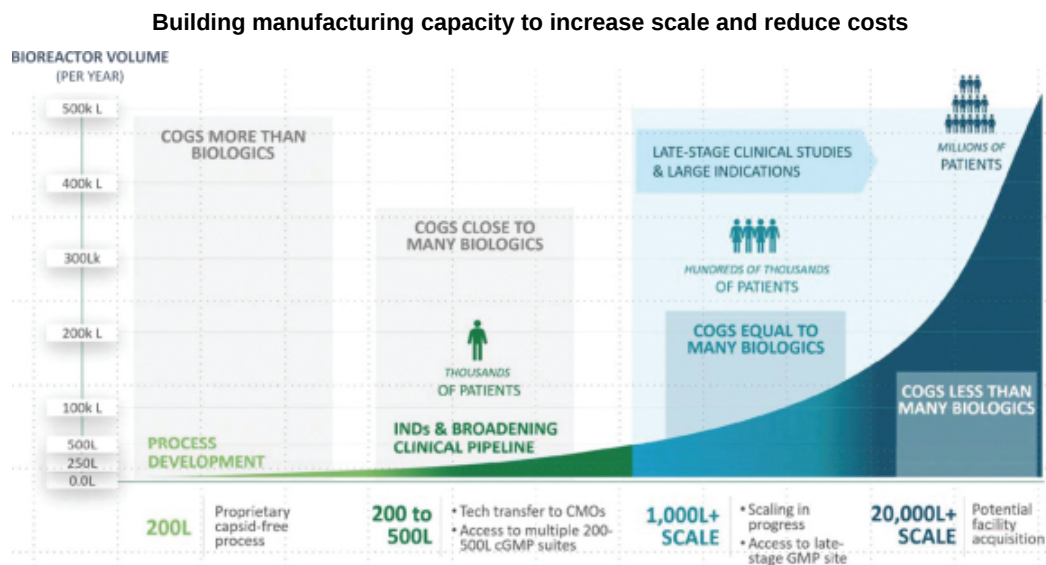
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In addition to this accumulated experience, we have built state-of-the-art development laboratories to create novel upstream and downstream processes as well as analytical methodologies that have shifted the product profile from early research efforts of approximately 10% pure ceDNA to one that is consistently greater than 99% pure ceDNA. This level and consistency of purity is differentiated from current viral gene therapy and similar to the purity attained in the production of biologics and small molecules. We continue to build new generations of know-how and competitive advantage in this area.

Our efforts have established a capsid-free manufacturing process for ceDNA that is scalable, cost-effective and consistently yields a high-quality product. This process for ceDNA is based on a well-known Sf9-cell line suspension process. Internal development efforts have focused on industrialization of the process to enable unit operations that are scalable using standard biological production equipment.

We produce material at 50-liter scale internally to support research studies, and our CDMOs have produced material at 200-liter scale, using our cGMP-ready process, to supply IND-enabling preclinical studies and early clinical trials. Additional development efforts have been initiated to further scale the process to thousands of liters.

The scalability and productivity of the manufacturing process is a significant driver of the cost-effectiveness of any therapeutic. We believe being able to produce ceDNA at a scale of thousands of liters per batch should enable a significant reduction in our overall cost of goods, and our goal over time is to be competitive with traditional biologics in scale and cost. We believe that our ability to conduct our manufacturing process at the 200-liter scale with high product quality suggests that further scaling to thousands of liters is feasible using standard biological production equipment and engineering methods.



Our ongoing work in manufacturing technology has led to numerous innovations in production processes and analytics, some of which may have broader applications in related fields. We continue to seek to protect the full reach of these discoveries through both patent application filings and as trade secrets.

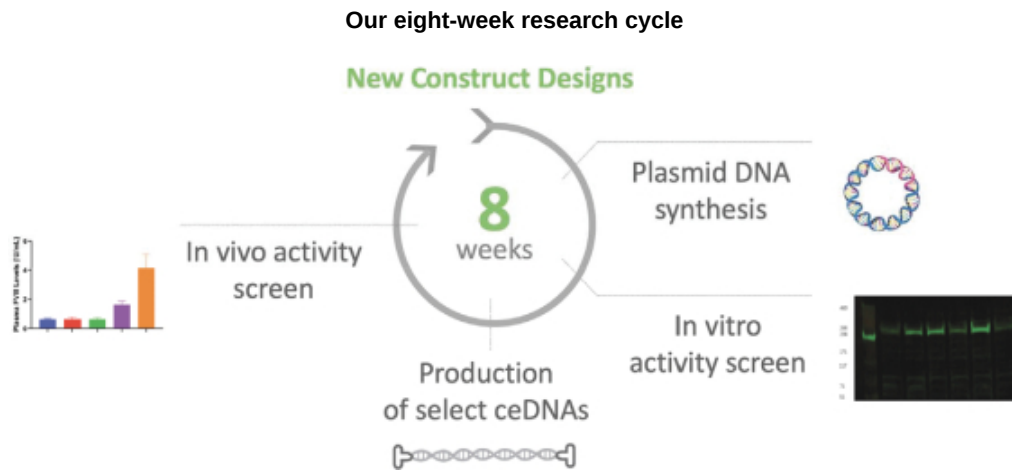
### Summary

We believe our non-viral gene therapy platform represents a powerful product engine fueled by ceDNA, which in preclinical studies has provided durable episomal expression with large capacity, and our highly selective and modular ctLNP delivery system. We have designed the combination of these platform components with a goal of enabling a broad and expandable product portfolio that can be deployed against a range of tissues, including liver, retina, skeletal muscle, the CNS and tumors. These characteristics are supported by our established, scalable capsid-free manufacturing process, which supports the potential to extend the reach of gene therapy beyond rare diseases to prevalent diseases.

### Our integrated research and development approach

We have established a highly efficient eight-week research cycle to design, produce and screen ceDNA constructs in order to evaluate disease correction in relevant animal model studies. Our research cycle, illustrated in the figure below, utilizes *in vitro* activity screens of novel plasmid DNA designs, followed by *in vivo* activity screens of select ceDNA constructs. This allows us to simultaneously optimize disease-specific elements in the gene of interest region and expression elements in the rest of the construct. At any point in this cycle, additional constructs can be designed and screened to explore new functionality.

We have iteratively employed this cycle to rapidly identify ceDNA constructs to assess for disease correction in animal models. We have observed that learnings from our more mature programs accelerate our ability to identify effective ceDNA constructs for subsequent indications.



Our development strategy is differentiated and informed by our extensive experience in rare disease drug development, regulatory engagement and commercialization. We are focused on achieving early human proof of concept for our most advanced rare disease programs, and in parallel, on developing the constructs and capacity for programs to efficiently address prevalent diseases.

Specifically, our development strategy is designed to:

- identify diseases with well-understood targets and biology that enable us to utilize our eight-week design-to-construct screening research cycle in order to rapidly proceed to evaluation of disease correction in relevant animal models;
- focus initially on rare monogenic diseases of the liver and retina with significant unmet need where we believe our platform features can make substantial improvements over current standards of care;

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- prioritize diseases with established biomarkers and well-established clinical and regulatory pathways that enable rapid human proof of concept; and
- incorporate redosing in our first-in-human clinical trials, which may allow us to reach the desired therapeutic expression level in every patient, providing early and robust human proof concept.

## **Our portfolio**

We are advancing a broad and expansive portfolio including eight programs for rare and prevalent diseases of the liver and retina. We are focused on diseases with significant unmet need for which our non-viral gene therapy platform may substantially improve clinical efficacy relative to current gene therapy approaches. We are prioritizing rare monogenic diseases of the liver and retina that have well-established biomarkers and clear clinical and regulatory pathways. We plan to expand our portfolio by pursuing additional programs in rare and prevalent diseases of the liver and retina, as well as in the skeletal muscle, the CNS and oncology by developing discrete ctLNPs, each engineered to reach a different tissue.

In addition, we believe that our non-viral gene therapy platform may allow patients to produce antibody therapies from their own cells for years at a time from a single dose and plan to advance programs to deliver antibody genes, directing the body's own cells to express and secrete therapeutic antibodies. We believe the combination of the expected multi-year durability of a single dose of ceDNA, tissue-specific delivery and manufacturing capacity may provide dosing for millions of patients living with prevalent diseases.

### **Liver diseases**

For the majority of our liver programs, we have designed and manufactured disease-modifying ceDNAs that have shown expression *in vitro* and/or disease correction *in vivo*. We have employed GalNAc, a ligand which binds to the ASGPr on hepatocytes, given that the biology of this selective ligand-receptor pair for delivery to hepatocytes has been well validated in human clinical trials. For each liver program, we plan to formulate ceDNA within a ctLNP-GalNAc expressing the relevant gene of interest for intravenous delivery. GalNAc targeting has been demonstrated to efficiently deliver ceDNA to up to 100% of hepatocytes and we believe that broad biodistribution of ctLNP-GalNAc to hepatocytes will be a key strength of our pipeline programs since current gene therapy approaches deliver payload to 30% or fewer of hepatocytes with a single dose. Additionally, our potential to redose patients until they are in the therapeutic range of expression may enable early and robust human proof of concept in Phase 1/2 clinical trials and is a key differentiator from current gene therapy approaches.

### **Phenylketonuria**

#### *Overview*

Phenylketonuria, or PKU, is a rare autosomal recessive genetic disorder caused by deficiency of the hepatic enzyme phenylalanine hydroxylase, or PAH, that metabolizes the essential amino acid phenylalanine, or Phe, to tyrosine, or Tyr, an essential amino acid for CNS development and function. PAH deficiency leads to elevated levels of Phe in the blood and toxic Phe accumulation in the brain resulting in neurocognitive manifestations including failure to attain early developmental milestones and progressive impairment of cerebral function. Patients with uncontrolled severe PKU will develop profound neuropsychiatric disorders and irreversible intellectual disability.

There are approximately 15,000 patients with PKU in the United States and 41,000 patients in the European Union. PKU is usually diagnosed at birth through newborn screening, which provides the opportunity to introduce therapies that reduce Phe levels. Attaining consistent levels of Phe in childhood is correlated with

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higher IQ levels and executive functioning in adults. Even without optimal correction of Phe levels in childhood, doing so in adolescent and adult PKU patients has been shown to result in higher levels of executive function and attention and lower levels of depression and anxiety.

### *Current approaches and limitations*

The standard of care for PKU is strict, life-long dietary modification to control blood Phe levels to the recommended target range of 120 to 360 micromoles per liter. This requires PKU patients and caregivers to carefully monitor diet and severely restrict protein intake and other Phe-rich foods. Medical foods including formula and foods modified to be low in protein are also required.

Initiation of a Phe-restricted diet is recommended as early as possible in infancy to avoid irreversible neurocognitive decline due to elevated Phe levels. While this allows some patients to approach target Phe levels, it is challenging to maintain as a life-long treatment. Even with full compliance of dietary therapy during childhood, many patients have episodic or chronic high Phe levels and suffer irreversible neurocognitive impairment as a result.

PALYNZIQ (pegvaliase) and KUVAN (sapropterin dihydrochloride) , a biologic and a small molecule, respectively, are approved in the United States for patients with PKU and both have been shown to have modest benefits in a subset of patients. PALYNZIQ is approved only for adult PKU patients. KUVAN does not normalize Phe in most patients and has shown to be ineffective in patients with severe PKU. Despite treatment advances, including availability of a larger selection of medical and low protein foods and the approval of the two treatments, many patients still do not achieve the recommended daily Phe levels and are at risk of developing neurocognitive manifestations, including intellectual disability and neuropsychiatric disorders.

Several AAV gene therapy approaches are currently being tested in clinical trials in patients with PKU. However, following a single dose of AAV, antibodies are induced against the AAV capsid and thus, AAV gene therapy can only be administered once. The antibodies formed following a single dose of AAV prevent re-treatment to increase or extend efficacy for patients with inadequate initial response or declining levels of expression. Further, it precludes treatment of pediatric patients, whose organ growth and dividing cells would dilute expression over time, and therefore would require redosing. As a result, these therapies do not have the opportunity to correct PKU at or near the onset of disease, allowing for normal neurocognitive development.

Given the limitations of currently approved therapies and the AAV gene therapy approaches under development, there is a substantial unmet need for more effective therapies for PKU.

### *Our approach*

Our non-viral gene therapy approach aims to:

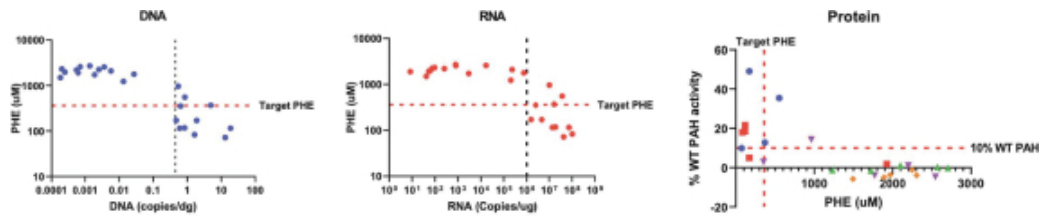
- achieve stable correction of Phe levels in PKU patients of all ages upon initial dose or through individualized patient dose titration, allowing them to normalize their diet, eliminate the burden of ongoing treatment and stabilizing and/or preserving neurocognitive function;
- achieve the desired level of gene expression for each patient through our ability to redose;
- treat patients effectively for life by episodically monitoring serum Phe levels and redosing therapy as expression wanes over time;
- reverse attention, memory or executive function deficits in adults and adolescents; and
- preserve normal neurocognitive development in infants and children.

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To establish benchmarks for full correction in PKU, we conducted a study utilizing the established mouse model of PKU, known as PAH<sup>enu2</sup>, a mouse model in which the PAH gene is mutated, leading to dramatically elevated Phe, to assess how many copies of ceDNA, ceDNA-derived PAH RNA and ceDNA-derived PAH protein are required for full correction in this model. In this study, we administered a ceDNA-PAH construct by hydrodynamic injection into PAH<sup>enu2</sup> mice. As shown in the figure below, we found that animals that had full correction of Phe, which is greater than 360 micromolar, or  $\mu\text{M}$ , had the following:

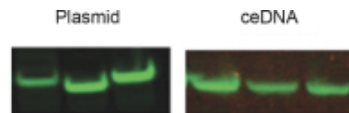
- ceDNA-PAH DNA copies in the liver of at least  $\sim 0.5$  copies per diploid genome, or dg;
- ceDNA-PAH derived RNA transcripts at least  $1 \times 10^6$  copies/ $\mu\text{g}$ ; and
- ceDNA-PAH derived protein of at least 10% of normal levels.

### Threshold levels for ceDNA-PAH correction in PKU mice



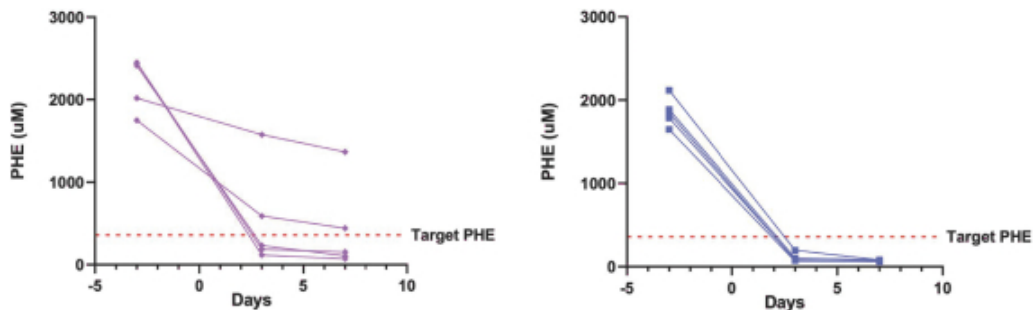
As shown in the figure below, both plasmid-PAH constructs, the left image, and several ceDNA-PAH constructs, the right image, express PAH protein at varying levels by Western Blot in cell culture.

### Expression of plasmid-PAH and ceDNA-PAH constructs within cells *in vitro*



We tested two of our most potent ceDNA-PAH constructs to determine whether, when administered hydrodynamically, they could normalize Phe levels in PAH<sup>enu2</sup> mice. Serum samples from PAH<sup>enu2</sup> mice were taken at baseline and exhibited very high Phe levels, of greater than 1500  $\mu\text{M}$ . Serum samples were taken three and seven days after IV administration of ceDNA-PAH and assessed for Phe levels. As shown in the images, two ceDNA-hPAH constructs resulted in improvement in Phe levels in all mice, dropping below the threshold for Phe levels in normal mice, shown as the dashed lines. One of these constructs, ceDNA-hPAH #2 in the right figure, resulted in complete normalization of Phe level in all mice tested.

### Correction of Phe levels after hydrodynamic injection of ceDNA-hPAH *in vivo*



*Next steps*

We expect to achieve murine preclinical proof of concept, defined as Phe normalization in PAH<sup>enu2</sup> in mice after administration of ceDNA-PAH formulated in ctLNP. We plan to begin IND-enabling studies in \_\_\_\_\_ and to submit an IND application for this program in \_\_\_\_\_.

**Hemophilia A**

*Overview*

Hemophilia A is a rare X-linked hereditary bleeding disorder characterized by impaired blood coagulation as a result of deficiencies in the production or function of coagulation Factor VIII. There are approximately 16,000 hemophilia A patients in the United States and 320,000 patients worldwide. Because of the deficiency of coagulation Factor VIII, hemophilia A patients bleed in joints, muscles, soft tissues and within mucous membranes, which can be either spontaneous or due to internal or external trauma, depending on the severity of the disease. The clinical presentation of hemophilia A can be mild, moderate or severe, depending on the residual level of circulating Factor VIII. The diagnosis of hemophilia A is often made at a young age, earlier than 36 months, based on persistent bleeding or internal hemorrhage.

*Current approaches and limitations*

Most patients with hemophilia A in the United States and the European Union are currently treated with clotting factors according to practice guidelines, which are relatively consistent around the world. Children are generally treated prophylactically, while adults may be treated either prophylactically or on demand, depending on the residual level of Factor VIII activity in the blood. Compliance with clotting factors is 50% to 80%, and up to 30% of patients with severe disease develop inhibitors to Factor VIII replacement. Approximately 75% of hemophilia A patients worldwide still receive inadequate treatment or have no access to treatment.

HEMLIBRA (emicizumab) is a bispecific Factor IXa- and Factor X-directed antibody indicated for routine adult and pediatric patients ages newborn and older with hemophilia A. However, this product has safety concerns as several cases of thrombotic microangiopathy/thrombosis have occurred, in some cases in the context of co-administration of activated prothrombin complex concentrate.

There are several AAV gene therapies currently in late-stage clinical trials, including Valrox (valoctocogene roxaparvovec), for which a biologics license application, or BLA, is under review by the FDA. Early data from these trials have shown variation in the amount of Factor VIII expressed from patient-to-patient with a substantial proportion of patients either becoming suprathreshold or remaining subtherapeutic after Valrox administration. Many hemophilia A patients have pre-existing immunity to AAV and, therefore, are not candidates for this therapy. Mean Factor VIII levels decline over two to three years after treatment with Valrox, which means that it is possible that patients will become subtherapeutic over three to five years and cannot be redosed with AAV gene therapy. In addition, AAV gene therapy will not be available to children with hemophilia A since their livers are still growing and only one dose is possible.

There are no current or investigational therapies that have been shown to durably induce therapeutic Factor VIII levels in all patients of all ages, nor therapies that can be redosed when Factor VIII expression wanes through the life of the patient. Given the variable response to gene therapy in patients with hemophilia A, titration at onset of therapy is critical to ensure that all patients get within a therapeutic range. Since there are potential consequences to expressing too much Factor VIII, such as excess blood clotting, the ability to titrate the therapy will enable an optimal dosing regimen.



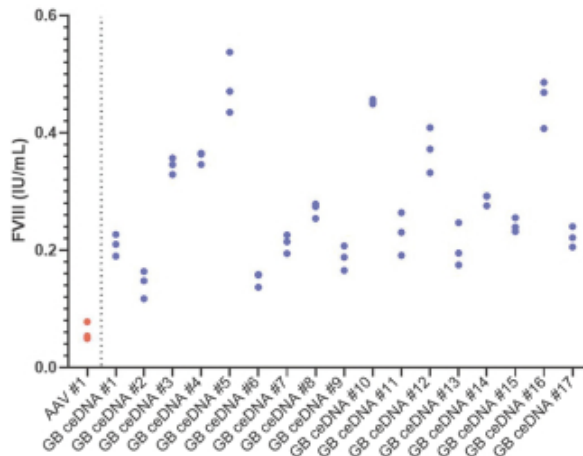
*Our approach*

Our gene therapy approach aims to do the following in hemophilia A:

- achieve therapeutic Factor VIII levels in patients of all ages, resulting in normalization of bleeding risk;
- treat infants and children to prevent bleeds early in disease and prevent irreversible tissue and organ dysfunction;
- achieve the desired level of gene expression in a greater proportion of patients participating in our early clinical trials through our ability to redose;
- treat patients for life by episodically following serum Factor VIII levels and redosing as expression wanes;
- ensure that all patients achieve curative levels of Factor VIII of greater than 25% of normal activity levels with higher levels possible for some patients based on lifestyle and circumstances;
- avoid supratherapeutic Factor VIII levels through individual patient titration, avoiding a thrombotic, or clotting, risk to the patient;
- deliver ceDNA-FVIII to a large percentage of hepatocytes resulting in a relatively low burden of expression for each hepatocyte, which may lead to more durable expression compared to other gene therapy modalities; and
- increase the safety index by achieving therapeutic Factor VIII levels at lower doses because of more potent ceDNA-FVIII constructs and greater biodistribution.

We have created multiple distinct ceDNA-FVIII constructs with different expression cassette elements, including promoter, intron, untranslated regions, or UTRs, and codon optimization sequences. *In vitro* screening of ceDNA-FVIII constructs revealed that several constructs express Factor VIII, as defined by functional activity measures, as shown in the figure below. All of our ceDNA constructs, labeled as GB ceDNA in the figure below, expressed greater activity of FVIII compared to a ceDNA construct with the expression cassette of an AAV-FVIII product candidate that is currently in clinical trials, AAV #1, with some expressing up to six-fold more Factor VIII activity.

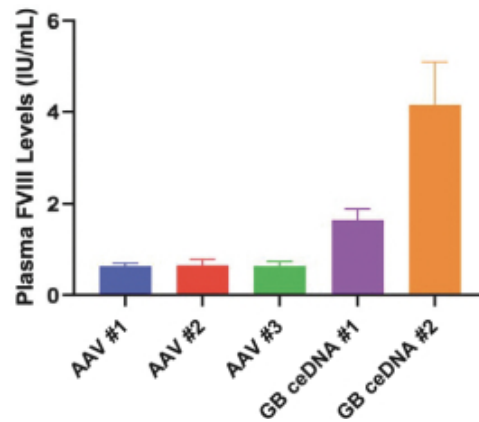
**Several ceDNA-FVIII demonstrated greater activity compared to AAV-FVIII**



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The enhanced potency of our ceDNA-FVIII constructs has also been confirmed *in vivo*. In an *in vivo* study, we measured serum levels of human Factor VIII after hydrodynamic injection of various ceDNA constructs into the tail vein of mice and assessed protein levels by enzyme-linked immunosorbent assay for human Factor VIII. As shown in the figure below, two ceDNA constructs listed as GB ceDNA #1 and GB ceDNA #2 demonstrated 1.0 and 6.0 IU/ml Factor VIII activity, or 100% and 600% of normal levels, respectively. In contrast, at the same dose, ceDNA constructs with the expression cassette of each of three AAV-FVIII product candidates currently in clinical development, demonstrated approximately 0.5 IU/ml Factor VIII activity, or 50% of normal levels in animals.

***In vivo* activity of ceDNA-FVIII constructs**



The direct relevance of having more potent ceDNA constructs is that the input dose required to have a therapeutic effect may be lower, thereby increasing the safety profile and lowering the metabolic requirements on individual hepatocytes to produce and secrete Factor VIII.

### *Next steps*

We expect to achieve murine preclinical proof of concept in \_\_\_\_\_, defined as demonstrating normal Factor VIII levels after administration of ceDNA-FVIII formulated in ctLNP. We plan to begin IND-enabling studies in \_\_\_\_\_, and to submit an IND application for this program in \_\_\_\_\_.

## **Wilson disease**

### *Overview*

Wilson disease is a rare autosomal recessive disease due to a loss-of-function mutation in the ATP7B copper transporter. There are approximately 11,000 patients with Wilson disease in the United States and 17,000 patients in the European Union. Mutations in the ATP7B copper transporter prevent incorporation of copper into ceruloplasmin and diminishes biliary secretion of copper excess, resulting in toxic accumulation of copper in the liver and brain. Clinically, liver damage begins by six years of life and then progresses with inflammation, fibrosis and joint pain. Many patients also develop CNS manifestations of copper overload, including psychosis, tremors, dysarthria, or slurred speech and muscle stiffness.

### *Current approaches and limitations*

There are no approved gene therapies for Wilson disease. Current treatment approaches include chelators promoting excretion of copper from the body and zinc salts that reduce copper absorption. These therapies

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have demonstrated limited effectiveness and safety issues, resulting in poor compliance in patients. The only curative option for acute liver failure related to Wilson disease today is a liver transplant, which cannot be offered to most patients due to lack of availability of matched donors. The average age of Wilson disease patients undergoing liver transplantation is 15 years old for those who have an available donor, indicating the need for intervention in childhood to prevent progression to this point.

There are no current gene therapy clinical trials for Wilson disease. The ATP7B gene is 4.4 kb, which means the size of the gene plus the remainder of the expression cassette cannot fit within an AAV. One preclinical AAV gene therapy program, known as VTX-801, is in development for Wilson disease utilizing a truncated ATP7B gene that may not have the full functionality of ATP7B.

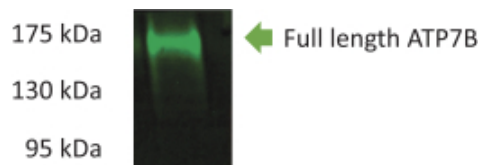
### *Our approach*

Our gene therapy approach aims to do the following:

- enable full correction of copper metabolism by broadly transducing hepatocytes and utilizing full-length ATP7B gene and an optimized expression cassette;
- achieve full hepatic correction early in the disease, re-directing copper to be incorporated into ceruloplasmin and normalizing bile excretion, thereby avoiding hepatic and CNS tissue accumulation and irreversible liver fibrosis and neurocognitive decline;
- initiate treatment in children, early in the disease, to prevent the accumulation of liver damage and irreversible neurocognitive decline;
- maintain correction of hepatocytes by redosing to compensate for the effect of injury-driven cell division in the liver;
- achieve the appropriate therapeutic level of ATP7B needed for correction using serum biomarkers via individualized patient titration at the onset of therapy; and
- treat patients for life by maintaining copper and ceruloplasmin levels in the normal range with maintenance therapy.

We have generated Wilson disease plasmid constructs and demonstrated the expression and appropriate sub-cellular location of the expressed protein *in vitro*. We have induced cells to produce the full length ATP7B protein at 157 kilodaltons using Western Blot, as shown in the left figure below. We have also demonstrated, by immunohistochemistry, appropriate localization of ATP7B in the golgi apparatus around the nucleus within cells, consistent with where native ATP7B localizes, as shown in the right figure below.

### **ATP7B plasmid constructs result in ATP7B protein production and appropriate subcellular localization *in vitro***



### *Next steps*

We plan to declare a development candidate and enter IND-enabling studies in \_\_\_\_\_ for this program.

## **Gaucher disease, Type 1**

### *Overview*

Gaucher disease is a rare inherited autosomal recessive disorder caused by the insufficient expression of lysosomal enzyme glucocerebrosidase, or GCase. Gaucher disease is the most common inherited lysosomal storage disease. There are approximately 6,000 patients with Gaucher disease in the United States and 9,000 patients in the European Union. Ninety percent of Gaucher patients are classified as Type 1, the most common form, which has no CNS involvement in the disease. Patients with Type 1 disease are typically diagnosed between 10 to 15 years of age.

Patients with Gaucher disease do not produce adequate levels of GCase, which causes glucosylceramide, a toxic lipid, to accumulate in macrophage lineage cells in visceral organs. This results in splenomegaly, hepatomegaly and cytopenia. Disease progression commonly involves loss of bone mass, either osteopenia or osteoporosis and can lead to painful bone crises and avascular necrosis, or death of bone tissue. We are specifically developing a gene therapy approach to address Type 1 Gaucher disease.

### *Current approaches and limitations*

The current standard of care for Gaucher disease includes enzyme replacement therapy, or ERT, for patients of all ages or substrate reduction therapy, or SRT, for adults with Type 1. ERT is recommended as a potentially suitable treatment for all symptomatic Type 1 patients, though the required IV infusions every two weeks can lead to difficult treatment burden.

SRT treats Gaucher disease by inhibiting cellular production of glucosylceramide, rather than increasing the degradation of glucosylceramides through ERT. ZAVESCA (miglustat) and CERDELGA (eliglustat) effectively treat hepatosplenomegaly in a similar time course to ERT, though improvement in hematological aspects of the disease takes longer to materialize. SRT does not correct the fundamental lack of GCase and biochemical and tissue abnormalities persist.

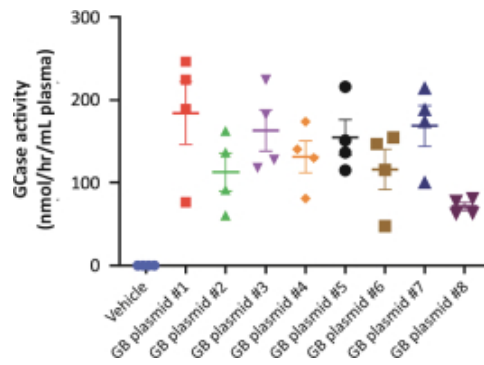
### *Our approach*

Our gene therapy approach aims to:

- provide continuous therapeutic levels of GCase in serum and tissues that can break down glucosylceramide, which may enhance tissue correction over episodic ERT;
- administer ceDNA-GCase early in disease before the onset of inflammation, fibrosis and irreversible tissue injury;
- achieve the appropriate GCase levels for potential disease modification using biomarkers such as glucosylsphingosine, or Lyso-Gb1, via individualized patient titration;
- treat patients for life by maintaining Lyso-Gb1 levels in the normal range through redosing; and
- reduce immune reactions to GCase, which occur in 2% to 15% of Gaucher patients.

We have created multiple distinct GCase plasmid constructs and have tested them for activity *in vivo*. These constructs differ in various expression cassette elements, including promoter, intron, UTR and codon optimization expression elements. We administered these plasmid GCase constructs hydrodynamically into mice and measured GCase enzymatic activity in the serum. As shown in the figure below, many of our GCase plasmid constructs have exhibited functional GCase activity in serum, indicating that hepatocytes are not only able to produce the GCase, but can also secrete it for uptake by other tissues that are affected in Gaucher.

### Detection of GCCase in plasma after hydrodynamic injection of plasmid constructs *in vivo*



#### Next steps

We plan to declare a development candidate and enter IND-enabling studies in \_\_\_\_\_ for this program.

### Antibody gene therapy

#### Overview

We plan to advance product candidates to deliver antibody genes to direct the liver to express and secrete antibodies. Monoclonal antibodies, or mAbs, have demonstrated therapeutic benefit in many areas, including infectious diseases, rheumatology, hematology and oncology.

We believe that utilizing the patient's own cells to produce and secrete therapeutic mAbs, fragments or derivatives is a potentially transformative approach that can result in greater efficacy, produced at a larger scale and with lower cost of goods compared to the passive administration of some mAbs. This approach is called antibody gene therapy, or AGT.

#### Current approaches and limitations

The cost of goods, the burden of frequent administration and the manufacturing scale limitations of mAb therapy preclude their widespread utilization in many diseases.

#### Our approach

Our gene therapy approach aims to:

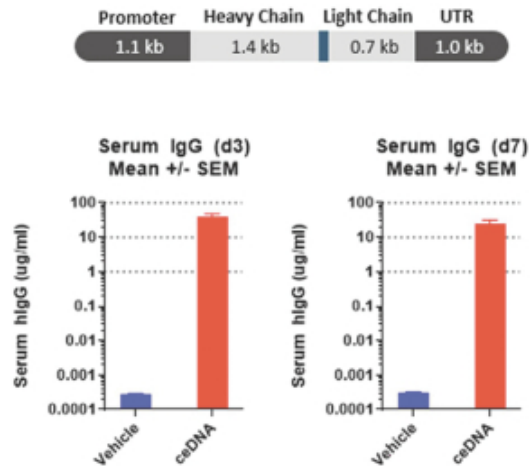
- enable a patient's own body to sustainably produce and secrete a therapeutic mAb from the liver by introducing ceDNA-AGT;
- enable continuous production of protein, resulting in a stable, effective levels of serum mAb, thereby avoiding toxicity due to off-target effects of pulsatile delivery and/or loss of efficacy often associated with rapid reduction in concentrations when delivered passively;
- rapidly induce a therapeutic effect due to liver production of the therapeutic mAb within days of administration;

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- potentially eliminate compliance risk due to sustained expression after a single dose;
- encode multiple mAbs on a single ceDNA construct for broad therapeutic application;
- achieve the appropriate mAb levels in the blood via individualized patient titration; and
- induce rapid onset, durable immunity to prevent infection for large populations.

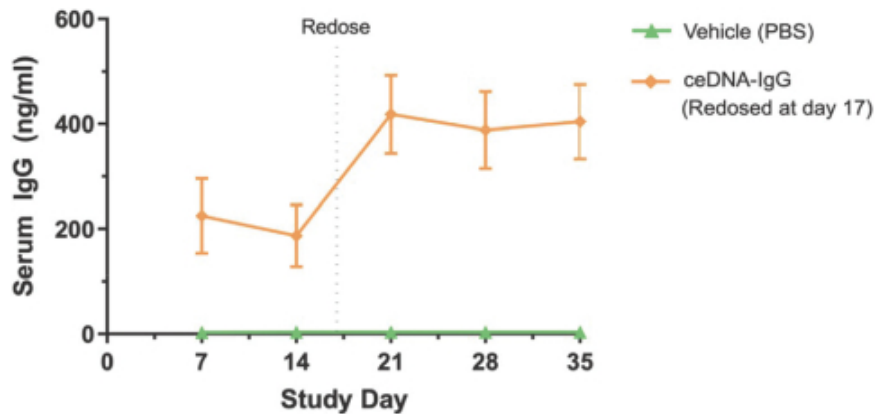
We have demonstrated hepatic expression of antibodies with ceDNA-AGT. As shown in the figure below, hydrodynamic injection of ceDNA-AGT resulted in serum mAb levels of greater than 40.0 µg/ml. Since many therapeutic mAbs are effective at serum concentrations of 1.0 to 5.0 µg/ml, we believe the level of mAb expression observed with our ceDNA-AGT is likely to be therapeutically relevant in humans.

### Hydrodynamic injection of ceDNA-AGT results in serum levels up to 40.0µg/ml



We have also demonstrated stacking of mAb expression after repeat dosing via hydrodynamic injection, as shown in the figure below.

### mAb expression increases after repeat dosing via hydrodynamic injection



*Next steps*

We are advancing our initial AGT program for the treatment of an infectious disease and we are evaluating ceDNA constructs against a surface protein for a variety of infectious agents, including hepatitis B virus, or HBV, human immunodeficiency virus, or HIV, and respiratory syncytial virus, or RSV. Separately, we have established a research collaboration with Vir Biotechnology, Inc. to explore the potential for our non-viral gene therapy platform to extend the duration and reach of Vir's mAb therapies against the SARS-CoV-2 coronavirus.

**Retinal diseases**

Approximately 200 million individuals suffer from inherited retinal diseases, in which a gene mutation leads to degeneration of the retina. Many of these diseases are caused by genes too large to be enclosed within AAV, including several types of LCA10 and Stargardt disease. Current AAV gene therapy approaches are not able to deliver genetic payload efficiently to cells that require replacement of the missing gene such as photoreceptors and/or retinal pigment epithelial cells, or RPE. We believe using ceDNA to deliver large gene payloads efficiently and specifically to relevant cell types in the retina by minimally invasive routes represents an important therapeutic approach.

**Lebers Congenital Amaurosis Type 10 (LCA10)**

*Overview*

LCA10 is the most common genetic cause of childhood vision loss. There are approximately 2,200 patients with LCA10 in the United States and 3,400 patients in the European Union. It is a severe, autosomal recessive retinal dystrophy due to a mutation in the CEP290 protein that is expressed in and required for proper functioning of retinal photoreceptors. LCA10 causes blindness or severe vision loss, often from birth or within the first year of life.

*Current approaches and limitations*

There are no approved therapies for LCA10. Several therapeutic programs are in clinical development for LCA10, including a splicing oligonucleotide given episodically by intravitreal injection and a gene editing approach. Both approaches address only a subset of LCA10 patients, specifically those with a CEP290 mutation amenable to splicing alteration, which makes up approximately 60% of the population. CEP290 is a large gene of 7.4 kb, which exceeds the 4.7 kb payload capacity of AAV. No single approach has been able to address the many mutations that cause LCA10.

*Our approach*

Our gene therapy approach aims to:

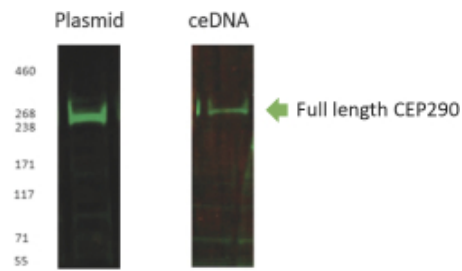
- deliver directly to the retina;
- treat patients irrespective of their CEP290 mutation;
- utilize ctLNP to specifically deliver CEP290 to photoreceptors and not to retinal glial and immune cells, thereby potentially increasing the safety, tolerability and durability of transgene expression;
- enable single-dose therapy delivered subretinally, which may increase efficacy and compliance when compared, for example, to episodic intravitreal delivery; and

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- treat infants and children to halt further decline of and restore vision.

We have created multiple plasmid and ceDNA constructs encoding the CEP290 gene and have demonstrated expression of full-length protein after transfection in cells in culture, as shown in the figure below.

### Plasmid and CEP290 express full-length protein in HEK293 cells *in vitro*



#### Next steps

We plan to declare a development candidate and enter IND-enabling studies in \_\_\_\_\_ for this program.

## **Stargardt disease**

### Overview

Stargardt disease is the most common inherited macular dystrophy. There are approximately 37,000 patients with Stargardt disease in the United States and 66,000 patients in the European Union. Stargardt disease is an autosomal recessive disease due to mutation in the ABCA4 gene that is expressed in both the RPE and retinal photoreceptors. Loss of ABCA4 function disrupts normal processing of retinaldehyde, or Vitamin A, in both photoreceptors and RPE cells leading to accumulation of toxic bis-retinoid byproducts and photoreceptor death. Patients usually present in childhood with loss of visual acuity and exhibit progressive loss of RPE and photoreceptor cells.

### Current approaches and limitations

There are currently no proven treatments for Stargardt disease. The ABCA4 gene is approximately 6.7 kb, too large to be delivered by AAV. There are currently no ongoing gene therapy trials for Stargardt disease. The goal of therapy in Stargardt disease is to preserve vision by correcting ABCA4 expression in enough photoreceptors and RPE cells to halt the progressive loss of these cells that would otherwise occur.

### Our approach

Our gene therapy approach aims to:

- deliver to photoreceptors and RPE cells to halt progressive loss in cells and preserve vision;
- protect photoreceptors and RPE cells through expression of normal ABCA4, resulting in clearance of bis-retinoid byproducts;

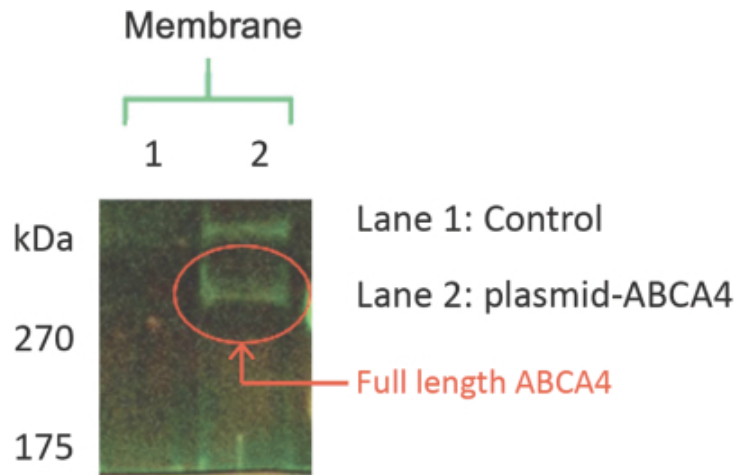


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- drive the appropriate level of ABCA4 expression in photoreceptors and RPE cells utilizing a native ABCA4 promoter;
- maintain normal macular vision and visual acuity when given to earlier-diagnosed patients; and
- deliver the entire ABCA4 gene in a single gene therapy vector, thereby increasing efficacy relative to dual AAV approaches.

We have generated plasmid constructs that express full length ABCA4. Further, as shown in the figure below, when we fractionate cellular lysates into cytosolic and membrane compartments, we see that plasmid ABCA4 expresses appropriately in the membrane bound subcellular compartment. This is important because ABCA4 is expressed in the cellular membrane of photoreceptors. Its cellular function requires that it be expressed and also appropriately trafficked to the correct intracellular compartment. The red circle in the figure below highlights the ABCA4 protein localized within cellular membrane.

### Expression and appropriate subcellular localization of plasmid ABCA4 in vitro



#### Next steps

We plan to declare a development candidate and enter IND-enabling studies in \_\_\_\_\_ for this program.

#### **Wet age-related macular degeneration (Wet AMD)**

##### Overview

Age-related macular degeneration, or AMD, is the leading cause of irreversible vision loss occurring in approximately 10 million people in the United States. Wet AMD is the most severe form, characterized by neovascularization of the retina, leading to significant loss in visual acuity and rapid progression to blindness. There are approximately 1.2 million patients with wet AMD in the United States and over 2.5 million patients in the European Union. Wet AMD is most common in individuals over the age of 50, with increasing incidence every decade thereafter.

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### *Current approaches and limitations*

Passive administration of anti-vascular endothelial growth factor, or anti-VEGF, mAbs are an established therapy for patients with wet AMD. Anti-VEGF therapy, such as EYLEA (aflibercept), is effective in slowing the loss of visual acuity in patients with wet AMD. However, the frequency of intravitreal administration is a barrier to adherence and to widespread adoption.

AAV gene therapy to establish intra-ocular expression of anti-VEGF molecules is in early clinical development, either by subretinal or intravitreal delivery. It is unclear today if these therapies will generate sufficient expression of anti-VEGF in the retina. If successful, the scale limitations of AAV may present additional challenges to providing therapy for a substantial proportion of wet AMD patients.

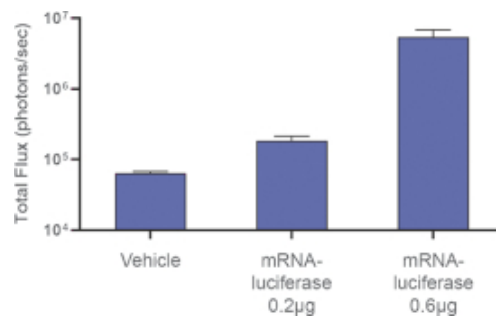
### *Our approach*

Our gene therapy approach aims to:

- deliver intravitreally to enable retinal cells to express and secrete an anti-VEGF molecule;
- provide a durable and sustained level of expression of anti-VEGF, which may engage regression of neovascularization and improve visual acuity and may enable administration only a few times over the life of a patient; and
- define when repeat therapy would be indicated to optimally preserve the retina.

In order to establish a baseline for the biodistribution of ctLNP in the eye, we administered mRNA-luciferase intravitreally at two doses of 0.2 $\mu$ g and 0.6 $\mu$ g using our ctLNP delivery system. As shown in the figure below, this resulted in luciferase expression in the retina.

### **Expression after intravitreal administration of mRNA-luciferase using ctLNP**



We have generated several anti-VEGF mAb ceDNA constructs and have shown that these constructs result in high levels of anti-VEGF mAb levels in the blood after hydrodynamic intravenous delivery.

### *Next steps*

We aim to combine these constructs with ctLNP to achieve intravitreal delivery of relevant antibody levels in the retina. We plan to declare a development candidate and enter IND-enabling studies in \_\_\_\_\_ for this program.

## ***Expansion opportunities in other tissues***

### ***Skeletal muscle***

There are a variety of genetic muscle disorders, including muscular dystrophies, that may be treated by efficient and systemic gene therapy to skeletal muscle. We are currently developing a ctLNP utilizing a targeting ligand to deliver ceDNA specifically to skeletal muscle.

We believe this approach would have several benefits for rare monogenic diseases of the skeletal muscle, including:

- early treatment, near the onset of disease, before inflammation and fibrosis progressively replace muscle fibers;
- delivery of full-length of defective skeletal muscle genes, which are often very large as is the case, for example, for dystrophin, the gene responsible for Duchenne Muscular Dystrophy, or DMD;
- efficient delivery of the gene of interest to enough muscle fibers to change the course of disease;
- sufficient expression within transduced muscle fibers to allow for normal constitution of the skeletal muscle fibers during growth and development; and
- keeping up with patients' needs over time through redosing.

We believe there are many diseases in the skeletal muscle with unmet need such as DMD, myotonic dystrophy, limb girdle dystrophies and fascioscapulohumeral dystrophies, which we may pursue in the future.

### ***CNS***

We plan to explore the use of ceDNA to correct disorders of the CNS. The work we are doing with local delivery in the retina to photoreceptors may inform expansion into the CNS, beginning for example with focal epilepsies, in which expression of a novel gene within a specific region of neurons may stop the abnormal seizures. One such example is Dravet syndrome, an epilepsy disorder that begins in infancy or early childhood usually caused by a loss-of-function mutation in the SCN1A gene.

### ***Oncology***

We plan to develop the use of ceDNA to treat a variety of cancer indications. We believe that we can use distinct targeting ligands to deliver ceDNA specifically and efficiently to tumors and can utilize ceDNA to express high levels of relevant proteins within the tumor. For example, we believe after systemic delivery and efficient uptake by tumors, the expression of checkpoint inhibitors and particular cytokines encoded by the ceDNA may have both direct tumoricidal activity and may stimulate the immune system to respond to and attack tumor cells. Many of the genes that we can express within tumors have limited efficacy and substantial safety and tolerability issues when given systemically. The key attribute of this approach is the ability to drive efficient and selective uptake of ceDNA within tumors and then local, high concentrations of relevant tumoricidal agents.

### ***Manufacturing***

We have personnel with extensive technical, manufacturing, analytical and quality experience to oversee all internal and contracted manufacturing and testing activities. Relying on these personnel, we have built development laboratories to produce ceDNA (drug substance) and lipid nanoparticle-encapsulated ceDNA (drug product) for use in our research activities. We have produced ceDNA drug substance at up to 200 liters and

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converted these materials to drug product through our ctLNP manufacturing process to support our research studies. We plan to use third-party CDMOs to support our IND-enabling studies and to fully supply our clinical trials and commercial activities. As we scale manufacturing, we intend to continue to expand and strengthen our network of CDMOs, and we will also consider investing in internal cGMP manufacturing capabilities and infrastructure in the future if there is a technical need or a strategic or financial benefit.

Manufacturing is subject to extensive regulations that impose procedural and documentation requirements. These regulations govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance. Our systems and contractors are required to comply with these regulations and are assessed through regular monitoring and formal audits.

### ***Drug substance***

We believe that the ceDNA drug substance requirements for our programs can be met by a variety of domestic and international contractors with standard biological manufacturing equipment. We have established a cGMP-ready process at the 200-liter scale, which we have transferred to our drug substance CDMOs to supply ceDNA drug substance for IND-enabling studies, clinical trials and early commercial activities. To ensure supply chain continuity, we have also established service agreements with additional suppliers to afford redundancy and flexibility in scaling. We have access rights to a biologics facility at one of our CDMOs that we believe could fully support multiple clinical programs if and when we advance our programs into clinical trials, as well as the early phases of commercialization.

We have invested in technical expertise and internal capabilities to optimize and develop the ceDNA drug substance process and to provide technical management and quality oversight for our process transfers to CDMOs.

Future ceDNA drug substance processes may require additional manufacturing capabilities, which may be addressed by either expanding our capabilities with existing contractors or establishing manufacturing supply relationships with new contract manufacturers. These changes in processes may also require new supply chain agreements with CDMOs that specialize in raw material manufacturing.

### ***Drug product***

Our drug product is ceDNA formulated with ctLNP. We believe that our drug product requirements can be met by a variety of domestic and international CDMOs. We have selected a subset of experienced organizations familiar with the specific operations that our current drug product processes require. We have established a service agreement with one of these CDMOs and have also engaged with suppliers for key components of our ctLNP delivery system.

We have invested in technical expertise and internal capabilities to optimize and develop the drug product process and to provide technical management and quality oversight for our process transfers to CDMOs. We have transitioned our drug product process from research-scale using microfluidics to standard clinical-scale equipment supporting cGMP operations for other established LNP-based modalities. These scaled systems have generated representative and well-characterized drug product that we have used in our preclinical studies. Additionally, as with our drug substance processes, progress on analytics has allowed us to leverage insights into our delivery system to upgrade and characterize purity and homogeneity. We plan to continue to implement process changes to improve purity and yield.

### **Intellectual property**

We strive to protect our proprietary technology, inventions, improvements, platforms, product candidates and components thereof, their methods of use and processes for their manufacture that we believe are important to

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our business, including by obtaining, maintaining, defending and enforcing patent and other intellectual property rights for the foregoing in the United States and in certain foreign jurisdictions. We also rely on trade secrets and confidentiality agreements to protect our confidential information and know-how and other aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection.

Our success depends in part on our ability to:

- obtain, maintain, enforce and defend patent and other intellectual property rights for our commercially important technology, inventions and improvements;
- preserve the confidentiality of our trade secrets and other confidential information;
- obtain and maintain licenses to use and exploit intellectual property owned or controlled by third parties;
- operate without infringing, misappropriating or otherwise violating any valid and enforceable patents and other intellectual property rights of third parties; and
- defend against challenges and assertions by third parties challenging the validity or enforceability of our intellectual property rights, or our rights in our intellectual property, or asserting that the operation of our business infringes, misappropriates or otherwise violates their intellectual property rights.

### **Patent portfolio**

As of \_\_\_\_\_, 2020, we own approximately \_\_\_\_\_ Patent Cooperation Treaty, or PCT, patent applications, \_\_\_\_\_ of which have entered the national stage in the United States and certain foreign jurisdictions, including Europe and Japan, and we exclusively license one patent application family, which has entered the national stage in the United States and certain foreign jurisdictions, including Europe and Japan. We also non-exclusively license one patent application family, which includes issued patents in each of the United States, Australia and Israel and national stage patent applications in several other jurisdictions, including Europe and Japan. In addition, we own approximately \_\_\_\_\_ U.S. provisional patent applications within the priority year. We do not currently own or exclusively license any issued patents covering any of our programs or technology, including the ceDNA platform, ctLNP delivery system and manufacturing processes. Our owned and licensed patent applications cover various aspects of our programs and technology, including our ceDNA construct, ctLNP delivery system and manufacturing process as further described below. Any U.S. or foreign patents issued from national stage filings of our owned or exclusively in-licensed PCT patent applications and any U.S. patents issued from non-provisional applications we may file in connection with our provisional patent applications would be scheduled to expire on various dates from \_\_\_\_\_ through \_\_\_\_\_, without taking into account any possible patent term adjustments or extensions and assuming payment of all appropriate maintenance, renewal, annuity and other governmental fees.

### *ceDNA construct*

As of \_\_\_\_\_, 2020, we own approximately \_\_\_\_\_ PCT patent applications, \_\_\_\_\_ of which have entered the national stage in a number of jurisdictions outside the United States, and exclusively license from the University of Massachusetts one patent application family, which has entered the national stage in the United States and other jurisdictions, including Europe and Japan. These patent applications cover various aspects of our ceDNA construct, including ceDNA construct variants, certain disease-targeted ceDNA compositions and methods of use. We have also non-exclusively licensed one patent application family from the National Institutes of Health, or the NIH, and the Institut de Myologie, Universite Pierre et Marie Curie, Centre National de la Recherche Scientifique and Inserm Transfert SA, which we refer to as the French Institutions, which includes issued patents in each of the United States, Australia and Israel and national stage patent applications

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in other jurisdictions, including Europe and Japan, which cover our ceDNA construct, certain disease-targeted ceDNA compositions and methods of use. In addition, we own approximately [redacted] U.S. provisional patent applications within the priority year, which cover ceDNA construct variants, general applications of the ceDNA construct technology and certain properties of the construct, specific disease-targeted ceDNA compositions and methods of use. Any U.S. or foreign patents issued from the pending U.S. or foreign non-provisional patent applications or from non-provisional applications we may file in connection with the pending provisional patent applications would be scheduled to expire on various dates from [redacted] through [redacted], without taking into account any possible patent term adjustments or extensions and assuming payment of all appropriate maintenance, renewal, annuity and other governmental fees.

### *ctLNP delivery system*

As of [redacted], 2020, we own approximately [redacted] PCT patent applications, [redacted] of which have entered the national stage in the United States and a number of jurisdictions outside the United States, and approximately [redacted] U.S. provisional patent applications within the priority year with respect to our ctLNP delivery system, including certain lipid and lipid nanoparticle compositions and combinations with ceDNA and/or targeting agents and methods of use. Any U.S. or foreign patents issued from the pending U.S. or foreign non-provisional patent applications or from any non-provisional applications we may file in connection with these provisional patent applications would be scheduled to expire on various dates from [redacted] through [redacted], without taking into account any possible patent term adjustments or extensions and assuming payment of all appropriate maintenance, renewal, annuity and other governmental fees.

### *Manufacturing processes*

As of [redacted], 2020, we own approximately [redacted] PCT patent applications and approximately [redacted] U.S. provisional patent applications within the priority year with respect to our ceDNA manufacturing processes. Any U.S. or foreign patents issued from the pending U.S. or foreign non-provisional patent applications or from any non-provisional applications we may file in connection with these provisional patent applications would be scheduled to expire on various dates from [redacted] through [redacted], without taking into account any possible patent term adjustments or extensions and assuming payment of all appropriate maintenance, renewal, annuity and other governmental fees.

### **Patent prosecution**

A PCT patent application is not eligible to become an issued patent until, among other things, we file one or more national stage patent applications within 30 months, 31 months or 32 months of the PCT application's priority date, depending on the jurisdiction, in the countries in which we seek patent protection. If we do not timely file any national stage patent applications, we may lose our priority date with respect to our PCT patent application and any potential patent protection on the inventions disclosed in such PCT patent application.

Moreover, a provisional patent application is not eligible to become an issued patent. A provisional patent application may serve as a priority filing for a non-provisional patent application we file within 12 months of such provisional patent application. If we do not timely file non-provisional patent applications, we may lose our priority date with respect to our existing provisional patent applications and any potential patent protection on the inventions disclosed in our provisional patent applications.

While we intend to timely file additional provisional patent applications and national stage and non-provisional patent applications relating to our PCT patent applications, we cannot predict whether any of our patent

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applications will result in the issuance of patents. If we do not successfully obtain patent protection, or if the scope of the patent protection we or our licensors obtain with respect to our product candidates or technology, including our ceDNA constructs, ctLNP delivery system or manufacturing processes is not sufficiently broad, we will be unable to prevent others from using our technology or from developing or commercializing technology and products similar or identical to ours or other similar competing products and technologies. Our ability to stop third parties from making, using, selling, offering to sell, importing or otherwise commercializing any of our technology, inventions and improvements, either directly or indirectly, will depend in part on our success in obtaining, maintaining, defending and enforcing patent claims that cover our technology, inventions and improvements.

The patent positions of companies like ours are generally uncertain and involve complex legal and factual questions. The protection afforded by a patent varies on a product-by-product basis, from jurisdiction-to-jurisdiction, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of patent term adjustments and regulatory-related patent term extensions, the availability of legal remedies in a particular jurisdiction and the validity and enforceability of the patent. No consistent policy regarding the scope of patent claims allowable in the field of genetic therapy has emerged in the United States. Moreover, patent laws and related enforcement in various jurisdictions outside of the United States are uncertain and may not protect our rights to the same extent as the laws of the United States. Changes in the patent laws and rules, whether by legislation, judicial decisions or regulatory interpretation, in the United States and other jurisdictions may diminish our ability to protect our inventions and obtain, maintain, defend and enforce our patent rights, and could therefore affect the value of our business.

The area of patent and other intellectual property rights in biotechnology is evolving and has many risks and uncertainties, and third parties may have blocking patents and other intellectual property that could be used to prevent us from commercializing our platforms and product candidates and practicing our proprietary technology. Our patent rights may be challenged, narrowed, circumvented, invalidated or ruled unenforceable, which could limit our ability to stop third parties from marketing and commercializing related platforms or product candidates or limit the term of patents that cover our platforms and product candidates. In addition, the rights granted under any issued patents may not provide us with protection or competitive advantages against third parties with similar technology, and third parties may independently develop similar technologies. Moreover, because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that before any of our product candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby reducing any competitive advantage provided by the patent. For this and other risks related to our proprietary technology, inventions, improvements, platforms and product candidates and intellectual property rights related to the foregoing, please see the section entitled “Risk factors—Risks related to our intellectual property.”

### ***Patent term extensions***

The term of individual patents depends upon the laws of the jurisdictions in which they are obtained. In most jurisdictions in which we file, the patent term is 20 years from the earliest date of filing of the first non-provisional patent application to which the patent claims priority. However, the term of U.S. patents may be extended or adjusted for delays incurred due to compliance with FDA requirements or by delays encountered during prosecution that are caused by the United States Patent and Trademark Office, or the USPTO. For example, in the United States, a patent claiming a new biologic product, its method of use or its method of manufacture may be eligible for a limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, for up to five years beyond the normal expiration date of the patent. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product’s approval date in the United States. Only one patent applicable to an

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approved product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. For more information on patent term extensions, see "Business—Government regulation—Patent term restoration and extension". In the future, if and when any product candidates we may develop receive FDA approval, we expect to apply for patent term extensions on issued patents covering those product candidates. Moreover, we intend to seek patent term adjustments and extensions for any of our issued patents in any jurisdiction where such adjustments and extensions are available. However, there is no guarantee that the applicable authorities, including the USPTO and FDA, will agree with our assessment of whether such adjustments and extensions should be granted, and even if granted, the length of such adjustments and extensions.

### **Trade secrets**

In addition to patent protection, we also rely on trade secrets, know-how, unpatented technology and other proprietary information to strengthen our competitive position. We take steps to protect and preserve our trade secrets and other confidential and proprietary information and prevent the unauthorized disclosure of the foregoing, including by entering into non-disclosure and invention assignment agreements with parties who have access to our trade secrets or other confidential and proprietary information, such as employees, consultants, outside scientific collaborators, contract research and manufacturing organizations, sponsored researchers and other advisors, at the commencement of their employment, consulting or other relationships with us. In addition, we take other appropriate precautions, such as maintaining physical security of our premises and physical and electronic security of our information technology systems, to guard against any misappropriation or unauthorized disclosure of our trade secrets and other confidential and proprietary information by third parties.

Despite these efforts, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or other confidential or proprietary information. In addition, we cannot provide any assurances that all of the foregoing non-disclosure and invention assignment agreements have been duly executed, and any of the counterparties to such agreements may breach them and disclose our trade secrets and other confidential and proprietary information. Although we have confidence in the measures we take to protect and preserve our trade secrets and other confidential and proprietary information, they may be inadequate, our agreements or security measures may be breached, and we may not have adequate remedies for such breaches. Moreover, to the extent that our employees, contractors, consultants, collaborators and advisors use intellectual property owned by others in their work for us, disputes may arise as to our rights in any know-how or inventions arising out of such work. For more information, please see the section entitled "Risk factors—Risks related to our intellectual property."

### **License agreements**

We are a party to a number of license agreements under which we license patents, patent applications and other intellectual property from third parties. These licenses impose various diligence and financial payment obligations on us. We expect to continue to enter into these types of license agreements in the future. We consider the following license agreements to be material to our business.

#### *License agreement with the National Institutes of Health*

In February 2017, we entered into a license agreement with the NIH, which was amended in July 2019 to include the French Institutions as licensors. Pursuant to the amended agreement, or the NIH Agreement, NIH and the French Institutions granted us a worldwide, non-exclusive license under a patent application family related to



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our ceDNA construct. This patent application family includes national stage patent applications in jurisdictions outside the United States, including Europe and Japan, and issued patents in each of the United States, Australia and Israel. The non-exclusive license confers the right to make and have made, research and have researched, use and have used, sell and have sold, offer to sell and import products and to practice processes, in each case, covered by the licensed patents and patent applications, for the treatment, prevention or palliation of any human disease or condition.

Under the NIH Agreement, we are obligated to use reasonable commercial efforts to ensure that the licensed products and processes are utilized and made available to the public on reasonable terms, including pursuing commercially reasonable broad international patient accessibility for licensed gene therapy products for the treatment of rare diseases, in accordance with an agreed upon commercial development plan for gene therapy-based human therapeutics and certain performance milestone events.

Unless terminated earlier, the NIH Agreement remains in effect until the last to expire of the licensed patent rights on a licensed product-by-licensed product and country-by-country basis. NIH and the French Institutions may terminate the NIH Agreement if we fail to perform our material obligations, including but not limited to our failure to meet the applicable performance milestones despite using commercially reasonable efforts, and have not remediated such deficiency within a specified time period. NIH and the French Institutions can terminate the NIH Agreement in the event we become insolvent, file a petition in bankruptcy, have such a petition filed against us, or determine to file a petition in bankruptcy. In addition, NIH and the French Institutions may terminate the NIH Agreement in the event of a material breach by us and failure to cure such breach within a certain period of time. We can voluntarily terminate the NIH Agreement with prior notice to NIH and the French Institutions.

As part of the NIH Agreement, we agreed to make milestone payments upon the achievement of certain milestones up to a maximum aggregate total of \$350,000 for each licensed product, as well as a low single-digit royalty on net sales of licensed products. These royalty obligations last on a licensed product-by-licensed product and country-by-country basis until the expiration of the last licensed patent rights covering such licensed product in such country. In addition, if we sublicense rights under the NIH Agreement, we are required to pay a high single-digit percent of the sublicense revenue to NIH. Additionally, under the NIH Agreement, we may be required to reimburse the French Institutions for a portion of certain past and ongoing patent related expenses, including expenses associated with the preparation, filing, prosecution and maintenance of all patents and patent applications. As of December 31, 2019, there have been no invoiced expenses related to these reimbursable costs.

### *License Agreement with the University of Massachusetts*

In June 2017, we entered into a license agreement, or the UMass Agreement, with the University of Massachusetts as represented by and solely on behalf of its Medical School, or UMass, pursuant to which UMass granted us an exclusive, worldwide license under a patent application family related to our ceDNA construct, which has pending national stage patent applications in the United States and certain foreign jurisdictions. The exclusive license confers the right to research, develop, manufacture, have manufactured, use, offer for sale, sell and import products and practice and have practiced processes, in each case, covered by the patent application family.

Unless terminated earlier, the UMass Agreement will continue until the last-to-expire valid claim of the licensed patents. UMass may terminate the UMass Agreement if we fail to perform our material obligations, including but not limited to our failure to meet the applicable performance milestones despite using commercially reasonable efforts, and have not remediated such deficiency within a specified time period or negotiated a revised performance timeline. UMass can terminate the UMass Agreement if we fail to make any payments

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within a specified period after receiving written notice of such failure, or in the event of a material breach by us and failure to cure such breach within a certain period of time, provided that, if we fail to make payments due under the UMass Agreement more than a certain number of times, UMass may terminate the agreement immediately without any cure period. We can voluntarily terminate the UMass Agreement with prior notice to UMass.

Under the UMass Agreement, we agreed to use diligent efforts to develop the licensed products and introduce them into the commercial market and make them reasonably available to the public thereafter. Specifically, we agreed to achieve regulatory approval for and commercially launch at least one licensed product in the U.S. by certain specified dates.

As part of the UMass Agreement, we have issued to UMass 221,985 shares of our common stock. In addition, we may be obligated to make milestone payments up to \$762,500 per licensed product that are contingent upon the achievement of certain regulatory and commercialization milestones, as well as low single-digit royalties on net sales of licensed products on a licensed product-by-licensed product and country-by-country basis. If we sublicense our rights under the UMass Agreement, we are required to pay a low-to-mid single-digit percentage of the license revenue to UMass, which will vary depending on when the sublicense agreement to a third party was executed. Royalty obligations under the UMass Agreement will continue until the expiration of the last valid claim of a licensed patent covering such licensed product in such country. As of December 31, 2019, we have recorded no royalty or milestone liabilities under the UMass Agreement.

## **Competition**

The biotechnology and biopharmaceutical industries generally, and the genetic medicine field specifically, are characterized by rapid evolution of technologies, sharp competition and strong defense of intellectual property. Any product candidates that we successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While we believe that our technology, development experience and scientific knowledge in the field of gene therapy, nucleic acid delivery and manufacturing provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies and public and private research institutions.

There are numerous companies that are selling or developing genetic medicines, including in indications for which we may develop our non-viral gene therapies. These companies include viral gene therapy companies such as BioMarin Pharmaceuticals, Inc., Homology Medicines, Inc., Adverum Biotechnologies, Inc. and Hoffmann La Roche Ltd.; gene editing companies such as CRISPR Therapeutics, AG and Intellia Therapeutics, Inc.; and mRNA companies such as Moderna, Inc.

Many of our competitors, either independently or with strategic partners, have substantially greater financial, technical and human resources than we do. Accordingly, our competitors may be more successful than we are in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approval for treatments and achieving widespread market acceptance. Merger and acquisition activity in the biotechnology and biopharmaceutical industries may result in resources being concentrated among a smaller number of our competitors. These companies also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials and acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Our commercial opportunity could be substantially limited if our competitors develop and commercialize products that are more effective, safer, less toxic, more convenient or less expensive than products we may

develop. In geographies that are critical to our commercial success, competitors may also obtain regulatory approvals before us, resulting in our competitors building a strong market position in advance of the entry of our products. In addition, our ability to compete may be affected in many cases by insurers or other third-party payers seeking to encourage the use of other drugs. The key competitive factors affecting the successful of all any products we may develop are likely to be their efficacy, safety, convenience, price and availability of reimbursement.

## **Government regulation**

Government authorities in the United States, at the federal, state and local level and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, pricing, reimbursement, sales, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting and import and export of pharmaceutical products, including biological products. The processes for obtaining marketing approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

### ***Licensure and regulation of biologics in the United States***

In the United States, any product candidates we may develop would be regulated as biological products, or biologics, under the Public Health Service Act, or PHSA, and the Federal Food, Drug and Cosmetic Act, or FDCA, and its implementing regulations and guidance. The failure to comply with the applicable U.S. requirements at any time during the product development process, including preclinical testing, clinical testing, the approval process, or post-approval process, may subject an applicant to delays in the conduct of the study, regulatory review and approval and/or administrative or judicial sanctions. These sanctions may include, but are not limited to, the FDA's refusal to allow an applicant to proceed with clinical testing, refusal to approve pending applications, license suspension, or revocation, withdrawal of an approval, warning letters, adverse publicity, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines and civil or criminal investigations and penalties brought by the FDA or the Department of Justice, or DOJ, and other governmental entities, including state agencies.

An applicant seeking approval to market and distribute a new biologic in the United States generally must satisfactorily complete each of the following steps:

- preclinical laboratory tests, animal studies and formulation studies all performed in accordance with the FDA's Good Laboratory Practices, or GLP regulations;
- completion of the manufacture, under cGMP conditions, of the drug substance and drug product that the sponsor intends to use in human clinical trials along with required analytical and stability testing;
- submission to the FDA of an IND application for human clinical testing, which must become effective before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials to establish the safety, potency and purity of the product candidate for each proposed indication, in accordance with current Good Clinical Practices, or GCP;

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- preparation and submission to the FDA of a BLA for a biologic product requesting marketing for one or more proposed indications, including submission of detailed information on the manufacture and composition of the product in clinical development and proposed labelling;
- review of the product by an FDA advisory committee, where appropriate or if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities, including those of third parties, at which the product, or components thereof, are produced to assess compliance with current Good Manufacturing Practices, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of any FDA audits of the preclinical studies and clinical trial sites to assure compliance with GLP, as applicable, and GCP, and the integrity of clinical data in support of the BLA;
- payment of user Prescription Drug User Fee Act, or PDUFA, securing FDA approval of the BLA and licensure of the new biologic product; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and any post-approval studies or other post-marketing commitments required by the FDA.

### *Preclinical studies and investigational new drug application*

Before testing any biologic product candidate in humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests include laboratory evaluations of product chemistry, formulation and stability, as well as studies to evaluate the potential for efficacy and toxicity in animal studies. The conduct of the preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements. The results of the preclinical tests, together with manufacturing information and analytical data, are submitted to the FDA as part of an IND application.

An IND is an exemption from the FDCA that allows an unapproved product candidate to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer such investigational product to humans. The IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions about the product or conduct of the proposed clinical trial, including concerns that human research subjects will be exposed to unreasonable health risks. In that case, the IND sponsor and the FDA must resolve any outstanding FDA concerns before the clinical trials can begin or recommence.

As a result, submission of the IND may result in the FDA not allowing the trials to commence or allowing the trial to commence on the terms originally specified by the sponsor in the IND. If the FDA raises concerns or questions either during this initial 30-day period, or at any time during the IND review process, it may choose to impose a partial or complete clinical hold. Clinical holds are imposed by the FDA whenever there is concern for patient safety, may be a result of new data, findings, or developments in clinical, preclinical and/or chemistry, manufacturing and controls or where there is non-compliance with regulatory requirements. This order issued by the FDA would delay either a proposed clinical trial or cause suspension of an ongoing trial, until all outstanding concerns have been adequately addressed and the FDA has notified the company that investigations may proceed. This could cause significant delays or difficulties in completing our planned clinical trial or future clinical trials in a timely manner.

Additionally, gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the NIH also are potentially subject to review by a committee within the NIH's Office of Science

Policy called the Novel and Exceptional Technology and Research Advisory, or the NExTRAC. As of 2019, the charter of this review group has evolved to focus public review on clinical trials that cannot be evaluated by standard oversight bodies and pose unusual risks. With certain gene therapy protocols, FDA review of or clearance to allow the IND to proceed could be delayed if the NExTRAC decides that full public review of the protocol is warranted. The FDA also may impose clinical holds on a biologic product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA.

*Expanded access to an investigational drug for treatment use*

Expanded access, sometimes called “compassionate use,” is the use of investigational products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. The rules and regulations related to expanded access are intended to improve access to investigational products for patients who may benefit from investigational therapies. FDA regulations allow access to investigational products under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the investigational product under a treatment protocol or treatment IND application.

When considering an IND application for expanded access to an investigational product with the purpose of treating a patient or a group of patients, the sponsor and treating physicians or investigators will determine suitability when all of the following criteria apply: patient(s) have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; the potential patient benefit justifies the potential risks of the treatment and the potential risks are not unreasonable in the context or condition to be treated; and the expanded use of the investigational drug for the requested treatment will not interfere with initiation, conduct, or completion of clinical investigations that could support marketing approval of the product or otherwise compromise the potential development of the product.

There is no obligation for a sponsor to make its drug products available for expanded access; however, as required by the 21st Century Cures Act, or Cures Act, passed in 2016, if a sponsor has a policy regarding how it responds to expanded access requests, it must make that policy publicly available. Although these requirements were rolled out over time, they have now come into full effect. This provision requires drug and biologic companies to make publicly available their policies for expanded access for individual patient access to products intended for serious diseases. Sponsors are required to make such policies publicly available upon the earlier of initiation of a Phase 2 or Phase 3 trial; or 15 days after the investigational drug or biologic receives designation as a breakthrough therapy, fast track product, or regenerative medicine advanced therapy.

In addition, on May 30, 2018, the Right to Try Act was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a manufacturer to make its investigational products available to eligible patients as a result of the Right to Try Act.

*Human clinical trials in support of a BLA*

Clinical trials involve the administration of the investigational product candidate to healthy volunteers or patients with the disease or condition to be treated under the supervision of a qualified principal investigator in

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accordance with GCP requirements. Clinical trials are conducted under protocols detailing, among other things, the objectives of the trial, inclusion and exclusion criteria, the parameters to be used in monitoring safety, and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND.

A sponsor who wishes to conduct a clinical trial outside the United States may, but need not, obtain FDA authorization to conduct the clinical trial under an IND. When a foreign clinical trial is conducted under an IND, all FDA IND requirements must be met unless waived. When a foreign clinical trial is not conducted under an IND, the sponsor must ensure that the trial complies with certain regulatory requirements of the FDA in order to use the trial as support for an IND or application for marketing approval. Specifically, the FDA requires that such trials be conducted in accordance with GCP, including review and approval by an independent ethics committee and informed consent from participants. The GCP requirements encompass both ethical and data integrity standards for clinical trials. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical trials, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign trials are conducted in a manner comparable to that required for clinical trials in the United States.

Further, each clinical trial must be reviewed and approved by an IRB either centrally or individually at each institution at which the clinical trial will be conducted. The IRB will consider, among other things, clinical trial design, patient informed consent, ethical factors, the safety of human subjects, and the possible liability of the institution. An IRB must operate in compliance with FDA regulations. The FDA, IRB, or the clinical trial sponsor may suspend or discontinue a clinical trial at any time for various reasons, including a finding that the clinical trial is not being conducted in accordance with FDA requirements or that the participants are being exposed to an unacceptable health risk. Clinical testing also must satisfy extensive GCP rules and the requirements for informed consent.

Additionally, some clinical trials are overseen by an independent group of qualified experts organized by the clinical trial sponsor, known as a data safety monitoring board, or DSMB. This group may recommend continuation of the trial as planned, changes in trial conduct, or cessation of the trial at designated check points based on certain available data from the trial to which only the DSMB has access. Finally, research activities involving infectious agents, hazardous chemicals, recombinant DNA and genetically altered organisms and agents may be subject to review and approval of an Institutional Biosafety Committee, or IBC, in accordance with NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules.

Clinical trials typically are conducted in three sequential phases, but the phases may overlap or be combined. Additional studies may be required after approval.

- *Phase 1* clinical trials are initially conducted in a limited population to test the product candidate for safety, including adverse effects, dose tolerance, absorption, metabolism, distribution, excretion and pharmacodynamics in healthy humans or, on occasion, in patients, such as cancer patients.
- *Phase 2* clinical trials are generally conducted in a limited patient population to identify possible adverse effects and safety risks, evaluate the efficacy of the product candidate for specific targeted indications and determine dose tolerance and optimal dosage. Multiple Phase 2 clinical trials may be conducted by the sponsor to obtain information prior to beginning larger and more costly Phase 3 clinical trials.
- *Phase 3* clinical trials proceed if the Phase 2 clinical trials demonstrate that a dose range of the product candidate is potentially effective and has an acceptable safety profile. Phase 3 clinical trials are undertaken within an expanded patient population to further evaluate dosage, provide substantial evidence of clinical efficacy and further test for safety in an expanded and diverse patient population at multiple, geographically dispersed clinical trial sites. A well-controlled, statistically robust Phase 3 trial may be designed to deliver the

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data that regulatory authorities will use to decide whether or not to approve, and, if approved, how to appropriately label a biologic; such Phase 3 studies are referred to as “pivotal.”

In some cases, the FDA may approve a BLA for a product but require the sponsor to conduct additional clinical trials to further assess the product’s safety and effectiveness after approval. Such post-approval trials are typically referred to as Phase 4 clinical trials. These studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication and to document a clinical benefit in the case of biologics approved under accelerated approval regulations. If the FDA approves a product while a company has ongoing clinical trials that were not necessary for approval, a company may be able to use the data from these clinical trials to meet all or part of any Phase 4 clinical trial requirement or to request a change in the product labeling. The failure to exercise due diligence with regard to conducting Phase 4 clinical trials could result in withdrawal of approval for products.

Under the Pediatric Research Equity Act of 2003, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA, and the FDA’s internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

For products intended to treat a serious or life-threatening disease or condition, the FDA must, upon the request of an applicant, meet to discuss preparation of the initial pediatric study plan or to discuss deferral or waiver of pediatric assessments. In addition, FDA will meet early in the development process to discuss pediatric study plans with sponsors and FDA must meet with sponsors by no later than the end-of-phase 1 meeting for serious or life-threatening diseases and by no later than 90 days after FDA’s receipt of the study plan.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in the Food and Drug Administration Safety and Innovation Act. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

Information about applicable clinical trials must be submitted within specific timeframes to the NIH for public dissemination on its ClinicalTrials.gov website.

### *Special regulations and guidance governing gene therapy products*

We expect that the procedures and standards applied to gene therapy products will be applied to any product candidates we may develop. The FDA has defined a gene therapy product as one that seeks to modify or manipulate the expression of a gene or to alter the biological properties of living cells for therapeutic use. The products may be used to modify cells *in vivo* or transferred to cells *ex vivo* prior to administration to the recipient.

Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the Office of Tissues and

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Advanced Therapies, or OTAT, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The NIH, including the NExTRAC also advises the FDA on gene therapy issues and other issues related to emerging biotechnologies. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols.

The FDA has issued various guidance documents regarding gene therapies, including recent final guidance documents released in January 2020 relating to chemistry, manufacturing and controls information for gene therapy INDs, long-term follow-up after the administration of gene therapy products, gene therapies for rare diseases and gene therapies for retinal disorders. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, compliance with them is likely necessary to gain approval for any gene therapy product candidate. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things: the proper preclinical assessment of gene therapies; the chemistry, manufacturing and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe for potential delayed adverse effects in participants who have received investigational gene therapies with the duration of follow-up based on the potential for risk of such effects. For AAV vectors specifically, the FDA typically recommends that sponsors continue to monitor participants for potential gene therapy-related adverse events for up to a 5-year period.

Until 2019, most gene therapy clinical trials in the United States required pre-review by the predecessor of NExTRAC before being approved by the IRBs and any local biosafety boards or being allowed to proceed by FDA. In 2019, the NIH substantially eliminated the pre-review process and going forward, the review of gene therapy clinical trial protocols would be largely handled by local IRBs and IBCs, in addition to FDA. Furthermore, in 2019, the NIH removed from public access the Genetic Modification Clinical Research Information System database, which previously contained substantial amounts of safety and other participant information regarding human gene therapy trials performed up to that time.

### *Compliance with cGMP requirements*

Before approving a BLA, the FDA typically will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in full compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. The PHSA emphasizes the importance of manufacturing control for products like biologics whose attributes cannot be precisely defined.

Manufacturers and others involved in the manufacture and distribution of products must also register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Any product manufactured by or imported from a facility that has not registered, whether foreign or domestic, is deemed misbranded under the FDCA. Establishments may be subject to periodic unannounced inspections by government authorities to ensure compliance with cGMPs and other laws. Inspections must follow a “risk-based schedule” that may result in certain establishments being inspected more frequently. Manufacturers may also have to provide, on request, electronic or physical records regarding their establishments. Delaying, denying, limiting, or refusing inspection by the FDA may lead to a product being deemed to be adulterated.

### *Review and approval of a BLA*

The results of product candidate development, preclinical testing and clinical trials, including negative or ambiguous results as well as positive findings, are submitted to the FDA as part of a BLA requesting license to



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market the product. The BLA must contain extensive manufacturing information and detailed information on the composition of the product and proposed labeling as well as payment of a user fee. Under federal law, the submission of most BLAs is subject to an application user fee, which for federal fiscal year 2020 is \$2,942,965 for an application requiring clinical data. The sponsor of a licensed BLA is also subject to an annual program fee, which for fiscal year 2020 is \$325,424. Certain exceptions and waivers are available for some of these fees, such as an exception from the application fee for products with orphan designation and a waiver for certain small businesses.

The FDA has 60 days after submission of the application to conduct an initial review to determine whether it is sufficient to accept for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission has been accepted for filing, the FDA begins an in-depth review of the application. Under the goals and policies agreed to by the FDA under the PDUFA, the FDA has ten months in which to complete its initial review of a standard application and respond to the applicant, and six months for a priority review of the application. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs. The review process may often be significantly extended by FDA requests for additional information or clarification. The review process and the PDUFA goal date may be extended by three months if the FDA requests or if the applicant otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Under the PHSA, the FDA may approve a BLA if it determines that the product is safe, pure and potent, and the facility where the product will be manufactured meets standards designed to ensure that it continues to be safe, pure and potent. On the basis of the FDA's evaluation of the application and accompanying information, including the results of the inspection of the manufacturing facilities and any FDA audits of preclinical and clinical trial sites to assure compliance with GCPs, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. If the application is not approved, the FDA will issue a complete response letter, which will contain the conditions that must be met in order to secure final approval of the application, and when possible will outline recommended actions the sponsor might take to obtain approval of the application. Sponsors that receive a complete response letter may submit to the FDA information that represents a complete response to the issues identified by the FDA. Such resubmissions are classified under PDUFA as either Class 1 or Class 2. The classification of a resubmission is based on the information submitted by an applicant in response to an action letter. Under the goals and policies agreed to by the FDA under PDUFA, the FDA has two months to review a Class 1 resubmission and six months to review a Class 2 resubmission. The FDA will not approve an application until issues identified in the complete response letter have been addressed.

The FDA may also refer the application to an advisory committee for review, evaluation and recommendation as to whether the application should be approved. In particular, the FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

If the FDA approves a new product, it may limit the approved indication(s) for use of the product. It may also require that contraindications, warnings, or precautions be included in the product labeling. In addition, the FDA may call for post-approval studies, including Phase 4 clinical trials, to further assess the product's efficacy and/or safety after approval. The agency may also require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, to help ensure that the benefits of the product outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals and

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elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patent registries. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, many types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

### *Fast track, breakthrough therapy, priority review and regenerative medicine advanced therapy designations*

The FDA is authorized to designate certain products for expedited review if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition. These programs are referred to as fast track designation, breakthrough therapy designation, priority review designation and regenerative medicine advanced therapy, or RMAT, designation. These designations are not mutually exclusive, and a product candidate may qualify for one or more of these programs. While these programs are intended to expedite product development and approval, they do not alter the standards for FDA approval.

Specifically, the FDA may designate a product for fast track review if it is intended, whether alone or in combination with one or more other products, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For fast track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a fast track product's application before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a fast track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. However, the FDA's time period goal for reviewing a fast track application does not begin until the last section of the application is submitted. In addition, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Second, in 2012, Congress enacted the Food and Drug Administration Safety and Innovation Act. This law established a new regulatory scheme allowing for expedited review of products designated as "breakthrough therapies." A product may be designated as a breakthrough therapy if it is intended, either alone or in combination with one or more other products, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA may take certain actions with respect to breakthrough therapies, including holding meetings with the sponsor throughout the development process; providing timely advice to the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; and taking other steps to design the clinical trials in an efficient manner. Breakthrough designation may be rescinded if a product no longer meets the qualifying criteria.

Third, the FDA may designate a product for priority review if it is a product that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The FDA determines, on a case-by-case basis, whether the proposed product represents a significant improvement when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting product reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, and evidence of safety and effectiveness in a new subpopulation. A priority designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action

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on a marketing application from ten months to six months. Priority designation may be rescinded if a product no longer meets the qualifying criteria.

With passage of the Cures Act in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative medicine advanced therapies. A product is eligible for RMAT designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product has the potential to address unmet medical needs for such disease or condition. In a recent guidance on expedited programs for regenerative medicine therapies for serious conditions, FDA specified that its interpretation of the definition of regenerative medicine advanced therapy products includes gene therapies that lead to a sustained effect on cells or tissues, such as *in vivo* AAV vectors delivered to non-dividing cells. The benefits of a regenerative medicine advanced therapy designation include early interactions with FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review, and accelerated approval based on surrogate or intermediate endpoints. RMAT designation may be rescinded if a product no longer meets the qualifying criteria.

### *Accelerated approval pathway*

The FDA may grant accelerated approval to a product for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a condition when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, or IMM, and that is reasonably likely to predict an effect on IMM or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Products granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign, or other measure that is thought to predict clinical benefit but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. An intermediate clinical endpoint is a measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a product, such as an effect on IMM. The FDA has limited experience with accelerated approvals based on intermediate clinical endpoints, but has indicated that such endpoints generally may support accelerated approval where the therapeutic effect measured by the endpoint is not itself a clinical benefit and basis for traditional approval, if there is a basis for concluding that the therapeutic effect is reasonably likely to predict the ultimate clinical benefit of a product.

The accelerated approval pathway is most often used in settings in which the course of a disease is long and an extended period of time is required to measure the intended clinical benefit of a product, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Thus, accelerated approval has been used extensively in the development and approval of products for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large trials to demonstrate a clinical or survival benefit.

The accelerated approval pathway is usually contingent on a sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the product's clinical benefit. As a result, a product candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, confirm a clinical benefit during

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post-marketing studies or dissemination of false or misleading promotional materials would allow the FDA to withdraw the product from the market on an expedited basis. All promotional materials for product candidates approved under accelerated regulations are subject to prior review by the FDA.

### *Post-approval regulation*

If regulatory approval for marketing of a product or new indication for an existing product is obtained, the sponsor will be required to comply with all regular post-approval regulatory requirements as well as any post-approval requirements that the FDA have imposed as part of the approval process. The sponsor will be required to report certain adverse reactions and production problems to the FDA, provide updated safety and efficacy information and comply with requirements concerning advertising and promotional labeling requirements. Manufacturers and certain of their subcontractors are required to register their establishments with the FDA and certain state agencies and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with ongoing regulatory requirements, including cGMP regulations, which impose certain procedural and documentation requirements upon manufacturers. Accordingly, the sponsor and its third-party manufacturers must continue to expend time, money and effort in the areas of production and quality control to maintain compliance with cGMP regulations and other regulatory requirements.

A product may also be subject to official lot release, meaning that the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official lot release, the manufacturer must submit samples of each lot, together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot, to the FDA. The FDA may in addition perform certain confirmatory tests on lots of some products before releasing the lots for distribution. Finally, the FDA will conduct laboratory research related to the safety, purity, potency and effectiveness of pharmaceutical products.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending applications or supplements to approved applications, or suspension or revocation of product license approvals;
- product recall, seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

Pharmaceutical products may be promoted only for the approved indications and in accordance with the provisions of the approved label. Although healthcare providers may prescribe products for uses not described in the drug's labeling, known as off-label uses, in their professional judgment, drug manufacturers are prohibited from soliciting, encouraging or promoting unapproved uses of a product. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

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The FDA strictly regulates the marketing, labeling, advertising and promotion of prescription drug products placed on the market. This regulation includes, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities and promotional activities involving the Internet and social media. Promotional claims about a drug's safety or effectiveness are prohibited before the drug is approved. After approval, a drug product generally may not be promoted for uses that are not approved by the FDA, as reflected in the product's prescribing information. In the United States, healthcare professionals are generally permitted to prescribe drugs for such off-label uses because the FDA does not regulate the practice of medicine. However, FDA regulations impose rigorous restrictions on manufacturers' communications, prohibiting the promotion of off-label uses. It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information.

If a company is found to have promoted off-label uses, it may become subject to adverse public relations and administrative and judicial enforcement by the FDA, the DOJ, or the Office of the Inspector General of the Department of Health and Human Services, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

### *Orphan drug designation*

Orphan drug designation in the United States is designed to encourage sponsors to develop products intended for treatment of rare diseases or conditions. In the United States, a rare disease or condition is statutorily defined as a condition that affects fewer than 200,000 individuals in the United States or that affects more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available the biologic for the disease or condition will be recovered from sales of the product in the United States.

Orphan drug designation qualifies a company for tax credits and market exclusivity for seven years following the date of the product's marketing approval if granted by the FDA. An application for designation as an orphan product can be made any time prior to the filing of an application for approval to market the product. A product becomes an orphan when it receives orphan drug designation from the Office of Orphan Products Development at the FDA based on acceptable confidential requests made under the regulatory provisions. The product must then go through the review and approval process like any other product.

A sponsor may request orphan drug designation of a previously unapproved product or new orphan indication for an already marketed product. In addition, a sponsor of a product that is otherwise the same product as an already approved orphan drug may seek and obtain orphan drug designation for the subsequent product for the same rare disease or condition if it can present a plausible hypothesis that its product may be clinically superior to the first drug. More than one sponsor may receive orphan drug designation for the same product for the same rare disease or condition, but each sponsor seeking orphan drug designation must file a complete request for designation.

If a product with orphan designation receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will receive orphan drug exclusivity. Orphan drug exclusivity means that the FDA may not approve another sponsor's marketing application for the same product for the same indication for

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seven years, except in certain limited circumstances. In particular, the concept of what constitutes the “same drug” for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA has issued recent draft guidance suggesting that it would not consider two gene therapy products to be different drugs solely based on minor differences in the transgenes or vectors within a given vector class. If a product designated as an orphan drug ultimately receives marketing approval for an indication broader than what was designated in its orphan drug application, it may not be entitled to exclusivity.

The period of exclusivity begins on the date that the marketing application is approved by the FDA and applies only to the indication for which the product has been designated. The FDA may approve a second application for the same product for a different use or a second application for a clinically superior version of the product for the same use. The FDA cannot, however, approve the same product made by another manufacturer for the same indication during the market exclusivity period unless it has the consent of the sponsor or the sponsor is unable to provide sufficient quantities.

### *Pediatric exclusivity*

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if a BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity that cover the product are extended by six months.

### *Biosimilars and exclusivity*

The 2010 Patient Protection and Affordable Care Act, which was signed into law in March 2010, included a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA. The BPCIA established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars. A biosimilar is a biological product that is highly similar to an existing FDA-licensed “reference product.” As of January 1, 2020, the FDA has approved 26 biosimilar products for use in the United States. No interchangeable biosimilars, however, have been approved. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars. Additional guidances are expected to be finalized by the FDA in the near term.

Under the BPCIA, a manufacturer may submit an application for licensure of a biologic product that is “biosimilar to” or “interchangeable with” a previously approved biological product or “reference product.” In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. For the FDA to approve a biosimilar product as interchangeable with a reference product, the agency must find that the biosimilar product can be expected to produce the same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date of approval of the reference product. The FDA may not approve a biosimilar product until 12 years from the date on which the reference product was approved. Even if a product is considered to be a reference product eligible for exclusivity, another company could market a competing version of that product if

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the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law. Since the passage of the BPCIA, many states have passed laws or amendments to laws, including laws governing pharmacy practices, which are state-regulated, to regulate the use of biosimilars.

### *Federal and state data privacy and security laws*

Under the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, the U.S. Department of Health and Human Services, or HHS, has issued regulations to protect the privacy and security of protected health information, or PHI, used or disclosed by covered entities including certain healthcare providers, health plans and healthcare clearinghouses. HIPAA also regulates standardization of data content, codes and formats used in healthcare transactions and standardization of identifiers for health plans and providers. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009 or HITECH, and their regulations, including the omnibus final rule published on January 25, 2013, also imposes certain obligations on the business associates of covered entities that obtain protected health information in providing services to or on behalf of covered entities. In addition to federal privacy regulations, there are a number of state laws governing confidentiality and security of health information that are applicable to our business. In addition to possible federal civil and criminal penalties for HIPAA violations, state attorneys general are authorized to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorney's fees and costs associated with pursuing federal civil actions. Accordingly, state attorneys general (along with private plaintiffs) have brought civil actions seeking injunctions and damages resulting from alleged violations of HIPAA's privacy and security rules. New laws and regulations governing privacy and security may be adopted in the future as well.

Additionally, California recently enacted legislation that has been dubbed the first "GDPR-like" law in the United States. Known as the California Consumer Privacy Act, or CCPA, it creates new individual privacy rights for consumers (as that word is broadly defined in the law) and places increased privacy and security obligations on entities handling personal data of consumers or households. The CCPA went into effect on January 1, 2020 and requires covered companies to provide new disclosures to California consumers, provide such consumers new ways to opt-out of certain sales of personal information, and allow for a new cause of action for data breaches. The CCPA could impact our business activities depending on how it is interpreted and exemplifies the vulnerability of our business to not only cyber threats but also the evolving regulatory environment related to personal data and protected health information.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available under such laws, it is possible that some of our current or future business activities, including certain clinical research, sales and marketing practices and the provision of certain items and services to our customers, could be subject to challenge under one or more of such privacy and data security laws. The heightening compliance environment and the need to build and maintain robust and secure systems to comply with different privacy compliance and/or reporting requirements in multiple jurisdictions could increase the possibility that a healthcare company may fail to comply fully with one or more of these requirements. If our operations are found to be in violation of any of the privacy or data security laws or regulations described above that are applicable to us, or any other laws that apply to us, we may be subject to penalties, including potentially significant criminal, civil and administrative penalties, damages, fines, imprisonment, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a consent decree or similar agreement to resolve allegations of

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non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any product candidates we may develop, once approved, are sold in a foreign country, we may be subject to similar foreign laws.

### *Patent term restoration and extension*

In the United States, a patent claiming a new biologic product, its method of use or its method of manufacture may be eligible for a limited patent term extension under the Hatch-Waxman Act, which permits a patent extension of up to five years for patent term lost during product development and FDA regulatory review. Assuming grant of the patent for which the extension is sought, the restoration period for a patent covering a product is typically one-half the time between the effective date of the IND involving human beings and the submission date of the BLA, plus the time between the submission date of the BLA and the ultimate approval date. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date in the United States. Only one patent applicable to an approved product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent for which extension is sought. A patent that covers multiple products for which approval is sought can only be extended in connection with one of the approvals. The USPTO reviews and approves the application for any patent term extension in consultation with the FDA.

### *FDA approval of companion diagnostics*

In August 2014, the FDA issued final guidance clarifying the requirements that will apply to approval of therapeutic products and *in vitro* companion diagnostics. According to the guidance, for novel drugs, a companion diagnostic device and its corresponding therapeutic should be approved or cleared contemporaneously by the FDA for the use indicated in the therapeutic product's labeling. Approval or clearance of the companion diagnostic device will ensure that the device has been adequately evaluated and has adequate performance characteristics in the intended population. In July 2016, the FDA issued a draft guidance intended to assist sponsors of the drug therapeutic and *in vitro* companion diagnostic device on issues related to co-development of the products.

Under the FDCA, *in vitro* diagnostics, including companion diagnostics, are regulated as medical devices. In the United States, the FDCA and its implementing regulations, and other federal and state statutes and regulations govern, among other things, medical device design and development, preclinical and clinical testing, premarket clearance or approval, registration and listing, manufacturing, labeling, storage, advertising and promotion, sales and distribution, export and import and post-market surveillance. Unless an exemption applies, diagnostic tests require marketing clearance or approval from the FDA prior to commercial distribution.

The FDA previously has required *in vitro* companion diagnostics intended to select the patients who will respond to the product candidate to obtain pre-market approval, or PMA, simultaneously with approval of the therapeutic product candidate. The PMA process, including the gathering of clinical and preclinical data and the submission to and review by the FDA, can take several years or longer. It involves a rigorous premarket review during which the applicant must prepare and provide the FDA with reasonable assurance of the device's safety and effectiveness and information about the device and its components regarding, among other things, device design, manufacturing and labeling. PMA applications are subject to an application fee. For federal fiscal year 2020, the standard fee is \$340,995 and the small business fee is \$85,249.



## **Regulation and procedures governing approval of medicinal products in the European Union**

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of products. Whether or not it obtains FDA approval for a product, an applicant will need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. Specifically, the process governing approval of medicinal products in the European Union generally follows the same lines as in the United States. It entails satisfactory completion of preclinical studies and adequate and well-controlled clinical trials to establish the safety and efficacy of the product for each proposed indication. It also requires the submission to the relevant competent authorities of a marketing authorization application, or MAA, and granting of a marketing authorization by these authorities before the product can be marketed and sold in the European Union.

### *Clinical trial approval*

Pursuant to the currently applicable Clinical Trials Directive 2001/20/EC and the Directive 2005/28/EC on GCP, a system for the approval of clinical trials in the European Union has been implemented through national legislation of the member states. Under this system, an applicant must obtain approval from the competent national authority of a European Union member state in which the clinical trial is to be conducted, or in multiple member states if the clinical trial is to be conducted in a number of member states. Furthermore, the applicant may only start a clinical trial at a specific site after the competent ethics committee has issued a favorable opinion. The clinical trial application must be accompanied by an investigational medicinal product dossier with supporting information prescribed by Directive 2001/20/EC and Directive 2005/28/EC and corresponding national laws of the member states and further detailed in applicable guidance documents.

In April 2014, the European Union adopted a new Clinical Trials Regulation (EU) No 536/2014, but it has not yet become effective. It will overhaul the current system of approvals for clinical trials in the European Union. Specifically, the new legislation, which will be directly applicable in all member states, aims at simplifying and streamlining the approval of clinical trials in the European Union. For instance, the new Clinical Trials Regulation provides for a streamlined application procedure via a single-entry point and strictly defined deadlines for the assessment of clinical trial applications. As of January 1, 2020, the website of the European Commission reported that the implementation of the new Clinical Trials Regulation was dependent on the development of a fully functional clinical trials portal and database, which would be confirmed by an independent audit, and that the new legislation would come into effect six months after the European Commission publishes a notice of this confirmation. The website indicated that the audit was expected to commence in December 2020.

Parties conducting certain clinical trials must, as in the United States, post clinical trial information in the European Union at the EudraCT website: <https://eudract.ema.europa.eu>.

### *PRIME designation in the European Union*

In March 2016, the EMA launched an initiative to facilitate development of product candidates in indications, often rare, for which few or no therapies currently exist. The PRiority MEDicines, or PRIME, scheme is intended to encourage drug development in areas of unmet medical need and provides accelerated assessment of products representing substantial innovation reviewed under the centralized procedure. Products from small- and medium-sized enterprises may qualify for earlier entry into the PRIME scheme than larger companies. Many benefits accrue to sponsors of product candidates with PRIME designation, including but not limited to,

early and proactive regulatory dialogue with the EMA, frequent discussions on clinical trial designs and other development program elements, and accelerated marketing authorization application assessment once a dossier has been submitted. Importantly, a dedicated EMA contact and rapporteur from the Committee for Human Medicinal Products, or CHMP, or Committee for Advanced Therapies are appointed early in the PRIME scheme facilitating increased understanding of the product at the EMA's Committee level. A kick-off meeting initiates these relationships and includes a team of multidisciplinary experts at the EMA to provide guidance on the overall development and regulatory strategies.

#### *Marketing authorization*

To obtain a marketing authorization for a product under the European Union regulatory system, an applicant must submit an MAA, either under a centralized procedure administered by the EMA or one of the procedures administered by competent authorities in European Union Member States (decentralized procedure, national procedure, or mutual recognition procedure). A marketing authorization may be granted only to an applicant established in the European Union. Regulation (EC) No 1901/2006 provides that prior to obtaining a marketing authorization in the European Union, an applicant must demonstrate compliance with all measures included in an EMA-approved Pediatric Investigation Plan, or PIP, covering all subsets of the pediatric population, unless the EMA has granted a product-specific waiver, class waiver or a deferral for one or more of the measures included in the PIP.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid for all European Union member states. Pursuant to Regulation (EC) No. 726/2004, the centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy products and products with a new active substance indicated for the treatment of certain diseases, including products for the treatment of cancer. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, the centralized procedure may be optional. Manufacturers must demonstrate the quality, safety and efficacy of their products to the EMA, which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by the EMA.

Specifically, the grant of marketing authorization in the European Union for products containing viable human tissues or cells such as gene therapy medicinal products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC lays down specific rules concerning the authorization, supervision and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety and efficacy of their products to EMA which provides an opinion regarding the application for marketing authorization. The European Commission grants or refuses marketing authorization in light of the opinion delivered by EMA.

Under the centralized procedure, the CHMP established at the EMA is responsible for conducting an initial assessment of a product. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated evaluation may be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and, in particular, from the viewpoint of therapeutic innovation. If the CHMP accepts such a request, the time limit of 210 days will be reduced to 150 days, but it is possible that the CHMP may revert to the standard time limit for the centralized procedure if it determines that it is no longer appropriate to conduct an accelerated assessment.

*Specialized procedures for gene therapies*

The grant of marketing authorization in the European Union for gene therapy products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC includes specific rules concerning the authorization, supervision and pharmacovigilance of gene therapy medicinal products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety and efficacy of their products to the EMA, which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by the EMA.

*Regulatory data protection in the European Union*

In the European Union, new chemical entities approved on the basis of a complete independent data package qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity pursuant to Regulation (EC) No 726/2004, as amended, and Directive 2001/83/EC, as amended. Data exclusivity prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic (abbreviated) application for a period of eight years. During the additional two-year period of market exclusivity, a generic marketing authorization application can be submitted, and the innovator's data may be referenced, but no generic medicinal product can be marketed until the expiration of the market exclusivity. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to authorization, is held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity so that the innovator gains the prescribed period of data exclusivity, another company may market another version of the product if such company obtained marketing authorization based on an MAA with a complete independent data package of pharmaceutical tests, preclinical tests and clinical trials.

*Patent term extensions in the European Union and other jurisdictions*

The European Union also provides for patent term extension through Supplementary Protection Certificates, or SPCs. The rules and requirements for obtaining a SPC are similar to those in the United States. An SPC may extend the term of a patent for up to five years after its originally scheduled expiration date and can provide up to a maximum of fifteen years of marketing exclusivity for a drug. In certain circumstances, these periods may be extended for six additional months if pediatric exclusivity is obtained, which is described in detail below. Although SPCs are available throughout the European Union, sponsors must apply on a country-by-country basis. Similar patent term extension rights exist in certain other foreign jurisdictions outside the European Union.

*Periods of authorization and renewals*

A marketing authorization is valid for five years, in principle, and it may be renewed after five years on the basis of a reevaluation of the risk-benefit balance by the EMA or by the competent authority of the authorizing member state. To that end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing authorization was granted, at least six months before the marketing authorization ceases to be valid. Once renewed, the marketing authorization is valid for an unlimited period, unless the European Commission or the competent authority decides, on justified grounds relating to pharmacovigilance, to proceed with one additional five-year renewal period. Any authorization that is not

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followed by the placement of the drug on the European Union market (in the case of the centralized procedure) or on the market of the authorizing member state within three years after authorization ceases to be valid.

### *Regulatory requirements after marketing authorization*

Following approval, the holder of the marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of the medicinal product. These include compliance with the European Union's stringent pharmacovigilance or safety reporting rules, pursuant to which post-authorization studies and additional monitoring obligations can be imposed. In addition, the manufacturing of authorized products, for which a separate manufacturer's license is mandatory, must also be conducted in strict compliance with the EMA's GMP requirements and comparable requirements of other regulatory bodies in the European Union, which mandate the methods, facilities and controls used in manufacturing, processing and packing of drugs to assure their safety and identity. Finally, the marketing and promotion of authorized products, including industry-sponsored continuing medical education and advertising directed toward the prescribers of drugs and/or the general public, are strictly regulated in the European Union under Directive 2001/83EC, as amended.

### *Orphan drug designation and exclusivity*

Regulation (EC) No 141/2000 and Regulation (EC) No. 847/2000 provide that a product can be designated as an orphan drug by the European Commission if its sponsor can establish: that the product is intended for the diagnosis, prevention or treatment of (1) a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the European Union when the application is made, or (2) a life-threatening, seriously debilitating or serious and chronic condition in the European Union and that without incentives it is unlikely that the marketing of the drug in the European Union would generate sufficient return to justify the necessary investment. For either of these conditions, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Union or, if such method exists, the drug will be of significant benefit to those affected by that condition.

An orphan drug designation provides a number of benefits, including fee reductions, regulatory assistance and the possibility to apply for a centralized European Union marketing authorization. Marketing authorization for an orphan drug leads to a ten-year period of market exclusivity. During this market exclusivity period, neither the EMA nor the European Commission or the member states can accept an application or grant a marketing authorization for a "similar medicinal product." A "similar medicinal product" is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The market exclusivity period for the authorized therapeutic indication may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan drug designation because, for example, the product is sufficiently profitable not to justify market exclusivity.

### *Brexit and the regulatory framework in the United Kingdom*

On June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the European Union on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020, which is extendable up to two years. Discussions between the United Kingdom and the European Union have so far mainly focused on finalizing withdrawal issues and transition agreements but have been extremely difficult to date. To date, only an outline of a trade agreement has been reached. Much remains open but the Prime

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Minister has indicated that the United Kingdom will not seek to extend the transitional period beyond the end of 2020. If no trade agreement has been reached before the end of the transitional period, there may be significant market and economic disruption. The Prime Minister has also indicated that the UK will not accept high regulatory alignment with the European Union.

Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for any product candidates we may develop, which could significantly and materially harm our business.

Furthermore, while the Data Protection Act of 2018 in the United Kingdom that “implements” and complements the EU General Data Protection Regulation, or GDPR, has achieved Royal Assent on May 23, 2018 and is now effective in the United Kingdom, it is still unclear whether transfer of data from the European Economic Area, or EEA, to the United Kingdom will remain lawful under GDPR. During the period of “transition” (i.e., until December 31, 2020), EU law will continue to apply in the UK, including the GDPR, after which the GDPR will be converted into UK law. Beginning in 2021, the UK will be a “third country” under the GDPR. We may, however, incur liabilities, expenses, costs and other operational losses under GDPR and applicable European Union Member States and the United Kingdom privacy laws in connection with any measures we take to comply with them.

### *General Data Protection Regulation*

The collection, use, disclosure, transfer or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States, and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR will be a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance.

### **Coverage, pricing and reimbursement**

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we may seek regulatory approval by the FDA or other government authorities. In the United States and markets in other countries, patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payers to reimburse all or part of the associated healthcare costs. Patients are unlikely to use any product candidates we may develop unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of such product candidates. Even if any

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product candidates we may develop are approved, sales of such product candidates will depend, in part, on the extent to which third-party payers, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations, provide coverage and establish adequate reimbursement levels for, such product candidates. The process for determining whether a payer will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payer will pay for the product once coverage is approved. Third-party payers are increasingly challenging the prices charged, examining the medical necessity, and reviewing the cost-effectiveness of medical products and services and imposing controls to manage costs. Third-party payers may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the approved products for a particular indication.

In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA or other comparable marketing approvals. Nonetheless, product candidates may not be considered medically necessary or cost effective. A decision by a third-party payer not to cover any product candidates we may develop could reduce physician utilization of such product candidates once approved and have a material adverse effect on our sales, results of operations and financial condition. Additionally, a payer's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payer's determination to provide coverage for a product does not assure that other payers will also provide coverage and reimbursement for the product, and the level of coverage and reimbursement can differ significantly from payer to payer. Third-party reimbursement and coverage may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development. In addition, any companion diagnostic tests require coverage and reimbursement separate and apart from the coverage and reimbursement for their companion pharmaceutical or biological products. Similar challenges to obtaining coverage and reimbursement, applicable to pharmaceutical or biological products, will apply to any companion diagnostics.

The containment of healthcare costs also has become a priority of federal, state and foreign governments and the prices of pharmaceuticals have been a focus in this effort. Governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit a company's revenue generated from the sale of any approved products. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which a company or its collaborators receive marketing approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

If we obtain approval in the future to market in the United States any product candidates we may develop, we may be required to provide discounts or rebates under government healthcare programs or to certain government and private purchasers in order to obtain coverage under federal healthcare programs such as Medicaid. Participation in such programs may require us to track and report certain drug prices. We may be subject to fines and other penalties if we fail to report such prices accurately.

Outside the United States, ensuring adequate coverage and payment for any product candidates we may develop will face challenges. Pricing of prescription pharmaceuticals is subject to governmental control in many countries. Pricing negotiations with governmental authorities can extend well beyond the receipt of regulatory marketing approval for a product and may require us to conduct a clinical trial that compares the cost effectiveness of any product candidates we may develop to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our commercialization efforts.

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In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies (so called health technology assessments) in order to obtain reimbursement or pricing approval. For example, the European Union provides options for its member states to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other member states allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the European Union have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage healthcare expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the European Union. The downward pressure on healthcare costs in general, particularly prescription products, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states, and parallel trade (arbitrage between low-priced and high-priced member states), can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products, if approved in those countries.

### ***Healthcare law and regulation***

Healthcare providers and third-party payers play a primary role in the recommendation and prescription of pharmaceutical products that are granted marketing approval. Arrangements with providers, consultants, third-party payers and customers are subject to broadly applicable fraud and abuse, anti-kickback, false claims laws, reporting of payments to physicians and teaching physicians and patient privacy laws and regulations and other healthcare laws and regulations that may constrain our business and/or financial arrangements. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the U.S. federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the civil False Claims Act and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false, fictitious or fraudulent or knowingly making, using or causing to be made or used a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal civil monetary penalty and false statement laws and regulations relating to pricing and submission of pricing information for government programs, including penalties for knowingly and intentionally overcharging 340b eligible entities and the submission of false or fraudulent pricing information to government entities;

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- HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by HITECH, and their respective implementing regulations, including the Final Omnibus Rule published in January 2013, which impose obligations, including mandatory contractual terms, on certain covered healthcare providers, health plans and healthcare clearinghouses, as well as their respective business associates that perform services for them, that involve the use, or disclosure of, individually identifiable health information, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the Foreign Corrupt Practices Act, which prohibits companies and their intermediaries from making, or offering or promising to make improper payments to non-U.S. officials for the purpose of obtaining or retaining business or otherwise seeking favorable treatment;
- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the Patient Protection and Affordable Care Act, or PPACA, as amended by the Health Care Education Reconciliation Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare & Medicaid Services, or CMS, within the U.S. Department of Health and Human Services, information related to payments and other transfers of value made by that entity to physicians, as defined by such law, and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payers, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring pharmaceutical manufacturers to report information related to payments to physicians and other healthcare providers or marketing expenditures. In addition, certain state and local laws require drug manufacturers to register pharmaceutical sales representatives. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, disgorgement, exclusion from government funded healthcare programs, such as Medicare and Medicaid, integrity oversight and reporting obligations and the curtailment or restructuring of our operations.

### *Healthcare reform*

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. There have been a number of federal and state proposals during the last few years regarding the pricing of pharmaceutical and biopharmaceutical products, limiting coverage and reimbursement for drugs and other medical products, government control and other changes to the healthcare system in the United States.



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By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the PPACA, which, among other things, includes changes to the coverage and payment for products under government healthcare programs. Among the provisions of the PPACA of importance to our potential product candidates are:

- an annual, nondeductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs, although this fee would not apply to sales of certain products approved exclusively for orphan indications;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to certain individuals with income at or below 133% of the federal poverty level, thereby potentially increasing a manufacturer's Medicaid rebate liability;
- expanded manufacturers' rebate liability under the Medicaid Drug Rebate Program by increasing the minimum rebate for both branded and generic drugs and revising the definition of "average manufacturer price" for calculating and reporting Medicaid drug rebates on outpatient prescription drug prices and extending rebate liability to prescriptions for individuals enrolled in Medicare Advantage plans;
- addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for products that are inhaled, infused, instilled, implanted or injected;
- expanded the types of entities eligible for the 340B drug discount program;
- established the Medicare Part D coverage gap discount program by requiring manufacturers to provide a 50% point-of-sale-discount off the negotiated price of applicable products to eligible beneficiaries during their coverage gap period as a condition for the manufacturers' outpatient products to be covered under Medicare Part D;
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research;
- the Independent Payment Advisory Board, or IPAB, which has authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription products. However, the IPAB implementation has been not been clearly defined. The PPACA provided that under certain circumstances, IPAB recommendations will become law unless Congress enacts legislation that will achieve the same or greater Medicare cost savings; and
- established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription product spending. Funding has been allocated to support the mission of the Center for Medicare and Medicaid Innovation from 2011 to 2019.

Other legislative changes have been proposed and adopted in the United States since the PPACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of up to two percent per fiscal year, which went into effect in April 2013 and will remain in effect through 2029 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, which was enacted in January 2013, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers and

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increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Since enactment of the PPACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by President Trump on December 22, 2017, Congress repealed the “individual mandate.” The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, will become effective in 2019. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the PPACA-mandated “Cadillac” tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Further, the Bipartisan Budget Act of 2018, among other things, amended the PPACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.” The Congress may consider other legislation to replace elements of the PPACA during the next Congressional session.

The Trump Administration has also taken executive actions to undermine or delay implementation of the PPACA. Since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the PPACA or otherwise circumvent some of the requirements for health insurance mandated by the PPACA. One Executive Order directs federal agencies with authorities and responsibilities under the PPACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the PPACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers or manufacturers of pharmaceuticals or medical devices. The second Executive Order terminates the cost-sharing subsidies that reimburse insurers under the PPACA. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the PPACA for plans sold through such marketplaces. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than \$12 billion in PPACA risk corridor payments to third-party payers who argued were owed to them. This decision is under review by the U.S. Supreme Court during its current term. The full effects of this gap in reimbursement on third-party payers, the viability of the PPACA marketplace, providers and potentially our business, are not yet known.

In addition, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the PPACA is an essential and inseparable feature of the PPACA, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the PPACA are invalid as well. The Trump administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the same judge issued an order staying the judgment pending appeal. The Trump Administration recently represented to the Court of Appeals considering this judgment that it does not oppose the lower court’s ruling. On July 10, 2019, the Court of Appeals for the Fifth Circuit heard oral argument in this case. On December 18, 2019, that court affirmed the lower court’s ruling that the individual mandate portion of the PPACA is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the PPACA. On March 2, 2020, the United States Supreme Court granted the petitions for writs of certiorari to review this case, and has allotted one hour for oral arguments, which are expected to occur in the fall. Litigation and legislation over the PPACA are likely to continue, with unpredictable and uncertain results.

Further, there have been several recent U.S. congressional inquiries and proposed federal and proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the

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relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. For example, there have been several recent U.S. congressional inquiries and proposed federal and proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. For example, on May 11, 2018, the Administration issued a plan to lower drug prices. Under this blueprint for action, the Administration indicated that the Department of Health and Human Services will take steps to end the gaming of regulatory and patent processes by drug makers to unfairly protect monopolies; advance biosimilars and generics to boost price competition; evaluate the inclusion of prices in drug makers' ads to enhance price competition; speed access to and lower the cost of new drugs by clarifying policies for sharing information between insurers and drug makers; avoid excessive pricing by relying more on value-based pricing by expanding outcome-based payments in Medicare and Medicaid; work to give Part D plan sponsors more negotiation power with drug makers; examine which Medicare Part B drugs could be negotiated for a lower price by Part D plans, and improving the design of the Part B Competitive Acquisition Program; update Medicare's drug-pricing dashboard to increase transparency; prohibit Part D contracts that include "gag rules" that prevent pharmacists from informing patients when they could pay less out-of-pocket by not using insurance; and require that Part D plan members be provided with an annual statement of plan payments, out-of-pocket spending and drug price increases. On March 10, 2020, the current presidential administration sent "principles" for drug pricing to Congress, calling for legislation that would, among other things, cap Medicare Part D beneficiary out-of-pocket pharmacy expenses, provide an option to cap Medicare Part D beneficiary monthly out-of-pocket expenses, and place limits on pharmaceutical price increases. In addition, on December 23, 2019, the Trump Administration published a proposed rulemaking that, if finalized, would allow states or certain other non-federal government entities to submit importation program proposals to FDA for review and approval. Applicants would be required to demonstrate their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. At the same time, FDA issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional healthcare authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other healthcare programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for any product candidates we may develop or additional pricing pressures.

There have been, and likely will continue to be, additional legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. Such reforms could have an adverse effect on anticipated revenues from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop product candidates.

## **Employees**

As of March 31, 2020, we had 85 full-time employees, including a total of 32 employees with M.D. or Ph.D. degrees. Of these full-time employees, 64 employees are engaged in research and development. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

## **Facilities**

Our principal facilities consist of office and laboratory space. We occupy approximately 71,562 square feet of office space in Cambridge, Massachusetts under a lease that currently expires on April 30, 2029.

## **Legal proceedings**

We are not currently subject to any material legal proceedings.

# Management

## Executive officers and directors

The following table sets forth the name, age and position of each of our executive officers and directors as of March 31, 2020.

| Name                              | Age | Position  |
|-----------------------------------|-----|---|
| <i>Executive Officers</i>         |     |   |
| Geoff McDonough, M.D.             | 49  | President and Chief Executive Officer, Director |
| Matthew Stanton, Ph.D.            | 47  | Chief Scientific Officer                        |
| Mark Angelino, Ph.D.              | 47  | Chief Operating Officer                         |
| Douglas Kerr, M.D., Ph.D., M.B.A. | 53  | Chief Development Officer                       |
| <i>Non-Employee Directors</i>     |     |   |
| Jason Rhodes                      | 50  | Chairman of the Board of Directors              |
| Catherine Stehman-Breen, M.D.     | 57  | Director  |
| Gustav Christensen                | 72  | Director  |
| Jeffrey Jonas, M.D.               | 67  | Director  |
| Donald Nicholson, Ph.D.           | 62  | Director  |
| Charles Rowland                   | 61  | Director  |
| Anthony Quinn, M.B. Ch.B., Ph.D.  | 58  | Director  |

(1) Member of the Audit Committee.

(2) Member of the Talent Committee.

(3) Member of the Nominating and Corporate Governance Committee.

### Executive officers

**Geoff McDonough, M.D.** has served as our president and chief executive officer and as a member of our board of directors since October 2017. Previously, Dr. McDonough served as president and chief executive officer of Swedish Orphan Biovitrum AB, or Sobi, a biopharmaceutical company, from August 2011 until May 2017. Dr. McDonough serves on the boards of directors of Surface Oncology, Inc., a biotechnology company, and Zafgen, Inc., a biopharmaceutical company. Dr. McDonough earned a Bachelor of Science in biology and a Bachelor of Arts in philosophy, both summa cum laude, from University of North Carolina at Chapel Hill. Dr. McDonough earned his M.D. at Harvard Medical School and completed his residency training in internal medicine and pediatrics at Massachusetts General Hospital and Boston Children's Hospital. We believe that Dr. McDonough's extensive leadership experience in the life sciences industry and his extensive knowledge of our company based on his role as our president and chief executive officer qualify him to serve as a member of our board of directors.

**Matthew Stanton, Ph.D.** has served as our chief scientific officer since May 2019. Previously, Dr. Stanton served as our chief technology officer from October 2017 to May 2019. Prior to joining us, Dr. Stanton served as vice president, head of chemistry and platform immunology at Moderna, Inc., a biotechnology company, from April 2014 until September 2017. Dr. Stanton earned a Bachelor of Science in chemistry from Virginia Tech, a Master of Science in chemistry and a doctorate in chemistry with a focus on physical organic chemistry and natural product synthesis from the University of North Carolina at Chapel Hill.

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**Mark Angelino, Ph.D.** co-founded our company and has served as our chief operating officer since our inception in October 2016. Prior to co-founding our company, Dr. Angelino was senior vice president of development and manufacturing at bluebird bio Inc., a biotechnology company, from April 2012 until October 2016. Dr. Angelino earned a Bachelor of Science in chemical engineering from The Cooper Union, and both a M.Sc. in chemical engineering practice and a doctorate in chemical engineering from the Massachusetts Institute of Technology.

**Douglas Kerr, M.D., Ph.D., M.B.A.** has served as our chief development officer since May 2019. Previously, Dr. Kerr served as our chief scientific officer and executive vice president of research and development from August 2017 to May 2019. Prior to joining us, Dr. Kerr served as global development team lead and vice president for neurology at Shire plc, a biopharmaceutical company, from October 2015 to August 2017. Dr. Kerr earned a B.A. degree in biochemistry from Princeton University and, an M.B.A. with a specialization in entrepreneurship and finance from Northeastern University and an M.D. from Jefferson Medical College, as well as a doctorate in biochemistry and molecular biology.

### **Non-employee directors**

**Jason Rhodes** has served as the chairman of our board of directors since October 2017 and served as our founding chief executive officer from October 2016 to October 2017. Mr. Rhodes has been a partner at Atlas Venture since 2014. From 2010 to 2014, Mr. Rhodes was at Epizyme, Inc., a biotechnology company, where he most recently served as President and Chief Financial Officer. Mr. Rhodes has been a member of the board of directors of Replimune Group, Inc. since September 2015. Mr. Rhodes earned a B.A. from Yale University and an M.B.A. from the Wharton School of the University of Pennsylvania. We believe Mr. Rhodes is qualified to serve on our Board based on his extensive knowledge of our company based on his role as the founding chief executive officer, as well as his extensive leadership experience, his biotechnology company board experience and his experience investing in life science companies.

**Catherine Stehman-Breen, M.D.** has served as a member of our board of directors since December 2017. Dr. Stehman-Breen has served as chief development officer of Obsidian Therapeutics, Inc., a biotechnology company, since July 2019. Previously, she served as an entrepreneur-in-residence at Atlas Ventures, serving as chief medical officer of both Dyne Therapeutics, Inc., a biotechnology company, from March 2018 to July 2019 and Disarm Therapeutics, Inc., a biotechnology company, from April 2018 to July 2019. Dr. Stehman-Breen also served as chief medical officer of Sarepta Therapeutics, Inc. from April 2017 to December 2017. Prior to that, Dr. Stehman-Breen served as vice president, clinical development and regulatory affairs at Regeneron Pharmaceuticals, Inc., a biotechnology company, initially as head, pain therapeutic area, and subsequently as head, clinical project management and operations from January 2015 to March 2017. Dr. Stehman-Breen earned a B.A. in biology and psychology from Colby College, a M.Sc. degree in epidemiology from the University of Washington, where she also conducted her residency and fellowship training, and a M.D. from the University of Chicago. We believe Dr. Stehman-Breen is qualified to serve on our Board based on her extensive leadership experience, her experience with clinical development and regulatory matters, and in the life science industry.

**Gustav Christensen** has served as a member of our board of directors since December 2017. Mr. Christensen has served as the chairman of the board of directors of MorpHC Holding, Inc., since April 2016. Previously, Mr. Christensen served as the president and chief executive officer and director at Dyax Corp., a biopharmaceutical company, from April 2007 to February 2016. Mr. Christensen earned a M.Sc. in Economics from the University of Aarhus (Denmark) and his M.B.A. from Harvard Business School. We believe that Mr. Christensen is qualified to serve on our board of directors due to his extensive leadership and business experience in the life sciences industry and in the commercialization of pharmaceutical products.

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**Jeffrey Jonas, M.D.** has served as a member of our board of directors since May 2018. Dr. Jonas has served as the chief executive officer, president and a member of the board of directors of Sage Therapeutics, Inc., a biopharmaceutical company, since August 2013. Dr. Jonas has served on the board of directors of Karuna Pharmaceuticals, Inc. since October 2018. Dr. Jonas earned his B.A. from Amherst College and M.D. from Harvard Medical School. He completed a residency in psychiatry at Harvard Medical School, and he served as Chief Resident in psychopharmacology at McLean Hospital, Harvard Medical School. We believe Dr. Jonas' is qualified to serve on our Board based on his extensive leadership experience, his experience with clinical development and regulatory matters, and in the life science industry.

**Donald Nicholson, Ph.D.** has served as a member of our board of directors since December 2017. Previously, Dr. Nicholson served as chief executive officer of Nimbus Therapeutics, Inc., a biotechnology company, from August 2014 to October 2018. Prior to that, Dr. Nicholson spent 25 years at Merck & Co., Inc., in various leadership, strategic and operational roles. Dr. Nicholson earned his B.S. with honors in biochemistry and his doctorate in biochemistry from the University of Western Ontario and trained as a Medical Research Council postdoctoral fellow at the University of Munich in Germany. We believe Dr. Nicholson's extensive experience in both scientific and management roles in the life sciences industry qualifies him to serve on our board of directors.

**Charles Rowland** has served as a member of our board of directors since July 2018. Mr. Rowland served as chief executive officer and director of Aurinia Pharmaceuticals Inc., a biopharmaceutical company, from January 2014 to February 2017. Mr. Rowland currently serves as a member of the boards of directors of Orchard Therapeutics plc, since June 2018, Viking Therapeutics, Inc., since July 2016, Blueprint Medicines Corporation, since March 2015, and Nabriva Therapeutics plc, since January 2015. Mr. Rowland earned his B.S. from Saint Joseph's University and M.B.A. from Rutgers University. We believe that Mr. Rowland is qualified to serve as a director due to his extensive experience in pharmaceutical operations and as well as in finance and accounting.

**Anthony Quinn, M.B. Ch.B., Ph.D.** has served as a member of our board of directors since December 2017. Dr. Quinn has served as president and chief executive officer and as a director of Aeglea BioTherapeutics, Inc., a biotechnology company since July 2017. Prior to that, from October 2015 to July 2017 he worked as a private consultant for IDBioPharm Consulting LLC, a consulting firm. From August 2009 to June 2015, Dr. Quinn served in several roles at Synageva BioPharma Corp., a biotechnology company that was acquired by Alexion Pharmaceuticals, Inc. in June 2015, including most recently as head of research & development and chief medical officer. Following the acquisition, Dr. Quinn worked for Alexion Pharmaceuticals, Inc., a pharmaceutical company, from June 2015 to September 2015. Dr. Quinn has also served on the board of directors of Kaleido BioSciences, Inc., since February 2016. Dr. Quinn received his Bachelor of Medical Sciences in general pathology, his MB ChB (M.D.) from the University of Dundee, UK. and his Ph.D. in cancer research from the University of Newcastle in Tyne, UK. We believe Dr. Quinn is qualified to serve on our board of directors because of his medical and clinical experience in the biopharmaceutical industry, including experience in the development of therapeutics for rare diseases.

### **Board composition and election of directors**

#### *Board composition*

Our board of directors currently consists of eight members. Our directors hold office until their successors have been elected and qualified or until the earlier of their death, resignation or removal.

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Our certificate of incorporation and bylaws that will become effective upon the closing of this offering provide that the authorized number of directors may be changed only by resolution of our board of directors. Our certificate of incorporation and bylaws will also provide that our directors may be removed only for cause by the affirmative vote of the holders of 75% of our shares of capital stock present in person or by proxy and entitled to vote, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

In accordance with the terms of our certificate of incorporation and bylaws that will become effective upon the closing of this offering, our board of directors will be divided into three classes, class I, class II and class III, with members of each class serving staggered three-year terms. Upon the closing of this offering, the members of the classes will be divided as follows:

- the class I directors will be \_\_\_\_\_, \_\_\_\_\_ and \_\_\_\_\_, and their term will expire at the annual meeting of stockholders to be held in 2021;
- the class II directors will be \_\_\_\_\_, \_\_\_\_\_ and \_\_\_\_\_, and their term will expire at the annual meeting of stockholders to be held in 2022; and
- the class III directors will be \_\_\_\_\_ and \_\_\_\_\_, and their term will expire at the annual meeting of stockholders to be held in 2023.

Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires.

The classification of our board of directors may have the effect of delaying or preventing changes in our control or management. See “Description of capital stock—Delaware anti-takeover law and certain charter and bylaw provisions.”

### *Director independence*

Applicable Nasdaq rules require a majority of a listed company’s board of directors to be comprised of independent directors within one year of listing. In addition, Nasdaq rules require that, subject to specified exceptions, each member of a listed company’s audit, compensation and nominating and corporate governance committees be independent under the Securities Exchange Act of 1934, as amended, or the Exchange Act. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act. Under applicable Nasdaq rules, a director will only qualify as an “independent director” if, in the opinion of the listed company’s board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director’s ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: (1) the source of compensation of the director, including any consulting advisory or other compensatory fee paid by such company to the director; and (2) whether the director is affiliated with the company or any of its subsidiaries or affiliates.



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In 2020, our board of directors undertook a review of the composition of our board of directors and its committees and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of our directors, with the exception of Dr. McDonough, is an “independent director” as defined under applicable Nasdaq rules, including, in the case of all the members of our audit committee, the independence criteria set forth in Rule 10A-3 under the Exchange Act, and in the case of all the members of our talent committee, which serves as our compensation committee, the independence criteria set forth in Rule 10C-1 under the Exchange Act. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each non-employee director. Dr. McDonough is not an independent director under these rules because he is our president and chief executive officer.

There are no family relationships among any of our directors or executive officers.

### ***Role of the board in risk oversight***

One of the key functions of our board of directors is informed oversight of our risk management process. Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure and our audit committee has the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee also monitors compliance with legal and regulatory requirements.

### ***Board committees***

Our board of directors has established an audit committee, a talent committee and a nominating and corporate governance committee, each of which operates under a charter that has been approved by our board. The composition of each committee will be effective as of the date of this prospectus.

#### *Audit committee*

The members of our audit committee are \_\_\_\_\_, \_\_\_\_\_ and \_\_\_\_\_, and \_\_\_\_\_ is the chair of the audit committee. Effective at the time of this offering, our audit committee's responsibilities will include:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from such firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics;
- overseeing our internal audit function;

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- overseeing our risk assessment and risk management policies;
- establishing policies regarding hiring employees from our independent registered public accounting firm and procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our internal auditing staff, if any, our independent registered public accounting firm and management;
- reviewing and approving or ratifying any related person transactions; and
- preparing the audit committee report required by Securities and Exchange Commission, or SEC, rules.

All audit and non-audit services, other than *de minimis* non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

Our board of directors has determined that \_\_\_\_\_ is an “audit committee financial expert” as defined by applicable SEC rules and that each of the members of our audit committee possesses the financial sophistication required for audit committee members under Nasdaq rules. We believe that the composition of our audit committee will meet the requirements for independence under current Nasdaq and SEC rules and regulations.

### *Talent committee*

The members of our talent committee are \_\_\_\_\_, \_\_\_\_\_ and \_\_\_\_\_, and \_\_\_\_\_ is the chair of the talent committee. The talent committee serves as the compensation committee of our board. Effective at the time of this offering, our talent committee's responsibilities will include:

- reviewing and approving, or making recommendations to our board of directors with respect to, the compensation of our chief executive officer and our other executive officers;
- overseeing an evaluation of our senior executives;
- overseeing and administering our cash and equity incentive plans;
- reviewing and making recommendations to our board of directors with respect to director compensation;
- reviewing and discussing annually with management our “Compensation discussion and analysis” disclosure if and to the extent required by SEC rules; and
- preparing the compensation committee report if and to the extent then required by SEC rules.

We believe that the composition of our talent committee will meet the requirements for independence under current Nasdaq and SEC rules and regulations.

### *Nominating and corporate governance committee*

The members of our nominating and corporate governance committee are \_\_\_\_\_, \_\_\_\_\_ and \_\_\_\_\_, and \_\_\_\_\_ is the chair of the nominating and corporate governance committee. Effective at the time of this offering, our nominating and corporate governance committee's responsibilities will include:

- recommending to our board of directors the persons to be nominated for election as directors and to each of our board's committees;
- reviewing and making recommendations to our board with respect to our board leadership structure;

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- reviewing and making recommendations to our board with respect to management succession planning;
- developing and recommending to our board of directors corporate governance principles; and
- overseeing a periodic evaluation of our board of directors.

We believe that the composition of our nominating and corporate governance committee will meet the requirements for independence under current Nasdaq and SEC rules and regulations.

### ***Compensation committee interlocks and insider participation***

No member of our talent committee is or has been a current or former officer or employee of our company. None of our executive officers serves, or in the past year has served, as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any other entity that has one or more of its executive officers serving as a member of our board of directors or our talent committee.

### ***Code of ethics and code of conduct***

We intend to adopt, upon the effectiveness of the registration statement of which this prospectus is a part, a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller or persons performing similar functions. We intend to post a current copy of the code on our website, [www.generationbio.com](http://www.generationbio.com). In addition, we intend to post on our website all disclosures that are required by law or Nasdaq listing standards concerning any amendments to, or waivers from, any provision of the code.

## Executive compensation

The following discussion relates to the compensation of our president and chief executive officer, Geoff McDonough, our chief development officer, Douglas Kerr, and our former chief financial officer, Thomas Graney, for the fiscal year ended December 31, 2019. Dr. McDonough, Dr. Kerr and Mr. Graney are collectively referred to in this prospectus as our named executive officers.

In preparing to become a public company, we have begun a thorough review of all elements of our executive compensation program, including the function and design of our equity incentive programs. We have begun, and expect to continue in the coming months, to evaluate the need for revisions to our executive compensation program to ensure that our program is competitive with the companies with which we compete for executive talent and is appropriate for a public company.

### Summary compensation table

The following table sets forth information regarding compensation awarded to, earned by or paid to each of our named executive officers during our fiscal year ended December 31, 2019.

| Name and Principal Position   | Year | Salary(\$) | Bonus(\$)<br>(1) | Option<br>awards(\$)<br>(2) | Total(\$) |
|---|------|------------|------------------|-----------------------------|-----------|
| Geoff McDonough<br><i>President and Chief<br/>Executive Officer</i> | 2019 | 423,500    | 169,400          | —                           | 592,900   |
| Douglas Kerr<br><i>Chief Development Officer</i>                    | 2019 | 396,069    | 138,624          | —                           | 534,693   |
| Thomas Graney<br><i>Former Chief Financial Officer</i>              | 2019 | 313,060(3) | 112,551          | 1,853,243                   | 2,278,854 |

- (1) The amounts reported in the "Bonus" column reflect discretionary annual cash bonuses earned by each of our named executive officers for their performance, as determined by the board of directors in its sole discretion.
- (2) The amounts reported in the "Option awards" column reflect the aggregate grant-date fair value of stock options awarded in 2019, calculated in accordance with the provisions of the Financial Accounting Standard Board Accounting Standards Codification Topic 718. See Note 8 to our consolidated financial statements appearing elsewhere in this prospectus regarding assumptions underlying the valuation of equity awards. These amounts reflect the accounting cost for these stock options and do not reflect the actual economic value that may be realized by the named executive officer upon the vesting of the stock options, the exercise of the stock options, or the sale of the common stock underlying such stock options.
- (3) Mr. Graney's salary was prorated to reflect the portion of the calendar year during which he was employed with us, taking into account the portion of the year in which Mr. Graney was a full-time employee. His salary also includes \$50,000 paid under a consulting agreement before the commencement of his employment with us.

### Narrative to summary compensation table

**Base salary.** During 2019, the annualized base salaries for Dr. McDonough, Dr. Kerr and Mr. Graney were \$423,500, \$396,069 and \$375,000, respectively. We use base salaries to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our named executive officers. None of our named executive officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary.

**Annual bonus.** Our board of directors may, in its discretion, award bonuses to our named executive officers from time to time. We typically establish annual bonus targets based on specified corporate goals and individual performance and conduct annual performance reviews to assess individual performance. Each of our named executive officers was eligible to receive an annual bonus for 2019, with the target amount of such bonus for each named executive officer set forth in his employment or letter agreement with us. For 2019, the

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target bonus amounts, expressed as a percentage of base salary, for each of Dr. McDonough, Dr. Kerr and Mr. Graney were as follows: 40%, 35% and 35%, respectively.

With respect to 2019, our board of directors awarded bonuses of \$169,400, \$138,624 and \$112,551 to Dr. McDonough, Dr. Kerr and Mr. Graney, respectively, with the bonus for Mr. Graney pro-rated to reflect the portion of the calendar year during which he was employed by us.

*Equity incentives.* Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers, or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executive officers with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executive officers and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incentivizes our executive officers to remain in our employment during the vesting period. Accordingly, our board of directors periodically reviews the equity incentive compensation of our executive officers, including our named executive officers, and from time to time may grant equity incentive awards to them in the form of stock options.

We granted an option to purchase 770,000 shares of our common stock to Mr. Graney in February 2019 in connection with him joining the company as chief financial officer. This option vested as to 25% of the shares underlying the option January 5, 2020, and then an additional 6.25% of the shares underlying the option in quarterly installments thereafter, subject to continuous service. When Mr. Graney's employment with us ends on April 10, 2020, 25% of the unvested portion of his option will vest subject to certain conditions and the remainder will be cancelled.

Prior to this offering, our executive officers were eligible to participate in our 2017 Stock Incentive Plan, as amended, or the 2017 Plan. All stock options were granted pursuant to the 2017 Plan. We did not grant any restricted stock awards during 2019. Following this offering, our employees and executive officers will be eligible to receive stock options and other stock-based awards pursuant to our 2020 Stock Incentive Plan, or the 2020 Plan.

We have used stock options to compensate our executive officers in the form of initial grants in connection with the commencement of employment. Prior to this offering, awards of stock options and restricted stock to our executive officers have been made by our board of directors. The options and restricted stock that we have granted to our executive officers are typically subject to time-based vesting, generally over four years following the vesting commencement date. Upon certain terminations of employment in connection with a change of control, vesting is fully accelerated; upon other involuntary terminations, 25% of the unvested portion of each grant will vest as of the date of the termination. Prior to the exercise of a stock option, the holder has no rights as a stockholder with respect to the shares subject to such option, including no voting rights and no right to receive dividends or dividend equivalents.

We have historically awarded stock options with exercise prices that are equal to the fair market value of our common stock on the date of grant as determined by our board of directors.

## Outstanding equity awards at fiscal year-end

The following table sets forth information regarding all outstanding equity awards for each of our named executive officers as of December 31, 2019:

| Name            | Number of securities underlying options (#) |               | Option awards              |                        | Stock awards                                       |  |
|-----------------|---|---------------|----------------------------|------------------------|--|--|
|                 | exercisable                                 | unexercisable | Option exercise price (\$) | Option expiration date | Number of shares of stock that have not vested (#) | Market value of shares of stock that have not vested (\$)(1) |
| Geoff McDonough | —   | —             | —                          | —                      | 1,427,115(2)                                       | —  |
| Douglas Kerr    | 81,975                                      | 136,625(3)    | 2.60                       | 4/2/2028               | 274,450(4)   | —  |
| Thomas Graney   | —   | 770,000(5)    | 2.60                       | (6)                    | —  | —  |

- (1) The market price of our common stock is based on an assumed initial public offering price of \$ per share, which is the midpoint of the price range set forth on the cover page of this prospectus.
- (2) Dr. McDonough's restricted stock award for 2,812,500 shares vests over four years, with 25% of the shares vesting on October 16, 2017, and the remainder vesting in equal quarterly installments thereafter, subject to continuous service. The vesting of this restricted stock award will accelerate upon a qualifying termination of Dr. McDonough's employment.
- (3) Dr. Kerr's option award for 218,600 shares vests over four years, with 25% of the shares vesting on April 2, 2019, and the remainder vesting in equal quarterly installments thereafter, subject to continuous service. The vesting of this option award will accelerate upon a qualifying termination of Dr. Kerr's employment.
- (4) Dr. Kerr's restricted stock awards vest over four years, (i) with respect to the award for 400,000 shares, 25% of the shares vested on August 14, 2018, and (ii) with respect to the award for 176,800 shares, 25% of the shares vested on January 4, 2019, with each of (i) and (ii) vesting in equal quarterly installments thereafter, subject to continuous service. The vesting of these restricted stock awards will accelerate upon a qualifying termination of Dr. Kerr's employment.
- (5) Mr. Graney's option award for 770,000 shares vested as to 25% of the shares underlying the option on January 5, 2020, and vests as to an additional 6.25% of the shares underlying the option in quarterly installments thereafter, subject to continuous service. When Mr. Graney's employment with us ends on April 10, 2020, an additional 25% of the unvested portion of his option award will vest subject to certain conditions and the remainder will be cancelled.
- (6) In connection with the termination of Mr. Graney's employment, his option expiration date was extended to the earlier of (i) 18 months after Mr. Graney's separation on April 10, 2020 or (ii) 30 days after the closing of our initial public offering.

## Stock option and other compensation plans

In this section we describe our 2017 Plan, our 2020 Plan and our Employee Stock Purchase Plan, or 2020 ESPP. Prior to this offering, we granted awards to eligible participants under the 2017 Plan. Following the effectiveness of the 2020 Plan, we expect to grant awards to eligible participants from time to time only under the 2020 Plan.

### 2017 Stock incentive plan

The 2017 Plan was initially approved by our board of directors and our stockholders in October 2017, and was subsequently amended in October 2017, November 2017, February 2018, December 2018 and January 2020, in each case solely to increase the total number of shares reserved for issuance under the 2017 Plan. The 2017 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, awards of restricted stock, restricted stock units and other stock-based awards. Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2017 Plan; however, incentive stock options may only be granted to our employees. The type of award granted under the 2017 Plan and the terms of such award are set forth in the applicable award agreement. Pursuant to the terms of the 2017 Plan, our board of directors (or a committee delegated by our board of directors) administers the plan and, subject to any limitations in the plan, selects the recipients of awards and determines:

- the number of shares of our common stock covered by options and the dates upon which the options become exercisable;

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- the type of options to be granted;
- the duration of options, which may not be in excess of ten years;
- the exercise price of options, which must be at least equal to the fair market value of our common stock on the date of grant; and
- the number of shares of our common stock subject to, and the terms and conditions of, any stock appreciation rights, awards of restricted stock, restricted stock units or other stock-based awards, including conditions for repurchase or cancellation, measurement price, issue price and repurchase price (though the measurement price of stock appreciation rights must be at least equal to the fair market value of our common stock on the date of grant and the duration of such awards may not be in excess of ten years).

The maximum number of shares of common stock authorized for issuance under the 2017 Plan is 18,150,000 shares. Our board of directors may amend, suspend or terminate the 2017 Plan at any time, except that stockholder approval may be required to comply with applicable law.

### *Effect of certain changes in capitalization*

In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock other than an ordinary cash dividend, we are required by the 2017 Plan to make equitable adjustments (or make substitute awards, if applicable), in the manner determined by our board of directors, to:

- the number and class of securities available under the 2017 Plan;
- the number and class of securities and exercise price per share of each outstanding option;
- the share and per-share provisions and measurement price of each outstanding stock appreciation right;
- the number of shares and the repurchase price per share subject to each outstanding award of restricted stock; and
- the share and per-share related provisions and purchase price, if any, of each outstanding restricted stock unit award and each other stock-based award.

### *Effect of certain corporate transactions*

Upon the occurrence of a merger or other reorganization event (as defined in the 2017 Plan), our board of directors may, on such terms as our board of directors determines (except to the extent specifically provided otherwise in an applicable award agreement or other agreement between the participant and us), take any one or more of the following actions pursuant to the 2017 Plan as to all or any (or any portion of) outstanding awards, other than awards of restricted stock:

- provide that outstanding awards will be assumed, or substantially equivalent awards will be substituted, by the acquiring or succeeding corporation (or an affiliate of the acquiring or succeeding corporation);
- upon written notice to a participant, provide that all of the participant's unexercised and/or unvested awards will terminate immediately prior to the consummation of such transaction unless exercised, to the extent exercisable, by the participant within a specified period following the date of such notice;
- provide that outstanding awards will become exercisable, realizable or deliverable, or restrictions applicable to an award will lapse, in whole or in part, prior to or upon the reorganization event;

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- in the event of a reorganization event pursuant to which holders of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award;
- provide that, in connection with our liquidation or dissolution, awards convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings); or
- any combination of the foregoing.

In taking any of the foregoing actions, our board of directors is not obligated by the 2017 Plan to treat all awards, all awards held by a participant, or all awards of the same type, identically.

In the case of certain restricted stock units, no assumption or substitution is permitted, and the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement.

Upon the occurrence of a reorganization event other than our liquidation or dissolution, our repurchase and other rights with respect to outstanding awards of restricted stock will continue for the benefit of the succeeding company and will, unless our board of directors determines otherwise, apply to the cash, securities or other property which our common stock was converted into or exchanged for pursuant to the reorganization event in the same manner and to the same extent as they applied to the common stock subject to the restricted stock award. However, our board of directors may provide for the termination or deemed satisfaction of such repurchase or other rights under the restricted stock award agreement or any other agreement between a participant and us, either initially or by amendment, or provide for forfeiture of such restricted stock if issued at no cost. Upon the occurrence of a reorganization event involving our liquidation or dissolution, except to the extent specifically provided to the contrary in the restricted stock award agreement or any other agreement between the participant and us, all restrictions and conditions on all outstanding restricted stock awards will automatically be deemed terminated or satisfied.

Our board of directors may at any time provide that any award under the 2017 Plan shall become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

As of March 31, 2020, there were options to purchase an aggregate of 8,894,975 shares of common stock outstanding under the 2017 Plan at a weighted-average exercise price of \$2.77 per share and options to purchase 1,669,606 shares of common stock were available for future issuance under the 2017 Plan. No further awards will be made under the 2017 Plan on or after the effective date of the 2020 Plan described below; however, awards outstanding under the 2017 Plan will continue to be governed by their existing terms.

### **2020 Stock incentive plan**

We expect our board of directors to adopt and our stockholders to approve the 2020 Plan, which will become effective immediately prior to the effectiveness of the registration statement for this offering. The 2020 Plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, restricted stock awards, restricted stock units and other stock-based awards. Upon effectiveness of the 2020 Plan, the number of shares of our common stock that will be reserved for issuance under the 2020 Plan will be



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the sum of (1) \_\_\_\_\_ ; plus (2) the number of shares (up to a maximum of \_\_\_\_\_ shares) as is equal to the sum of (x) the number of shares of our common stock reserved for issuance under the 2017 Plan that remain available for grant under the 2017 Plan immediately prior to the effectiveness of the registration statement for this offering and (y) the number of shares of our common stock subject to outstanding awards granted under the 2017 Plan that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by us at their original issuance price pursuant to a contractual repurchase right; plus (3) an annual increase, to be added on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2021 and continuing until, and including, the fiscal year ending December 31, 2030, equal to the lowest of (i) \_\_\_\_\_ shares of our common stock, (ii) \_\_\_\_\_ % of the number of shares of our common stock outstanding on such date, and (iii) an amount determined by our board of directors.

Our employees, officers, directors, consultants and advisors are eligible to receive awards under the 2020 Plan; however, incentive stock options may only be granted to our employees.

Pursuant to the terms of the 2020 Plan, our board of directors (or a committee delegated by our board of directors) will administer the 2020 Plan and, subject to any limitations set forth in the 2020 Plan, will select the recipients of awards and determine:

- the number of shares of our common stock covered by options and the dates upon which the options become exercisable;
- the type of options to be granted;
- the exercise price of options, which price must be at least equal to the fair market value of our common stock on the date of grant;
- the duration of options, which may not be in excess of ten years;
- the methods of payment of the exercise price of options; and
- the number of shares of our common stock subject to and the terms and conditions of any stock appreciation rights, awards of restricted stock, restricted stock units or other stock-based awards, including conditions for repurchase, measurement price, issue price and repurchase price (though the measurement price of stock appreciation rights must be at least equal to the fair market value of our common stock on the date of grant and the duration of such awards may not be in excess of ten years).

If our board of directors delegates authority to one or more of our officers to grant awards under the 2020 Plan, the officer will have the power to make awards to all of our employees, except officers and executive officers (as such terms are defined in the 2020 Plan). Our board of directors will fix the terms of the awards to be granted by any such officer, the maximum number of shares subject to awards that any such officer may grant, and the time period in which such awards may be granted.

The 2020 Plan contains limits on the compensation that may be paid to our non-employee directors. The maximum amount of cash and equity compensation (calculated based on grant-date fair value for financial reporting purposes) granted in any calendar year to any individual non-employee director in his or her capacity as a non-employee director may not exceed \$ \_\_\_\_\_ in the case of an incumbent director, \$ \_\_\_\_\_ in the case of the chair of our board of directors, or \$ \_\_\_\_\_ in the case of a new director during his or her first year of service; provided, however, that fees paid by us on behalf of any non-employee director in connection with regulatory compliance and any amounts paid to the non-employee director as reimbursement of an expense shall not count against the foregoing limit. However, our board of directors may make exceptions to this limit for individual non-employee directors in extraordinary circumstances, as the board of directors may determine in its discretion, provided that the non-employee director receiving such additional compensation may not

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participate in the decision to award such compensation. For the avoidance of doubt, cash and awards granted under the 2020 Plan to non-employee directors in their capacity as consultants or advisors to us are not subject to the foregoing limit.

### *Effect of certain changes in capitalization*

In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock other than an ordinary cash dividend, we are required by the 2020 Plan to make equitable adjustments (or make substitute awards, if applicable), in the manner determined by our board of directors, to:

- the number and class of securities available under the 2020 Plan;
- the share counting rules and sublimits of the 2020 Plan;
- the number and class of securities and exercise price per share of each outstanding option;
- the share and per-share provisions and measurement price of each outstanding stock appreciation right;
- the number of shares and the repurchase price per share subject to each outstanding award of restricted stock; and
- the share and per-share related provisions and purchase price, if any, of each outstanding restricted stock unit award and each other stock-based award.

### *Effect of certain corporate transactions*

Upon the occurrence of a merger or other reorganization event (as defined in the 2020 Plan), our board of directors may, on such terms as our board of directors determines (except to the extent specifically provided otherwise in an applicable award agreement or other agreement between the participant and us), take any one or more of the following actions pursuant to the 2020 Plan as to all or any (or any portion of) outstanding awards, other than awards of restricted stock:

- provide that outstanding awards will be assumed, or substantially equivalent awards will be substituted, by the acquiring or succeeding corporation (or an affiliate of the acquiring or succeeding corporation);
- upon written notice to a participant, provide that all of the participant's unvested awards will be forfeited immediately prior to the consummation of the reorganization event and/or vested but unexercised awards will terminate immediately prior to the consummation of such transaction unless exercised, to the extent exercisable, by the participant within a specified period following the date of such notice;
- provide that outstanding awards will become exercisable, realizable or deliverable, or restrictions applicable to an award will lapse, in whole or in part, prior to or upon the reorganization event;
- in the event of a reorganization event pursuant to which holders of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award;

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- provide that, in connection with our liquidation or dissolution, awards convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings); or
- any combination of the foregoing.

In taking any of the foregoing actions, our board of directors is not obligated by the 2020 Plan to treat all awards, all awards held by a participant, or all awards of the same type, identically.

In the case of certain restricted stock units, no assumption or substitution is permitted, and the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement.

Upon the occurrence of a reorganization event other than our liquidation or dissolution, our repurchase and other rights with respect to each outstanding award of restricted stock will continue for the benefit of the succeeding company (or any affiliate of the succeeding corporation) and will, unless our board of directors determines otherwise, apply to the cash, securities, or other property which our common stock is converted into or exchanged for pursuant to the reorganization event in the same manner and to the same extent as they applied to the common stock subject to the restricted stock award. However, our board of directors may provide for the termination or deemed satisfaction of such repurchase or other rights under the restricted stock award agreement or in any other agreement between a participant and us, either initially or by amendment. Upon the occurrence of a reorganization event involving our liquidation or dissolution, except to the extent specifically provided to the contrary in the restricted stock award agreement or any other agreement between the participant and us, all restrictions and conditions on each outstanding restricted stock award will automatically be deemed terminated or satisfied.

Our board of directors may, at any time, provide that any award under the 2020 Plan will become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

Except with respect to certain actions requiring stockholder approval under the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, or Nasdaq Stock Market rules, our board of directors may amend, modify or terminate any outstanding award under the 2020 Plan, including but not limited to, substituting for the award another award of the same or a different type, changing the date of exercise or realization, and converting an incentive stock option to a nonstatutory stock option, subject to certain participant consent requirements. However, unless our stockholders approve such action, the 2020 Plan provides that we may not (except as otherwise permitted in connection with a change in capitalization or reorganization event):

- amend any outstanding stock option or stock appreciation right granted under the 2020 Plan to provide an exercise or measurement price per share that is lower than the then-current exercise or measurement price per share of such outstanding award;
- cancel any outstanding stock option or stock appreciation right (whether or not granted under the 2020 Plan) and grant a new award under the 2020 Plan in substitution for the cancelled award (other than substitute awards permitted in connection with a merger or consolidation of an entity with us or our acquisition of property or stock of another entity) covering the same or a different number of shares of our common stock and having an exercise or measurement price per share lower than the then-current exercise or measurement price per share of the cancelled award;
- cancel in exchange for a cash payment any outstanding option or stock appreciation right with an exercise or measurement price per share above the then-current fair market value of our common stock (valued in the manner determined by (or in the manner approved by) our board of directors); or

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- take any other action that constitutes a “repricing” within the meaning of Nasdaq Stock Market rules or rules of any other exchange or marketplace on which our common stock is listed or traded.

No award may be granted under the 2020 Plan on or after the date that is ten years from the effectiveness of the 2020 Plan. Our board of directors may amend, suspend or terminate the 2020 Plan at any time, except that stockholder approval may be required to comply with applicable law or stock market requirements.

### **2020 Employee stock purchase plan**

We expect our board of directors to adopt and our stockholders to approve the 2020 ESPP, which will become effective immediately prior to the effectiveness of the registration statement for this offering. The 2020 ESPP will be administered by our board of directors or by a committee appointed by our board of directors. The 2020 ESPP initially provides participating employees with the opportunity to purchase up to an aggregate of \_\_\_\_\_ shares of our common stock. The number of shares of our common stock reserved for issuance under the 2020 ESPP will automatically increase on the first day of each fiscal year, beginning with the fiscal year commencing on January 1, 2021 and continuing for each fiscal year until, and including the fiscal year commencing on, January 1, 2030, in an amount equal to the lowest of (1) \_\_\_\_\_ shares of our common stock, (2) \_\_\_\_\_ % of the number of shares of our common stock outstanding on such date, and (3) an amount determined by our board of directors.

All of our employees and employees of any designated subsidiary, as defined in the 2020 ESPP, are eligible to participate in the 2020 ESPP, provided that:

- such person is customarily employed by us or a designated subsidiary for more than 20 hours a week and for more than five months in a calendar year;
- such person has been employed by us or by a designated subsidiary for at least three months prior to enrolling in the 2020 ESPP; and
- such person was our employee or an employee of a designated subsidiary on the first day of the applicable offering period under the 2020 ESPP.

We retain the discretion to determine which eligible employees may participate in an offering under applicable regulations.

We expect to make one or more offerings to our eligible employees to purchase stock under the 2020 ESPP beginning at such time and on such dates as our board of directors may determine, or on the first business day thereafter. Each offering will consist of a six-month offering period during which payroll deductions will be made and held for the purchase of our common stock at the end of the offering period. Our board of directors or a committee designated by the board of directors may, at its discretion, choose a different period of not more than 12 months for offerings.

On each offering commencement date, each participant will be granted an option to purchase, on the last business day of the offering period, up to a number of shares of our common stock determined by multiplying \$2,083 by the number of full months in the offering period and dividing that product by the closing price of our common stock on the first day of the offering period. No employee may be granted an option under the 2020 ESPP that permits the employee’s rights to purchase shares under the 2020 ESPP and any other employee stock purchase plan of ours or of any of our subsidiaries to accrue at a rate that exceeds \$25,000 of the fair market value of our common stock (determined as of the first day of each offering period) for each calendar year in which the option is outstanding. In addition, no employee may purchase shares of our common stock under the 2020 ESPP that would result in the employee owning 5% or more of the total combined voting power or value of our stock or the stock of any of our subsidiaries.

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Each eligible employee may authorize up to a maximum of 15% of his or her compensation to be deducted by us during the offering period. Each employee who continues to be a participant in the 2020 ESPP on the last business day of the offering period will be deemed to have exercised an option to purchase from us the number of whole shares of our common stock that his or her accumulated payroll deductions on such date will pay for, not in excess of the maximum numbers set forth above. Under the terms of the 2020 ESPP, the purchase price will be determined by our board of directors or the committee for each offering period and will be at least 85% of the applicable closing price of our common stock. If our board of directors or the committee does not make a determination of the purchase price, the purchase price will be 85% of the lesser of the closing price of our common stock on the first business day of the offering period or on the last business day of the offering period.

An employee may at any time prior to the close of business on the fifteenth business day (or such other number of days as is determined by us) prior to the end of the offering period, and for any reason, permanently withdraw from participating in the offering and permanently withdraw the balance accumulated in the employee's account. Partial withdrawals are not permitted. If an employee elects to discontinue his or her payroll deductions during an offering period but does not elect to withdraw his or her funds, funds previously deducted will be applied to the purchase of common stock at the end of the offering period. If a participating employee's employment ends before the last business day of an offering period, no additional payroll deductions will be taken and the balance in the employee's account will be paid to the employee.

We will be required to make equitable adjustments to the extent determined by our board of directors or a committee thereof to the number and class of securities available under the 2020 ESPP, the share limitations under the 2020 ESPP, and the purchase price for an offering period under the 2020 ESPP to reflect stock splits, reverse stock splits, stock dividends, recapitalizations, combinations of shares, reclassifications of shares, spin-offs and other similar changes in capitalization or events or any dividends or distributions to holders of our common stock other than ordinary cash dividends.

In connection with a merger or other reorganization event, as defined in the 2020 ESPP, our board of directors or a committee of our board of directors may take any one or more of the following actions as to outstanding options to purchase shares of our common stock under the 2020 ESPP on such terms as our board of directors or committee thereof determines:

- provide that options will be assumed, or substantially equivalent options will be substituted, by the acquiring or succeeding corporation (or an affiliate of the acquiring or succeeding corporation);
- upon written notice to employees, provide that all outstanding options will be terminated immediately prior to the consummation of such reorganization event and that all such outstanding options will become exercisable to the extent of accumulated payroll deductions as of a date specified by the board of directors or committee thereof in such notice, which date will not be less than ten days preceding the effective date of the reorganization event;
- upon written notice to employees, provide that all outstanding options will be cancelled as of a date prior to the effective date of the reorganization event and that all accumulated payroll deductions will be returned to participating employees on such date;
- in the event of a reorganization event under the terms of which holders of our common stock will receive upon consummation thereof a cash payment for each share surrendered in the reorganization event, change the last day of the offering period to be the date of the consummation of the reorganization event and make or provide for a cash payment to each employee equal to (1) the cash payment for each share surrendered in the reorganization event times the number of shares of our common stock that the employee's accumulated payroll deductions as of immediately prior to the reorganization event could purchase at the applicable purchase price, where the cash payment for each share surrendered in the reorganization event is treated as

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the fair market value of our common stock on the last day of the applicable offering period for purposes of determining the purchase price and where the number of shares that could be purchased is subject to the applicable limitations under the 2020 ESPP minus (2) the result of multiplying such number of shares by the purchase price; and/or

- provide that, in connection with our liquidation or dissolution, options convert into the right to receive liquidation proceeds (net of the purchase price thereof).

Our board of directors may at any time, and from time to time, amend or suspend the 2020 ESPP or any portion of the 2020 ESPP. We will obtain stockholder approval for any amendment if such approval is required by Section 423 of the Code. Further, our board of directors may not make any amendment that would cause the 2020 ESPP to fail to comply with Section 423 of the Code. The 2020 ESPP may be terminated at any time by our board of directors. Upon termination, we will refund all amounts in the accounts of participating employees.

### **401(k) plan**

We maintain a defined contribution employee retirement plan for our employees, including our named executive officers. The plan is intended to qualify as a tax-qualified 401(k) plan so that contributions to the 401(k) plan, and income earned on such contributions, are not taxable to participants until withdrawn or distributed from the 401(k) plan (except in the case of contributions under the 401(k) plan designated as Roth contributions). Under the 401(k) plan, each employee is fully vested in his or her deferred salary contributions and our discretionary match after one year of employment with us. Employee contributions are held and invested by the plan's trustee as directed by participants. The 401(k) plan provides us with the discretion to match employee contributions, but to date we have not provided any employer matching contributions.

### **Limitation of liability and indemnification**

Our certificate of incorporation, which will become effective upon the closing of this offering, limits the personal liability of directors for breach of fiduciary duty to the maximum extent permitted by the Delaware General Corporation Law, or the DGCL, and provides that no director will have personal liability to us or to our stockholders for monetary damages for breach of fiduciary duty as a director. However, these provisions do not eliminate or limit the liability of any of our directors:

- for any breach of the director's duty of loyalty to us or our stockholders;
- for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law;
- for voting for or assenting to unlawful payments of dividends, stock repurchases or other distributions; or
- for any transaction from which the director derived an improper personal benefit.

Any amendment to or repeal of these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to such amendment or repeal. If the DGCL is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our directors will be further limited to the greatest extent permitted by the DGCL.

In addition, our certificate of incorporation, which will become effective upon the closing of this offering, provides that we must indemnify our directors and officers and we must advance expenses, including attorneys' fees, to our directors and officers in connection with legal proceedings, subject to very limited exceptions.

We maintain a general liability insurance policy that covers specified liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers. In addition, we

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intend to enter into indemnification agreements with all of our executive officers and directors prior to the completion of this offering. These indemnification agreements may require us, among other things, to indemnify each such executive officer or director for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by him or her in any action or proceeding arising out of his or her service as one of our executive officers or directors.

Some of our non-employee directors may, through their relationships with their employers, be insured or indemnified against specified liabilities incurred in their capacities as members of our board of directors.

Insofar as indemnification for liabilities arising under the Securities Act of 1933, or the Securities Act, may be permitted to directors, executive officers or persons controlling us, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

### **Rule 10b5-1 plans**

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from the director or officer. It also is possible that the director or officer could amend the plan in certain circumstances when not in possession of material, nonpublic information or terminate the plan. In addition, our directors and executive officers may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

### **Director compensation**

The table below shows all compensation to our non-employee directors during the year ended December 31, 2019.

| <b>Name</b>                      | <b>Fees earned or paid in cash(\$)</b> | <b>Stock awards(\$)<br/>(1)</b> | <b>Option awards(\$)<br/>(2)</b> | <b>Total(\$)</b> |
|----------------------------------|--|---------------------------------|----------------------------------|------------------|
| Jason Rhodes                     | —                                      | —                               | —                                | —                |
| Gustav Christensen               | 32,500(3)                              | —                               | —                                | 32,500           |
| Donald Nicholson, Ph.D.          | 32,500(3)                              | —                               | —                                | 32,500           |
| Catherine Stehman-Breen, M.D.    | 25,000                                 | —                               | —                                | 25,000           |
| Anthony Quinn, M.D. Ch.B., Ph.D. | 25,000                                 | —                               | —                                | 25,000           |
| Jeffrey Jonas, M.D.              | 25,000                                 | —                               | —                                | 25,000           |
| Charles Rowland                  | 40,000(3)(4)                           | —                               | —                                | 40,000           |

(1) As of December 31, 2019, the aggregate number of shares of our common stock held pursuant to restricted stock awards by each non-employee director was as follows: Mr. Rhodes, 0 shares; Mr. Christensen, 75,000 shares; Dr. Nicholson, 75,000 shares; Dr. Stehman-Breen, 75,000 shares; Dr. Quinn, 75,000 shares; Dr. Jonas, 0 shares; and Mr. Rowland 0 shares.

(2) As of December 31, 2019, the aggregate number of shares of our common stock subject to outstanding option awards for each non-employee director was as follows: Mr. Rhodes, 0 shares; Mr. Christensen, 0 shares; Dr. Nicholson, 0 shares; Dr. Stehman-Breen, 0 shares; Dr. Quinn, 0 shares; Dr. Jonas, 75,000 shares; and Mr. Rowland 75,000 shares.

(3) Mr. Christensen, Dr. Nicholson, and Mr. Rowland received an additional \$7,500 as a committee retainer fee for their service on our audit committee.

(4) Mr. Rowland received an additional \$7,500 as a chairmanship fee for his service as chair of our audit committee.

Prior to this offering, we granted restricted stock awards for 75,000 shares of our common stock or options to purchase 75,000 shares of our common stock to our non-employee directors upon their initial election to the Board and paid annual cash fees our non-employee directors for their service on our board of directors. However, we did not have a formal non-employee director compensation policy. In 2019, we did not grant any

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equity awards to any of our directors. We have historically reimbursed our non-employee directors for reasonable travel and out-of-pocket expenses incurred in connection with attending board of director and committee meetings. We do not pay any compensation to our President and Chief Executive Officer in connection with his service on our board of directors.

In \_\_\_\_\_, 2020, our board of directors approved a director compensation program that will become effective on the effective date of the registration statement of which this prospectus is a part. Under this director compensation program, we will pay our non-employee directors a cash retainer for service on the board of directors and for service on each committee on which the director is a member. The chair of the board and of each committee will receive higher retainers for such service. These fees are payable in arrears in four equal quarterly installments on the last day of each quarter, provided that the amount of such payment will be prorated for any portion of such quarter that the director is not serving on our board of directors and no fee will be payable in respect of any period prior to the completion of this offering. The fees paid to non-employee directors for service on the board of directors and for service on each committee of the board of directors on which the director is a member are as follows:

|   | <b>Member<br/>Annual Fee</b> | <b>Chair<br/>Annual Fee</b> |
|---|------------------------------|-----------------------------|
| Board of Directors                            | \$                           | \$                          |
| Audit Committee                               | \$                           | \$                          |
| Talent Committee                              | \$                           | \$                          |
| Nominating and Corporate Governance Committee | \$                           | \$                          |

We also will continue to reimburse our non-employee directors for reasonable travel and other expenses incurred in connection with attending meetings of our board of directors and any committee of our board of directors on which he or she serves.

In addition, under our director compensation program to be effective on the effective date of the registration statement of which this prospectus is a part, each non-employee director will receive, upon his or her initial election or appointment to our board of directors, an option to purchase \_\_\_\_\_ shares of our common stock under the 2020 Plan. Each of these options will vest as to \_\_\_\_\_ of the shares of our common stock underlying such option on the first anniversary of the grant, with the remainder vesting in equal monthly installments until the \_\_\_\_\_ anniversary of the date of grant, subject to the non-employee director's continued service as a director. Further, on the date of each annual meeting of stockholders, each non-employee director that has served on our board of directors for at least six months will receive, under the 2020 Plan, an option to purchase \_\_\_\_\_ shares of our common stock under the 2020 Plan. Each of these options will vest \_\_\_\_\_, subject to the non-employee director's continued service as a director. All options issued to our non-employee directors under our director compensation program will be issued at exercise prices equal to the fair market value of our common stock on the date of grant and will have a term of ten years.



## Transactions with related persons

Since January 1, 2017, we have engaged in the following transactions in which the amounts involved exceeded \$120,000 and any of our directors, executive officers or holders of more than 5% of our voting securities, or any member of the immediate family of, or person sharing the household with, the foregoing persons, had or will have a direct or indirect material interest. We believe that all of the transactions described below were made on terms no less favorable to us than could have been obtained from unrelated third parties.

### Convertible promissory notes and SAFEs

From November 2016 to February 2017, we issued convertible promissory notes in an aggregate principal amount of \$1,000,000 to Atlas Venture Fund X, L.P. These notes, which we refer to as the Atlas notes, accrued interest at a rate of 6% per annum. On November 20, 2017, all principal and accrued but unpaid interest under the notes were converted into 1,051,020 shares of our Series A preferred stock.

Between April and September 2017, we issued Simple Agreements for Future Equity, or SAFEs, in an aggregate principal amount of \$6,000,000 to Atlas Venture Fund X, L.P. On November 20, 2017, these agreements, which we refer to as the Atlas SAFEs, were converted into 6,000,000 shares of our Series A preferred stock.

### Series A preferred stock financing

From November to December 2017, we issued and sold (1) 8,400,000 shares of our Series A preferred stock at a price per share of \$1.00 in cash, for an aggregate purchase price of \$8,400,000, (2) 1,051,020 shares of our Series A preferred stock upon conversion of the Atlas notes, and (3) 6,000,000 shares of our Series A preferred stock upon conversion of the Atlas SAFEs. The following table sets forth the aggregate number of shares of our Series A preferred stock that we issued and sold to our directors, officers and 5% stockholders and their affiliates in this transaction and the aggregate amount of consideration for such shares:

| Purchaser(1)               | Shares of<br>Series A<br>preferred<br>stock | Cash<br>purchase<br>price |
|----------------------------|---|---------------------------|
| Atlas Venture Fund X, L.P. | 8,000,000                                   | \$ 8,000,000              |
| Geoff McDonough            | 200,000                                     | \$ 200,000                |

(1) See "Principal stockholders" for additional information about shares held by these entities.

### Series B preferred stock financing

In February 2018, we issued and sold an aggregate of 10,974,644 shares of our Series B preferred stock at a price per share of \$9.1457 in cash, for an aggregate purchase price of \$100,370,801.68. The following table sets forth the aggregate number of shares of our Series B preferred stock that we issued and sold to our directors, officers and 5% stockholders and their affiliates in this transaction and the aggregate amount of consideration for such shares:

| Purchaser(1)   | Shares of<br>Series B<br>preferred<br>stock | Cash purchase<br>price |
|--|---|------------------------|
| Entities affiliated with Fidelity Investment         | 5,731,743                                   | \$ 52,420,802          |
| Entities affiliated with Invus Public Equities, L.P. | 2,186,820                                   | \$ 20,000,000          |

(1) See "Principal stockholders" for additional information about shares held by these entities.

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### **Series C preferred stock financing**

In January 2020, we issued and sold an aggregate of 19,936,296 shares of our Series C preferred stock at a price per share of \$5.5914 in cash, for an aggregate purchase price of \$111,471,805.56. The following table sets forth the aggregate number of shares of our Series C preferred stock that we issued and sold to our directors, officers and 5% stockholders and their affiliates in this transaction and the aggregate amount of consideration for such shares:

| <b>Purchaser(1)</b>   | <b>Shares of Series C preferred stock</b> | <b>Cash purchase price</b> |
|---|---|----------------------------|
| Entities affiliated with Atlas Venture Fund X, L.P.               | 2,682,691                                 | \$ 14,999,998              |
| Entities affiliated with Fidelity Investment                      | 2,457,000                                 | \$ 13,738,070              |
| Invus Public Equities, L.P.                                       | 1,073,076                                 | \$ 5,999,997               |
| Certain funds and accounts advised or subadvised by T. Rowe Price | 5,365,382                                 | \$ 29,999,997              |
| Gustav Christensen  | 44,711                                    | \$ 249,997                 |
| Anthony Quinn   | 53,654                                    | \$ 300,001                 |
| Charles Rowland   | 89,423                                    | \$ 500,000                 |

(1) See "Principal stockholders" for additional information about shares held by these entities.

### **Registration rights**

We are a party to an investors' rights agreement with the holders of our preferred stock, including our 5% stockholders and their affiliates and certain of our directors and officers. This investors' rights agreement provides these stockholders the right, subject to certain conditions, beginning six months following the completion of this offering, to demand that we file a registration statement or to request that their shares be covered by a registration statement that we are otherwise filing.

See "Description of capital stock—Registration rights" for additional information regarding these registration rights.

### **Indemnification agreements**

Our certificate of incorporation, which will become effective upon the closing of this offering, provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we intend to enter into new indemnification agreements with all of our directors and executive officers prior to the completion of this offering. These indemnification agreements may require us, among other things, to indemnify each such director or executive officer for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by him or her in any action or proceeding arising out of his or her service as one of our directors or executive officers.

### **Policies and procedures for related person transactions**

Our board of directors adopted in \_\_\_\_\_, 2020 written policies and procedures for the review of any transaction, arrangement or relationship in which our company is a participant, the amount involved exceeds \$120,000, and one of our executive officers, directors, director nominees or 5% stockholders (or their immediate family members), each of whom we refer to as a "related person," has a direct or indirect material interest.

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If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a “related person transaction,” the related person must report the proposed related person transaction to our Chief Legal Officer. The policy calls for the proposed related person transaction to be reviewed and, if deemed appropriate, approved by our audit committee. Whenever practicable, the reporting, review and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the audit committee will review, and, in its discretion, may ratify the related person transaction. The policy also permits the chair of the audit committee to review and, if deemed appropriate, approve proposed related person transactions that arise between audit committee meetings, subject to ratification by the audit committee at its next meeting. Any related person transactions that are ongoing in nature will be reviewed annually.

A related person transaction reviewed under the policy will be considered approved or ratified if it is authorized by the audit committee after full disclosure of the related person’s interest in the transaction. As appropriate for the circumstances, the audit committee will review and consider:

- the related person’s interest in the related person transaction;
- the approximate dollar value of the amount involved in the related person transaction;
- the approximate dollar value of the amount of the related person’s interest in the transaction without regard to the amount of any profit or loss;
- whether the transaction was undertaken in the ordinary course of our business;
- whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;
- the purpose of, and the potential benefits to us of, the transaction; and
- any other information regarding the related person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

Our audit committee may approve or ratify the transaction only if it determines that, under all of the circumstances, the transaction is in, or is not inconsistent with, our best interests. Our audit committee may impose any conditions on the related person transaction that it deems appropriate.

In addition to the transactions that are excluded by the instructions to the Securities and Exchange Commission’s related person transaction disclosure rule, our board of directors has determined that the following transactions do not create a material direct or indirect interest on behalf of related persons and, therefore, are not related person transactions for purposes of this policy:

- interests arising solely from the related person’s position as an executive officer of another entity, whether or not the person is also a director of such entity, that is a participant in the transaction, where the related person and all other related persons own in the aggregate less than a 10% equity interest in such entity, the related person and his or her immediate family members are not involved in the negotiation of the terms of the transaction and do not receive any special benefits as a result of the transaction, and the amount involved in the transaction is less than the greater of \$200,000 or 5% of the annual gross revenues of the company receiving payment under the transaction; and
- a transaction that is specifically contemplated by provisions of our certificate of incorporation or bylaws.

The policy provides that transactions involving compensation of executive officers shall be reviewed and approved by our talent committee in the manner specified in the talent committee’s charter.

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We did not have a written policy regarding the review and approval of related person transactions prior to this offering. Nevertheless, with respect to such transactions, it has been the practice of our board of directors to consider the nature of and business reasons for such transactions, how the terms of such transactions compared to those which might be obtained from unaffiliated third parties and whether such transactions were otherwise fair to and in the best interests of, or not contrary to, our best interests.

## Principal stockholders

The following table sets forth information with respect to the beneficial ownership of our common stock, as of March 31, 2020 by:

- each of our directors;
- each of our named executive officers;
- all of our executive officers and directors as a group; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock.

The column entitled “Percentage of shares beneficially owned—Before offering” is based on a total of 60,239,750 shares of our common stock outstanding as of March 31, 2020, assuming the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 47,856,346 shares of our common stock upon the closing of this offering. The column entitled “Percentage of shares beneficially owned—After offering” is based on \_\_\_\_\_ shares of our common stock to be outstanding after this offering, including the shares of our common stock that we are selling in this offering and \_\_\_\_\_ shares of unvested restricted stock subject to repurchase by us, but not including any additional shares issuable upon exercise of outstanding options.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock that an individual has a right to acquire within 60 days after March 31, 2020 are considered outstanding and beneficially owned by the person holding such right for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person, except with respect to the percentage ownership of all directors and executive officers. Except as otherwise noted, the persons and entities in this table have sole voting and investing power with respect to all of the shares of our common stock beneficially

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owned by them, subject to community property laws, where applicable. Unless otherwise indicated, the address of each beneficial owner is c/o Generation Bio Co. 301 Binney Street, Cambridge, MA 02142.

| Name of beneficial owner  | Number of shares beneficially owned | Percentage of shares beneficially owned |                   |
|---|-------------------------------------|---|-------------------|
|   |                                     | Before offering(%)                      | After offering(%) |
| <b>5% Stockholders</b>  |                                     |   |                   |
| Entities affiliated with Atlas Venture Fund X, L.P.(1)                          | 22,309,711                          | 37.03%                                  |                   |
| Entities affiliated with Fidelity Investment(2)                                 | 8,969,217                           | 14.89%                                  |                   |
| Entities affiliated with Invus Public Equities, L.P.(3)                         | 3,557,667                           | 5.91%                                   |                   |
| Certain funds and accounts advised or subadvised by T. Rowe Price 4)            | 5,365,382                           | 8.91%                                   |                   |
| <b>Named Executive Officers and Directors</b>                                   |                                     |   |                   |
| Jason Rhodes(1)   | 22,309,711                          | 37.03%                                  |                   |
| Geoff McDonough, M.D.(6)  | 3,012,500                           | 5.00%                                   |                   |
| Douglas Kerr, M.D., Ph.D., M.B.A.(7)  | 686,100                             | 1.14%                                   |                   |
| Thomas Graney(8)  | 372,968                             | *                                       |                   |
| Gustav Christensen(9)   | 232,133                             | *                                       |                   |
| Anthony Quinn, M.B. Ch.B., Ph.D.(10)  | 128,654                             | *                                       |                   |
| Charles Rowland(11)   | 122,236                             | *                                       |                   |
| Catherine Stehman-Breen, M.D.(12)   | 83,942                              | *                                       |                   |
| Jeffrey Jonas, M.D.(13)   | 32,813                              | *                                       |                   |
| Donald Nicholson, Ph.D.(5)  | 175,000                             | *                                       |                   |
| <b>All current executive officers and directors as a group (11 persons)(14)</b> | <b>28,324,507</b>                   | <b>46.58%</b>                           |                   |

\* Less than one percent.

- (1) Consists of (i) 15,051,020 shares of common stock underlying shares of Series A convertible preferred stock held by Atlas Venture Fund X, L.P., or Atlas Fund X, (ii) 4,576,000 shares of common stock held by Atlas Fund X and (iii) 2,682,691 shares of common stock underlying shares of Series C convertible preferred stock held by Atlas Venture Opportunity Fund I, L.P., or Atlas Fund I. Atlas Venture Associates X, L.P. is the general partner of Atlas Fund X, and Atlas Venture Associates X, LLC is the general partner of Atlas Venture Associates X, L.P. Bruce Booth, Jean-Francois Formela, David Grayzel, Jason Rhodes and Kevin Bitterman are the members of Atlas Venture Associates X, LLC and collectively make investment decisions on behalf of Atlas Venture Fund X, LLC. Each of Atlas Fund X, Atlas Venture Associates X, L.P., and Atlas Venture Associates X, LLC may be deemed to beneficially own the shares held by Atlas Fund X. Atlas Fund I is managed by Atlas Venture Associates Opportunity I, L.P., which is managed by Atlas Venture Associates Opportunity I, LLC, or AVAO, LLC. Bruce Booth, Jean-Francois Formela, David Grayzel, Jason Rhodes, and Kevin Bitterman are the members of AVAO LLC and collectively make investment decisions on behalf of Atlas Fund I. Jason Rhodes is also a member of our board of directors. Mr. Rhodes disclaims beneficial ownership of the shares listed. The mailing address of Atlas Fund X and Atlas Fund I is 400 Technology Square, 10th Floor, Cambridge, MA 02139.
- (2) Consists of (i) 596,261 shares of common stock underlying shares of Series B convertible preferred stock and 584,300 shares of common stock underlying shares of Series C convertible preferred stock held by Mag & Co fbo Fidelity Growth Company Commingled Pool, (ii) 945,064 shares of common stock underlying shares of Series B convertible preferred stock and 705,100 shares of common stock underlying shares of Series C convertible preferred stock held by Powhatan & Co., LLC fbo Fidelity Mt. Vernon Street Trust: Fidelity Growth Company Fund, (iii) 523,205 shares of common stock underlying shares of Series B convertible preferred stock and 475,900 shares of common stock underlying shares of Series C convertible preferred stock held by M Gardiner & Co fbo Fidelity Securities Fund: Fidelity Blue Chip Growth Fund, (iv) 17,496 shares of common stock underlying shares of Series B convertible preferred stock and 15,400 shares of common stock underlying shares of Series C convertible preferred stock held by Mag & Co fbo Fidelity Blue Chip Growth Commingled Pool, (v) 48,287 shares of common stock underlying shares of Series B convertible preferred stock and 50,900 shares of common stock underlying shares of Series C convertible preferred stock held by FLAPPER CO fbo FIAM Target Date Blue Chip Growth Commingled Pool, (vi) 25,450 shares of common stock underlying shares of Series B convertible preferred stock and 44,000 shares of common stock underlying shares of Series C convertible preferred stock held by Booth & Co FBO Fidelity Securities Fund: Fidelity Blue Chip Growth K6 Fund, (vii) 124,978 shares of common stock underlying shares of Series B convertible preferred stock and 93,200 shares of common stock underlying shares of Series C convertible preferred stock held by WAVECHART + CO fbo Fidelity Securities Fund: Fidelity Series Blue Chip Growth Fund, (viii) 227 shares of common stock underlying shares of Series B convertible preferred stock and 573 shares of common stock underlying shares of Series C convertible preferred stock held by Booth & Co fbo Fidelity Securities Fund: Fidelity Flex Large Cap Growth Fund, (ix) 420,950 shares of common stock underlying shares of Series B convertible preferred stock held by M Gardiner & Co fbo Fidelity Puritan Trust: Fidelity Puritan Fund, (x) 56,581 shares of common stock underlying shares of Series B convertible preferred stock and 49,300 shares of common stock underlying shares of Series C convertible preferred stock held by M Gardiner &

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Co fbo Fidelity Central Investment Portfolios LLC: Fidelity Health Care Central Fund, (xi) 15,906 shares of common stock underlying shares of Series B convertible preferred stock and 14,400 shares of common stock underlying shares of Series C convertible preferred stock held by M Gardiner & Co fbo Variable Insurance Products Fund IV: Health Care Portfolio, (xii) 148,610 shares of common stock underlying shares of Series B convertible preferred stock and 124,500 shares of common stock underlying shares of Series C convertible preferred stock held by Mag & Co fbo Fidelity Select Portfolios: Health Care Portfolio, (xiii) 59,876 shares of common stock underlying shares of Series B convertible preferred stock and 71,927 shares of common stock underlying shares of Series C convertible preferred stock held by M Gardiner & Co fbo Fidelity Advisor Series VII: Fidelity Advisor Health Care Fund, (xiv) 2,761,570 shares of common stock underlying shares of Series B convertible preferred held by Mag & Co fbo Fidelity Contrafund: Fidelity Contrafund, (xv) 54,536 shares of common stock underlying shares of Series B convertible preferred stock held by Booth & Co FBO Fidelity Contrafund: Fidelity Contrafund K6, (xvi) 458,443 shares of common stock underlying shares of Series B convertible preferred stock held by Mag & Co fbo Fidelity Contrafund Commingled Pool, (xvii) 254,777 shares of common stock underlying shares of Series B convertible preferred stock and 175,600 shares of common stock underlying shares of Series C convertible preferred stock held by Mag & Co. fbo Fidelity Mt. Vernon Street Trust: Fidelity Series Growth Company Fund, (xviii) 13,200 shares of common stock underlying shares of Series C convertible preferred stock held by Mag & Co fbo Fidelity Select Portfolios: Pharmaceuticals Portfolio, (xix) 2,100 shares of common stock underlying shares of Series C convertible preferred stock held by Fidelity Blue Chip Growth Institutional Trust, and (xx) 36,600 shares of common stock underlying shares of Series C convertible preferred stock held by Fidelity Mt. Vernon Street Trust: Fidelity Growth Company K6 Fund. Fidelity Management & Research Company, or Fidelity, 82 Devonshire Street, Boston, Massachusetts 02109, a wholly-owned subsidiary of FMR LLC and an investment adviser registered under Section 203 of the Investment Advisers Act of 1940, is the beneficial owner of such shares of common stock as a result of acting as investment adviser to various investment companies registered under Section 8 of the Investment Company Act of 1940. Edward C. Johnson 3d and FMR LLC, through its control of Fidelity and the Fidelity Funds, each has sole power to dispose of the shares owned by the Fidelity Funds. Members of the family of Edward C. Johnson 3d, Chairman of FMR LLC, are the predominant owners, directly or through trusts, of Series B voting common shares of FMR LLC, representing 49% of the voting power of FMR LLC. The Johnson family group and all other Series B shareholders have entered into a shareholders' voting agreement under which all Series B voting common shares will be voted in accordance with the majority vote of Series B voting common shares. Accordingly, through their ownership of voting common shares and the execution of the shareholders' voting agreement, members of the Johnson family may be deemed, under the Investment Company Act of 1940, to form a controlling group with respect to FMR LLC. Neither FMR LLC nor Edward C. Johnson 3d, Chairman of FMR LLC, has the sole power to vote or direct the voting of the shares owned directly by the Fidelity Funds, which power resides with the Fidelity Funds' Boards of Trustees. Fidelity carries out the voting of the shares under written guidelines established by the Fidelity Funds' Boards of Trustees.

- (3) Consists of (i) 2,484,595 shares of common stock underlying shares of Series B convertible preferred stock held by Invus Public Equities, L.P. and (ii) 1,073,076 shares of common stock underlying shares of Series C convertible preferred stock held by Invus Public Equities, L.P. Invus Public Equities Advisors, LLC, as the general partner of Invus Public Equities, L.P., controls Invus Public Equities, L.P. and, accordingly, may be deemed to beneficially own the shares held by Invus Public Equities, L.P. Artal Treasury Ltd., as the managing member of Invus Public Equities Advisors, LLC, controls Invus Public Equities Advisors, LLC and, accordingly, may be deemed to beneficially own the shares held by Invus Public Equities, L.P. The Geneva branch of Artal International S.C.A. is the sole stockholder of Artal Treasury Ltd. and, accordingly, may be deemed to beneficially own the shares held by Invus Public Equities, L.P. Artal International Management S.A., as the managing partner of Artal International S.C.A., controls Artal International S.C.A. and, accordingly, may be deemed to beneficially own the shares held by Invus Public Equities, L.P. Artal Group, S.A., as the parent company of Artal International Management, S.A., controls Artal International Management S.A. and, accordingly, may be deemed to beneficially own the shares held by Invus Public Equities, L.P. Westend, S.A., as the parent company of Artal Group S.A., controls Artal Group S.A., and, accordingly, may be deemed to beneficially own the shares held by Invus Public Equities, L.P. Stichting Administratiekantoor Westend, as the parent company of Westend S.A., controls Westend S.A. and, accordingly, may be deemed to beneficially own the shares held by Invus Public Equities, L.P. Mr. Pascal Minne, as the sole member of the board of Stichting Administratiekantoor Westend, controls Stichting Administratiekantoor Westend and, accordingly, may be deemed to beneficially own the shares held by Invus Public Equities, L.P. The address for Invus Public Equities, L.P. is 750 Lexington Avenue, 30th Floor, New York, NY 10022.
- (4) Consists of shares of common stock underlying shares of Series C convertible preferred stock held by each of the following funds and accounts: (i) 1,430,119 shares of Series C convertible preferred stock held by T. Rowe Price Health Sciences Fund, Inc., (ii) 87,758 shares of Series C convertible preferred stock held by TD Mutual Funds – TD Health Sciences Fund, (iii) 85,638 shares of Series C convertible preferred stock held by VALIC Company I—Health Sciences Fund, (iv) 65,749 shares of Series C convertible preferred stock held by T. Rowe Price Health Sciences Portfolio, (v) 1,958,677 shares of Series C convertible preferred stock held by T. Rowe Price New Horizons Fund, Inc., (vi) 277,681 shares of Series C convertible preferred held by T. Rowe Price New Horizons Trust, (vii) 15,882 shares of Series C convertible preferred stock held by T. Rowe Price U.S. Equities Trust, (viii) 8,628 shares of Series C convertible preferred stock held by MassMutual Select Funds -MassMutual Select T. Rowe Price Small and Mid Cap Blend Fund, (ix) 719,502 shares of Series C convertible preferred stock held by T. Rowe Price Small-Cap Stock Fund, Inc., (x) 394,149 shares of Series C convertible preferred stock held by T. Rowe Price Institutional Small-Cap Stock Fund, (xi) 6,180 shares of Series C convertible preferred stock held by T. Rowe Price Spectrum Conservative Allocation Fund, (xii) 10,402 shares of Series C convertible preferred stock held by T. Rowe Price Spectrum Moderate Allocation Fund, (xiii) 16,769 shares of Series C convertible preferred stock held by T. Rowe Price Spectrum Moderate Growth Allocation Fund, (xiv) 757 shares of Series C convertible preferred stock held by T. Rowe Price Moderate Allocation Portfolio, (xv) 33,294 shares of Series C convertible preferred stock held by U.S. Small-Cap Stock Trust, (xvi) 7,499 shares of Series C convertible preferred stock held by VALIC Company I—Small Cap Fund, (xvii) 28,224 shares of Series C convertible preferred stock held by TD Mutual Funds—TD U.S. Small-Cap Equity Fund, (xviii) 167,896 shares of Series C convertible preferred stock held by T. Rowe Price U.S. Small-Cap Core Equity Trust, (xix) 8,186 shares of Series C convertible preferred stock held by Minnesota Life Insurance Company, (xx) 34,121 shares of Series C convertible preferred stock held by Costco 401(k) Retirement Plan, (xxi) 8,271 shares of Series C convertible preferred stock held by MassMutual Select Funds -MassMutual Select T. Rowe Price Small and Mid Cap Blend Fund. The foregoing accounts are advised or sub-advised by T. Rowe Price Associates, Inc., or "T. Rowe Price", a registered investment adviser. T. Rowe Price serves as investment adviser or subadviser, as applicable, with power to direct investments and/or sole power to vote the securities owned by the accounts (with the exception of one subadvisory fund that retains its own voting authority). Although T. Rowe Price may be deemed to be the beneficial owner of all the shares listed, T. Rowe Price expressly disclaims beneficial ownership of such securities. T. Rowe Price Investment Services, Inc., or TRPIS, a registered broker-dealer (and FINRA member), is a subsidiary of T. Rowe Price Associates, Inc., the investment adviser or subadviser, as applicable, to the accounts listed above. TRPIS was formed primarily for the limited purpose of acting as the principal underwriter and distributor of shares of the

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funds in the T. Rowe Price mutual fund family. TRPIS does not engage in underwriting or market-making activities involving individual securities. T. Rowe Price Associates, Inc. is the wholly-owned subsidiary of T. Rowe Price Group, Inc., which is a publicly traded financial services holding company. The principal address for T. Rowe Price is 100 East Pratt Street, Baltimore, Maryland 21202.

- (5) Consists of (i) 100,000 shares of common stock underlying shares of Series A convertible preferred stock and (ii) 75,000 shares of common stock held directly by Dr. Nicholson.
- (6) Consists of (i) 200,000 shares of common stock underlying shares of Series A convertible preferred stock and 2,412,500 shares of common stock owned by Geoff McDonough, M.D. and (ii) 400,000 shares of common stock owned by McDonough Family 2018 Irrevocable Trust, or the Trust. Dr. McDonough is the settlor of the Trust, Allison L. McDonough and David S. Grayzel are trustees for the Trust and the Trust is for the benefit of Graeme and Owen McDonough. Dr. McDonough may be deemed to beneficially own the shares of common stock owned by the Trust.
- (7) Consists of 576,800 shares of common stock and 109,300 shares of common stock issuable upon the exercise of options that are exercisable as of March 31, 2020 or will become exercisable within 60 days of such date.
- (8) Consists of 372,968 shares of common stock issuable upon the exercise of options that are exercisable as of March 31, 2020 or will become exercisable within 60 days of such date.
- (9) Consists of 100,000 shares of common stock underlying shares of Series A convertible preferred stock, 12,422 shares of common stock underlying shares of Series B convertible preferred stock, 44,711 shares of common stock underlying shares of Series C convertible preferred stock and 75,000 shares of common stock.
- (10) Consists of 53,654 shares of common stock underlying shares of Series C convertible preferred stock and 75,000 shares of common stock.
- (11) Consists of 89,423 shares of common stock underlying shares of Series C convertible preferred stock and 32,813 shares of common stock issuable upon the exercise of options that are exercisable as of March 31, 2020 or will become exercisable within 60 days of such date.
- (12) Consists of 8,942 shares of common stock underlying shares of Series C convertible preferred stock and 75,000 shares of common stock.
- (13) Consists of 32,813 shares of common stock issuable upon the exercise of options that are exercisable as of March 31, 2020 or will become exercisable within 60 days of such date.
- (14) Consists of 18,342,863 shares of common stock underlying shares of preferred stock, 9,415,300 shares of common stock and 566,344 shares of common stock issuable upon the exercise of options that are exercisable as of March 31, 2020 or will become exercisable within 60 days after such date.



## Description of capital stock

The following description of our capital stock and provisions of our certificate of incorporation and bylaws are summaries and are qualified by reference to the certificate of incorporation and bylaws that will become effective upon the closing of this offering. We will file copies of these documents with the SEC as exhibits to our registration statement of which this prospectus forms a part. The description of our common stock reflects changes to our capital structure that will occur upon the closing of this offering.

Upon the closing of this offering, our authorized capital stock will consist of \_\_\_\_\_ shares of our common stock, par value \$0.0001 per share, and \_\_\_\_\_ shares of our preferred stock, par value \$0.0001 per share, all of which preferred stock will be undesignated.

As of March 31, 2020, we had issued and outstanding:

- 12,383,404 shares of our common stock held by 37 stockholders of record;
- 15,451,020 shares of our Series A preferred stock held by four stockholders of record, convertible into 15,451,020 shares of our common stock;
- 10,974,644 shares of our Series B preferred stock held by 26 stockholders of record, convertible into 12,469,030 shares of our common stock; and
- 19,936,296 shares of our Series C preferred stock held by 54 stockholders of record, convertible into 19,936,296 shares of our common stock;

Upon the closing of this offering, all of the outstanding shares of our preferred stock will automatically convert into an aggregate of 47,856,346 shares of our common stock.

### Common stock

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. Each election of directors by our stockholders will be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights of outstanding preferred stock that we may designate and issue in the future.

In the event of our liquidation or dissolution, the holders of our common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any of our outstanding preferred stock. Holders of our common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of our common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of our preferred stock that we may designate and issue in the future.

### Preferred stock

As of March 31, 2020, we had issued and outstanding 15,451,020 shares of Series A preferred stock, 10,974,644 shares of Series B preferred stock and 19,936,296 shares of Series C preferred stock. Upon the closing of this offering, all of the outstanding shares of our preferred stock will automatically convert into an aggregate of 47,856,346 shares of our common stock. Under the terms of our certificate of incorporation that will become effective upon the closing of this offering, our board of directors is authorized to issue shares of preferred stock

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in one or more series without stockholder approval. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock. Upon the closing of this offering, there will be no shares of preferred stock outstanding, and we have no present plans to issue any shares of preferred stock.

### **Options and unvested restricted common stock**

As of March 31, 2020, options to purchase an aggregate of 8,894,975 shares of our common stock were outstanding, at a weighted average exercise price of \$2.77 per share, and 2,543,922 shares of unvested restricted common stock were outstanding.

### **Registration rights**

We have entered into an amended and restated investors' rights agreement dated as of January 9, 2020, or the investors' rights agreement, with holders of our preferred stock. Beginning six months following the closing of this offering, holders of a total of 56,322,971 shares of our common stock will have the right to require us to register these shares under the Securities Act under specified circumstances. We refer to the shares with these registration rights as registrable securities. After registration pursuant to these rights, the registrable securities will become freely tradable without restriction under the Securities Act.

#### ***Demand registration rights***

Beginning 180 days after the effective date of the registration statement of which this prospectus is a part, subject to specified limitations set forth in the investors' rights agreement, at any time, the holders of a majority of the then outstanding registrable securities may demand that we register at least 40% of the registrable securities then outstanding under the Securities Act for purposes of a public offering.

In addition, subject to specified limitations set forth in the investors' rights agreement, at any time after we become eligible to file a registration statement on Form S-3, certain holders of at least 20% of the registrable securities then outstanding may request that we register their registrable securities on Form S-3 for purposes of a public offering for which the anticipated aggregate offering price to the public would exceed, net of selling expenses, \$5.0 million.

We shall use our commercially reasonable efforts to cause such registration statements to become effective.

#### ***Incidental registration rights***

If, at any time after the closing of this offering, we propose to register for our own account any of our securities under the Securities Act, the holders of registrable securities will be entitled to notice of the registration and, subject to specified exceptions, have the right to require us to register all or a portion of the registrable securities then held by them in that registration. We have the right to terminate or withdraw any registration initiated by us before the effective date of such registration.

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In the event that any registration in which the holders of registrable securities participate pursuant to our investors' rights agreement is an underwritten public offering, we have agreed to enter into an underwriting agreement in usual and customary form.

### **Expenses**

Pursuant to the investors' rights agreement, we are required to pay all registration expenses, including all registration, filing and qualification fees; printing and accounting fees; reasonable fees and disbursements not to exceed \$25,000 of one counsel representing the selling stockholders or, in the event of an initial public offering, of one counsel representing the investors; but excluding underwriting discounts, selling commissions and stock transfer taxes applicable to the sale of registrable securities and the fees and expenses of the selling stockholders' own counsel (other than the counsel selected to represent all selling stockholders).

The investors' rights agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the registration statement attributable to us or any violation or alleged violation whether by action or inaction by us under the Securities Act, the Exchange Act, any state securities or Blue Sky law or any rule or regulation promulgated under the Securities Act, the Exchange Act or any state securities or Blue Sky law in connection with such registration statement or the qualification or compliance of the offering, and they are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them.

### **Delaware anti-takeover law and certain charter and bylaw provisions**

#### ***Delaware law***

We are subject to Section 203 of the Delaware General Corporation Law, or DGCL. Subject to certain exceptions, Section 203 prevents a publicly held Delaware corporation from engaging in a "business combination" with any "interested stockholder" for three years following the date that the person became an interested stockholder, unless either the interested stockholder attained such status with the approval of our board of directors, the business combination is approved by our board of directors and stockholders in a prescribed manner or the interested stockholder acquired at least 85% of our outstanding voting stock in the transaction in which it became an interested stockholder. A "business combination" includes, among other things, a merger or consolidation involving us and the "interested stockholder" and the sale of more than 10% of our assets. In general, an "interested stockholder" is any entity or person beneficially owning 15% or more of our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person. The restrictions contained in Section 203 are not applicable to any of our existing stockholders that will own 15% or more of our outstanding voting stock upon the closing of this offering.

#### ***Staggered board; removal of directors***

Our certificate of incorporation and our bylaws to be effective upon the closing of this offering divide our board of directors into three classes with staggered three-year terms. In addition, our certificate of incorporation and our bylaws to be effective upon the closing of this offering provide that directors may be removed only for cause and only by the affirmative vote of the holders of at least 75% of our shares of capital stock present in person or by proxy and entitled to vote. Under our certificate of incorporation and our bylaws to be effective upon the closing of this offering, any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

Furthermore, our certificate of incorporation to be effective upon the closing of this offering provides that the authorized number of directors may be changed only by the resolution of our board of directors. The

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classification of our board of directors and the limitations on the ability of our stockholders to remove directors, change the authorized number of directors and fill vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

### ***Super-majority voting***

The DGCL provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our bylaws to be effective upon the closing of this offering may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes that all our stockholders would be entitled to cast in any election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate of incorporation described above.

### ***Stockholder action; special meeting of stockholders; advance notice requirements for stockholder proposals and director nominations***

Our certificate of incorporation and our bylaws to be effective upon the closing of this offering provide that any action required or permitted to be taken by our stockholders at an annual meeting or special meeting of stockholders may only be taken if it is properly brought before such meeting and may not be taken by written action in lieu of a meeting. Our certificate of incorporation and our bylaws to be effective upon the closing of this offering also provide that, except as otherwise required by law, special meetings of the stockholders can only be called by our board of directors. In addition, our bylaws to be effective upon the closing of this offering establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of candidates for election to our board of directors. Stockholders at an annual meeting may only consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors, or by a stockholder of record on the record date for the meeting who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities. These provisions also could discourage a third party from making a tender offer for our common stock because even if the third party acquired a majority of our outstanding voting stock, it would be able to take action as a stockholder, such as electing new directors or approving a merger, only at a duly called stockholders meeting and not by written consent.

### ***Exclusive forum provision***

Our certificate of incorporation to be effective upon the closing of this offering provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery of the State of Delaware does not have jurisdiction, the federal district court for the District of Delaware) shall be the sole and exclusive forum for the following types of proceedings: (1) any derivative action or proceeding brought on behalf of our company, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers, employees or stockholders to our company or our stockholders, (3) any action asserting a claim arising pursuant to any provision of the General Corporation Law of the State of Delaware or as to which the General Corporation Law of the State of Delaware confers jurisdiction on the Court of Chancery of the State of Delaware, or (4) any action asserting a claim arising pursuant to any provision of our certificate of incorporation or bylaws (in each case, as they may be amended from time to time) or governed by

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the internal affairs doctrine. These choice of forum provisions will not apply to suits brought to enforce a duty or liability created by the Securities Act, the Exchange Act or any other claim for which federal courts have exclusive jurisdiction. Although our certificate of incorporation contains the choice of forum provisions described above, it is possible that a court could rule that such provisions are inapplicable for a particular claim or action or that such provisions are unenforceable.

### **Transfer agent and registrar**

The transfer agent and registrar for our common stock will be \_\_\_\_\_.

### **Nasdaq Global Market**

We intend to apply to have our common stock listed on the Nasdaq Global Market under the symbol "GBIO."

### **Shares eligible for future sale**

Prior to this offering, there has been no public market for our common stock, and a liquid trading market for our common stock may not develop or be sustained after this offering. As described below, only a limited number of shares will be available for sale shortly after this offering due to contractual and legal restrictions on resale. Future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options, or the anticipation of these sales, could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through sales of equity securities.

Upon the closing of this offering, we will have outstanding \_\_\_\_\_ shares of our common stock, based on the \_\_\_\_\_ shares of our common stock that were outstanding on \_\_\_\_\_, including \_\_\_\_\_ shares of unvested restricted stock subject to repurchase by us, and after giving effect to the issuance of \_\_\_\_\_ shares of our common stock in this offering, assuming no exercise by the underwriters of their option to purchase additional shares of our common stock and the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 47,856,346 shares of our common stock upon the closing of this offering. Of these shares, all shares sold in this offering will be freely tradable without restriction or further registration under the Securities Act of 1933, as amended, or the Securities Act, unless purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act.

The remaining \_\_\_\_\_ shares of our common stock will be "restricted securities" under Rule 144, and we expect that substantially all of these restricted securities will be subject to the 180-day lock-up period under the lock-up agreements as described below. These restricted securities may be sold in the public market upon release or waiver of any applicable lock-up agreements and only if registered or pursuant to an exemption from registration, such as Rule 144 or Rule 701 under the Securities Act.

### **Lock-up agreements**

We and each of our directors and executive officers and holders of substantially all of our outstanding securities have agreed that, without the prior written consent of J.P. Morgan Securities LLC and Jefferies LLC, on behalf of the underwriters, we and they will not, subject to limited exceptions, during the period ending 180 days after the date of this prospectus, subject to extension in specified circumstances:

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for common stock, or make any public announcement of an intention to do any of the foregoing; or

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- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of our common stock, whether any transaction described above is to be settled by delivery of our common stock or such other securities, in cash or otherwise.

These agreements are subject to certain exceptions, as described in the section of this prospectus entitled “Underwriting.”

### **Rule 144**

In general, under Rule 144 of the Securities Act, beginning 90 days after the date of this prospectus, any person who is not our affiliate and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell those shares without restriction, subject to the availability of current public information about us. In addition, under Rule 144, any person who is not our affiliate and has not been our affiliate at any time during the preceding three months and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available.

Beginning 90 days after the date of this prospectus, a person who is our affiliate or who was our affiliate at any time during the preceding three months and who has beneficially owned restricted securities for at least six months, including the holding period of any prior owner other than one of our affiliates, is entitled to sell a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately \_\_\_\_\_ shares immediately after this offering; and
- the average weekly trading volume in our common stock on the Nasdaq Global Market during the four calendar weeks preceding the date of filing of a Notice of Proposed Sale of Securities Pursuant to Rule 144 with respect to the sale.

Sales under Rule 144 by our affiliates are also subject to manner of sale provisions and notice requirements and to the availability of current public information about us.

Upon waiver or expiration of the 180-day lock-up period described below, approximately \_\_\_\_\_ shares of our common stock will be eligible for sale under Rule 144. We cannot estimate the number of shares of our common stock that our existing stockholders will elect to sell under Rule 144.

### **Rule 701**

In general, under Rule 701 of the Securities Act, any of our employees, consultants or advisors, other than our affiliates, who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement is eligible to resell these shares 90 days after the date of this prospectus in reliance on Rule 144, but without compliance with the various restrictions, including the availability of public information about us, holding period and volume limitations, contained in Rule 144. Subject to the 180-day lock-up period described above, approximately \_\_\_\_\_ shares of our common stock, based on shares outstanding as of \_\_\_\_\_, 2020 will be eligible for sale in accordance with Rule 701.

### **Stock options and form S-8 registration statement**

Following this offering, we intend to file one or more registration statements on Form S-8 under the Securities Act to register all of the shares of our common stock subject to outstanding awards and reserved for future

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issuance under the 2017 Plan, the 2020 Plan and the 2020 ESPP. See “Executive compensation—Stock option and other compensation plans” for additional information regarding these plans. Accordingly, shares of our common stock registered under the registration statements will be available for sale in the open market, subject to Rule 144 volume limitations applicable to affiliates, and subject to any vesting restrictions and lock-up agreements applicable to these shares.

***Registration rights***

Upon the closing of this offering, the holders of \_\_\_\_\_ shares of common stock will have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act. See “Description of capital stock—Registration rights” for additional information regarding these registration rights.

## Material U.S. tax considerations for non-U.S. holders of common stock

The following is a discussion of material U.S. federal income and estate tax considerations relating to ownership and disposition of our common stock by a non-U.S. holder. For purposes of this discussion, the term “non-U.S. holder” means a beneficial owner (other than a partnership or other pass-through entity) of our common stock that is not, for U.S. federal income tax purposes:

- an individual who is a citizen or resident of the United States;
- a corporation, or other entity treated as a corporation for U.S. federal income tax purposes, created or organized in or under the laws of the United States, any state thereof or the District of Columbia;
- an estate the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust, if a U.S. court is able to exercise primary supervision over the administration of the trust and one or more U.S. persons have authority to control all substantial decisions of the trust or if the trust has a valid election in effect to be treated as a U.S. person under applicable U.S. Treasury Regulations.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, or the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, as in effect as of the date of this prospectus, all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any change or different interpretation could alter the tax consequences to non-U.S. holders described in this prospectus. In addition, there can be no assurance that the Internal Revenue Service, or the IRS, will not challenge one or more of the tax consequences described in this prospectus.

This discussion addresses only non-U.S. holders that hold shares of our common stock as a capital asset (generally, property held for investment). This discussion does not address all aspects of U.S. federal income and estate taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder’s individual circumstances nor does it address the alternative minimum tax, the Medicare tax on net investment income or any aspects of U.S. state, local or non-U.S. taxes. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- insurance companies;
- tax-exempt organizations;
- financial institutions;
- brokers or dealers in securities;
- pension plans;
- controlled foreign corporations;
- passive foreign investment companies;
- owners that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment; and
- certain U.S. expatriates.



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In addition, this discussion does not address the tax treatment of partnerships or persons who hold their common stock through partnerships or other entities that are pass-through entities for U.S. federal income tax purposes. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its own tax advisor regarding the tax consequences of the purchase, ownership and disposition of our common stock through a partnership or other pass-through entity, as applicable.

**Prospective investors should consult their own tax advisors regarding the U.S. federal, state, local and non-U.S. income and other tax considerations of acquiring, holding and disposing of our common stock.**

## **Dividends**

If we pay distributions on our common stock, those distributions generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such non-U.S. holder's tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below under the heading "—Gain on disposition of common stock."

Dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence. A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such non-U.S. holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) and satisfy applicable certification and other requirements. A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim with the IRS. Non-U.S. holders are urged to consult their own tax advisors regarding their entitlement to benefits under a relevant income tax treaty.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States and, if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income is taxed on a net income basis at the same U.S. federal income tax rates applicable to United States persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such non-U.S. holder's country of residence.

## **Gain on disposition of common stock**

A non-U.S. holder generally will not be subject to U.S. federal income tax on gain recognized on a disposition of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business in the United States and, if an applicable income tax treaty so provides, the gain is attributable to a permanent establishment or fixed base maintained by the non-U.S. holder in the United States; in these cases, the non-U.S. holder will be taxed on a net income basis at the same U.S. federal income tax rates applicable to United States persons (as

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defined in the Code), and if the non-U.S. holder is a foreign corporation, an additional branch profits tax at a 30% rate, or such lower rate as may be specified by an applicable income tax treaty, may also apply;

- the non-U.S. holder is a nonresident alien present in the United States for 183 days or more in the taxable year of the disposition and certain other requirements are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty) on the net gain derived from the disposition, which may be offset by U.S.-source capital losses of the non-U.S. holder, if any; or
- we are, or have been at any time during the five-year period preceding such disposition (or the non-U.S. holder's holding period, if shorter), a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder held no more than 5% of our outstanding common stock, directly or indirectly, during the shorter of the five year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. If we are determined to be a U.S. real property holding corporation and the foregoing exception does not apply, then the non-U.S. holder generally will be taxed on its net gain derived from the disposition at the income tax rates applicable to United States persons (as defined in the Code). Generally, a corporation is a "U.S. real property holding corporation" if the fair market value of its "U.S. real property interests" equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business. Although there can be no assurance, we believe that we are not currently, and we do not anticipate becoming, a "U.S. real property holding corporation" for U.S. federal income tax purposes. No assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rule described above.

## **Information reporting and backup withholding**

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the non-U.S. holder is not a U.S. person (as defined in the Code) in order to avoid backup withholding at the applicable rate, with respect to dividends on our common stock. Generally, a non-U.S. holder will comply with such procedures if it provides a properly executed IRS Form W-8BEN or W-8BEN-E (or other applicable Form W-8) or otherwise meets documentary evidence requirements for establishing that it is a non-U.S. holder, or otherwise establishes an exemption. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above under the heading "—Dividends," will generally be exempt from U.S. backup withholding.

Information reporting and backup withholding generally will apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the non-U.S. holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their own tax advisors regarding the application of the information reporting and backup withholding rules to them.

Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement.

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Backup withholding is not an additional tax. Rather, any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is timely filed with the IRS.

### **FATCA**

Provisions of the Code commonly referred to as the Foreign Account Tax Compliance Act, or FATCA, generally impose a 30% withholding tax on dividends on, and gross proceeds from the sale or other disposition of, our common stock if paid to a foreign entity unless (1) if the foreign entity is a "foreign financial institution," the foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (2) if the foreign entity is not a "foreign financial institution," the foreign entity identifies certain of its U.S. investors, or (3) the foreign entity is otherwise excepted under FATCA.

Withholding under FATCA generally applies to payments of dividends on our common stock. While withholding under FATCA may apply to payments of gross proceeds from a sale or other disposition of our common stock, under recently proposed U.S. Treasury Regulations, withholding on payments of gross proceeds is not required. Although such regulations are not final, applicable withholding agents may rely on the proposed regulations until final regulations are issued.

If withholding under FATCA is required on any payment related to our common stock, investors not otherwise subject to withholding (or that otherwise would be entitled to a reduced rate of withholding) on such payment may be able to seek a refund or credit from the IRS. An intergovernmental agreement between the United States and an applicable foreign country may modify the requirements described in this section. Non-U.S. holders should consult their own tax advisors regarding the possible implications of FATCA on their investment in our common stock and the entities through which they hold our common stock.

### **Federal estate tax**

Common stock owned or treated as owned by an individual who is a non-U.S. holder (as specially defined for U.S. federal estate tax purposes) at the time of death will be included in the individual's gross estate for U.S. federal estate tax purposes and, therefore, may be subject to U.S. federal estate tax, unless an applicable estate tax or other treaty provides otherwise.

**The preceding discussion of material U.S. federal tax considerations is for prospective investors' information only. It is not tax advice. Prospective investors should consult their own tax advisors regarding the particular U.S. federal, state, local and non-U.S. tax consequences of purchasing, holding and disposing of our common stock, including the consequences of any proposed changes in applicable laws.**

## Underwriting

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC, Jefferies LLC and Cowen and Company, LLC are acting as joint book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the underwriters. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

| Name                       | Number of shares |
|----------------------------|------------------|
| J.P. Morgan Securities LLC |                  |
| Jefferies LLC              |                  |
| Cowen and Company, LLC     |                  |
| Wedbush Securities Inc.    |                  |
| <b>Total</b>               |                  |

The underwriters are committed to purchase all the common shares offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common shares directly to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of \$        per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to \$        per share from the initial public offering price. After the initial offering of the shares to the public, if all of the common shares are not sold at the initial public offering price, the underwriters may change the offering price and the other selling terms. Sales of any shares made outside of the United States may be made by affiliates of the underwriters.

The underwriters have an option to buy up to        additional shares of common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is \$        per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

|           | Without option to purchase additional shares exercise | With full option to purchase additional shares exercise |
|-----------|---|---|
| Per Share | \$  | \$  |
| Total     | \$  | \$  |

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We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately \$ .

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in the offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, or submit to, or file with, the Securities and Exchange Commission a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exercisable or exchangeable for any shares of our common stock, or publicly disclose the intention to undertake any of the foregoing, or (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of the ownership of any shares of common stock or any such other securities (whether any such transaction described in clause (1) or (2) above is to be settled by delivery of shares of common stock or such other securities, in cash or otherwise), without the prior written consent of J.P. Morgan Securities LLC and Jefferies LLC for a period of 180 days after the date of this prospectus, other than the shares of our common stock to be sold in this offering.

The restrictions on our actions, as described above, do not apply to certain transactions, including (1) the issuance of shares of common stock or securities convertible into or exercisable for shares of our common stock pursuant to the conversion or exchange of convertible or exchangeable securities or the exercise of warrants or options (including net exercise) or the settlement of RSUs (including net settlement), in each case outstanding on the date of the underwriting agreement and described in this prospectus; (2) grants of stock options, stock awards, restricted stock, RSUs or other equity awards and the issuance of shares of our common stock or securities convertible into or exercisable or exchangeable for shares of our common stock (whether upon the exercise of stock options or otherwise) to our employees, officers, directors, advisors or consultants pursuant to the terms of an equity compensation plan in effect as of the closing of this offering and described in this prospectus, provided that such recipients enter into a lock-up agreement with the underwriters; or (3) our filing of any registration statement on Form S-8 relating to securities granted or to be granted pursuant to any plan in effect on the date of the underwriting agreement and described in this prospectus or any assumed benefit plan pursuant to an acquisition or similar strategic transaction.

Our directors and executive officers, and substantially all of our shareholders, which we refer to as the lock-up parties, have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each lock-up party, with limited exceptions, for a period of 180 days after the date of this prospectus, or the restricted period, may not (and may not cause any of their direct or indirect affiliates to), without the prior written consent of J.P. Morgan Securities LLC and Jefferies LLC, (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such lock-up parties in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant (collectively with the common stock, the "lock-up securities")), (2) enter into any hedging, swap or other agreement or transaction that transfers, in whole or in part, any of the economic consequences of ownership of the lock-up securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of lock-up securities, in cash or otherwise,

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(3) make any demand for, or exercise any right with respect to, the registration of any lock-up securities, or (4) publicly disclose the intention to do any of the foregoing. Such persons or entities have further acknowledged that these undertakings preclude them from engaging in any hedging or other transactions or arrangements (including, without limitation, any short sale or the purchase or sale of, or entry into, any put or call option, or combination thereof, forward, swap or any other derivative transaction or instrument, however described or defined) designed or intended, or which could reasonably be expected to lead to or result in, a sale or disposition or transfer (by any person or entity, whether or not a signatory to such agreement) of any economic consequences of ownership, in whole or in part, directly or indirectly, of any lock-up securities, whether any such transaction or arrangement (or instrument provided for thereunder) would be settled by delivery of lock-up securities, in cash or otherwise.

The restrictions described in the immediately preceding paragraph and contained in the lock-up agreements between the underwriters and the lock-up parties do not apply, subject in certain cases to various conditions, to certain transactions, including (a) transfers or dispositions of lock-up securities: (1) as bona fide gifts, or for bona fide estate planning purposes, (2) by will, other testamentary document or intestate succession, (3) to any trust for the direct or indirect benefit of the lock-up party or any immediate family member, (4) to a corporation, partnership, limited liability company, trust or other entity of which the lock-up party and/or one or more members of its immediate family members are the legal and beneficial owner of all of the outstanding equity securities or similar interests, (5) to a nominee or custodian of a person or entity to whom a disposition or transfer would be permissible under clauses (1) through (4), (6) in the case of a corporation, partnership, limited liability company, trust or other entity, (A) to another corporation, partnership, limited liability company, trust or other entity that is an affiliate of the lock-up party, or to any investment fund or other entity controlling, controlled by, managing or managed by or under common control with the lock-up party or its affiliates or (B) as part of a distribution or other transfer or distribution to general or limited partners, members or stockholders of, or other holders of equity interests in, the lock-up party, (7) by operation of law, (8) to us from an employee or other service provider upon death, disability or termination of employment of such employee or service provider, (9) as part of a sale of lock-up securities acquired in this offering (other than, in the case of one of our officers or directors, any securities such officer or director may purchase in this offering) or in open market transactions after the completion of this offering, (10) to us in connection with the vesting, settlement or exercise of restricted stock units, options, warrants or other rights to purchase shares of our common stock (including "net" or "cashless" exercise), including for the payment of exercise price and tax and remittance payments, (11) pursuant to a bona fide third-party tender offer, merger, consolidation or other similar transaction made to all shareholders involving a change in control, provided that if such transaction is not completed, all such lock-up securities would remain subject to the restrictions in the immediately preceding paragraph, or (12) prior to the first public filing of the registration statement filed in connection with this prospectus; (b) exercise of the options, settlement of RSUs or other equity awards, or the exercise of warrants granted pursuant to plans or other equity compensation arrangements described in in this prospectus, provided that any lock-up securities received upon such exercise, vesting or settlement would be subject to restrictions similar to those in the immediately preceding paragraph; (c) exercise outstanding warrants and convert outstanding convertible securities of our or any securities received upon any such exercise or conversion, provided that any common stock or warrant received upon such exercise or conversion would be subject to restrictions similar to those in the immediately preceding paragraph; and (d) the establishment by lock-up parties of one or more trading plans under Rule 10b5-1 under the Exchange Act, provided that (1) such plan does not provide for the transfer or disposition of lock-up securities during the restricted period and (2) no filing by any party under the Exchange Act or other public announcement is required or made voluntarily in connection with such trading plan.

J.P. Morgan Securities LLC and Jefferies LLC, in their sole discretion, may release the securities subject to any of the lock-up agreements with the underwriters described above, in whole or in part at any time.

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We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act of 1933.

We will apply to have our common stock approved for listing/quotation on the Nasdaq Global Market under the symbol "GBIO."

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be "covered" shorts, which are short positions in an amount not greater than the underwriters' option to purchase additional shares referred to above, or may be "naked" shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act of 1933, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on the Nasdaq Global Market, in the over-the-counter market or otherwise.

Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;

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- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common shares, or that the shares will trade in the public market at or above the initial public offering price.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

Certain of the underwriters and their affiliates have provided in the past to us and our affiliates and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and such affiliates in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their affiliates may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

### **Notice to prospective investors in Canada**

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

### **Notice to prospective investors in the European Economic Area and United Kingdom**

In relation to each Member State of the European Economic Area and the United Kingdom (each a "Relevant State"), no shares have been offered or will be offered pursuant to this offering to the public in that Relevant



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State prior to the publication of a prospectus in relation to the shares which has been approved by the competent authority in that Relevant State or, where appropriate, approved in another Relevant State and notified to the competent authority in that Relevant State, all in accordance with the Prospectus Regulation, except that offers of shares may be made to the public in that Relevant State at any time under the following exemptions under the Prospectus Regulation:

- (a) to any legal entity which is a qualified investor as defined under the Prospectus Regulation;
- (b) to fewer than 150 natural or legal persons (other than qualified investors as defined under the Prospectus Regulation), subject to obtaining the prior consent of the underwriters; or
- (c) in any other circumstances falling within Article 1(4) of the Prospectus Regulation,

*provided* that no such offer of shares shall require us or any underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Regulation or supplement a prospectus pursuant to Article 23 of the Prospectus Regulation and each person who initially acquires any shares or to whom any offer is made will be deemed to have represented, acknowledged and agreed to and with each of the underwriters and the Company that it is a “qualified investor” within the meaning of Article 2(e) of the Prospectus Regulation. In the case of any shares being offered to a financial intermediary as that term is used in the Prospectus Regulation, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares to the public other than their offer or resale in a Relevant State to qualified investors as so defined or in circumstances in which the prior consent of the underwriters have been obtained to each such proposed offer or resale.

For the purposes of this provision, the expression an “offer to the public” in relation to shares in any Relevant State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares to be offered so as to enable an investor to decide to purchase or subscribe for any shares, and the expression “Prospectus Regulation” means Regulation (EU) 2017/1129.

### **Notice to prospective investors in the United Kingdom**

In addition, in the United Kingdom, this document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are “qualified investors” (as defined in the Prospectus Regulation) (1) who have professional experience in matters relating to investments falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended (the “Order”) and/or (2) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49(2)(a) to (d) of the Order (all such persons together being referred to as “relevant persons”) or otherwise in circumstances which have not resulted and will not result in an offer to the public of the shares in the United Kingdom within the meaning of the Financial Services and Markets Act 2000.

Any person in the United Kingdom that is not a relevant person should not act or rely on the information included in this document or use it as basis for taking any action. In the United Kingdom, any investment or investment activity that this document relates to may be made or taken exclusively by relevant persons.

### **Notice to prospective investors in Switzerland**

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document does not constitute a prospectus within the meaning of, and has been prepared without regard to the disclosure standards for

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issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document nor any other offering or marketing material relating to the shares or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this document nor any other offering or marketing material relating to the offering, the Company, the shares have been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA (FINMA), and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

### **Notice to prospective investors in Japan**

The shares have not been and will not be registered pursuant to Article 4, Paragraph 1 of the Financial Instruments and Exchange Act. Accordingly, none of the shares nor any interest therein may be offered or sold, directly or indirectly, in Japan or to, or for the benefit of, any “resident” of Japan (which term as used herein means any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to or for the benefit of a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Act and any other applicable laws, regulations and ministerial guidelines of Japan in effect at the relevant time.

### **Notice to prospective investors in Hong Kong**

The shares have not been offered or sold and will not be offered or sold in Hong Kong, by means of any document, other than (a) to “professional investors” as defined in the Securities and Futures Ordinance (Cap. 571 of the Laws of Hong Kong), or the SFO, of Hong Kong and any rules made thereunder; or (b) in other circumstances which do not result in the document being a “prospectus” as defined in the Companies (Winding Up and Miscellaneous Provisions) Ordinance (Cap. 32) of Hong Kong, or the CO, or which do not constitute an offer to the public within the meaning of the CO. No advertisement, invitation or document relating to the shares has been or may be issued or has been or may be in the possession of any person for the purposes of issue, whether in Hong Kong or elsewhere, which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted to do so under the securities laws of Hong Kong) other than with respect to shares which are or are intended to be disposed of only to persons outside Hong Kong or only to “professional investors” as defined in the SFO and any rules made thereunder.

### **Notice to prospective investors in Singapore**

Each representative has acknowledged that this prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, each representative has represented and agreed that it has not offered or sold any shares or caused the shares to be made the subject of an invitation for subscription or purchase and will not offer or sell any shares or cause the shares to be made the subject of an invitation for subscription or purchase, and has not circulated or distributed, nor will it circulate or distribute, this prospectus or any other document or material in connection with the offer or sale or invitation for subscription or purchase, of the shares, whether directly or indirectly, to any person in Singapore other than:

- (a) to an institutional investor (as defined in Section 4A of the Securities and Futures Act (Chapter 289) of Singapore, as modified or amended from time to time, or the SFA) pursuant to Section 274 of the SFA;

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- (b) to a relevant person (as defined in Section 275(2) of the SFA) pursuant to Section 275(1) of the SFA, or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions specified in Section 275 of the SFA; or
- (c) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

- (a) a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or
- (b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor, securities or securities-based derivatives contracts (each term as defined in Section 2(1) of the SFA) of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares pursuant to an offer made under Section 275 of the SFA except:
  - (i) to an institutional investor or to a relevant person, or to any person arising from an offer referred to in Section 275(1A) or Section 276(4)(i)(B) of the SFA;
  - (ii) where no consideration is or will be given for the transfer;
  - (iii) where the transfer is by operation of law;
  - (iv) as specified in Section 276(7) of the SFA; or
  - (v) as specified in Regulation 37A of the Securities and Futures (Offers of Investments) (Securities and Securities-based Derivatives Contracts) Regulations 2018.

## **Notice to prospective investors in the United Arab Emirates**

The shares have not been, and are not being, publicly offered, sold, promoted or advertised in the United Arab Emirates (including the Dubai International Financial Centre) other than in compliance with the laws of the United Arab Emirates (and the Dubai International Financial Centre) governing the issue, offering and sale of securities. Further, this prospectus does not constitute a public offer of securities in the United Arab Emirates (including the Dubai International Financial Centre) and is not intended to be a public offer. This prospectus has not been approved by or filed with the Central Bank of the United Arab Emirates, the Securities and Commodities Authority or the Dubai Financial Services Authority.

## **Legal matters**

The validity of the shares of common stock offered hereby is being passed upon for us by Wilmer Cutler Pickering Hale and Dorr LLP, Boston, Massachusetts. Davis Polk & Wardwell LLP, New York, New York, has acted as counsel for the underwriters in connection with certain legal matters related to this offering.

## **Experts**

The consolidated financial statements of Generation Bio Co. at December 31, 2018 and 2019, and for each of the two years in the period ended December 31, 2019, appearing in this prospectus and registration statement have

been audited by Ernst & Young LLP, independent registered public accounting firm, as set forth in their report thereon appearing elsewhere herein, and are included in reliance upon such report given on the authority of such firm as experts in accounting and auditing.

### **Where you can find more information**

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of common stock we are offering to sell. This prospectus, which constitutes part of the registration statement, does not include all of the information contained in the registration statement or the exhibits, schedules and amendments to the registration statement. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits and schedules to the registration statement. Statements contained in this prospectus about the contents of any contract, agreement or other document are not necessarily complete, and in each instance, we refer you to the copy of the contract, agreement or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference to such contract, agreement or document.

Upon completion of this offering, we will be subject to the informational requirements of the Exchange Act and will file annual, quarterly and current reports, proxy statements and other information with the SEC. You can read our SEC filings, including the registration statement, at the SEC's website at [www.sec.gov](http://www.sec.gov). We also maintain a website at [www.generationbio.com](http://www.generationbio.com) and upon completion of the offering, you may access, free of charge, our annual reports on Form 10-K, quarterly reports on Form 10-Q, current reports on Form 8-K and any amendments to those reports, as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. The information contained on, or that can be accessed through, our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

# Index to consolidated financial statements

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# Report of independent registered public accounting firm

To the Stockholders and the Board of Directors of Generation Bio Co.

## Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Generation Bio Co. (the Company) as of December 31, 2018 and 2019, the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholder's deficit, and cash flows for each of the two years in the period ended December 31, 2019, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2018 and 2019, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2019, in conformity with U.S. generally accepted accounting principles.

## Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB and in accordance with auditing standards generally accepted in the United States of America. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2018.

Boston, Massachusetts  
April 9, 2020

# Generation Bio Co.

## Consolidated balance sheets

| (In thousands, except share and per share amounts)   | December 31, |           |
|--|--------------|-----------|
|  | 2018         | 2019      |
| <b>Assets</b>  |              |           |
| Current assets:  |              |           |
| Cash and cash equivalents  | \$ 7,305     | \$ 15,076 |
| Marketable securities  | 67,565       | —         |
| Tenant receivable  | 1,288        | 448       |
| Prepaid expenses and other current assets  | 714          | 2,577     |
| Restricted cash  | 54           | 55        |
| Total current assets   | 76,926       | 18,156    |
| Property and equipment, net  | 4,486        | 21,845    |
| Restricted cash, noncurrent  | 2,107        | 2,052     |
| Deferred offering costs  | —            | 87        |
| Total assets   | \$ 83,519    | \$ 42,140 |
| <b>Liabilities, convertible preferred stock and stockholders' deficit</b>  |              |           |
| Current liabilities:   |              |           |
| Accounts payable   | \$ 1,681     | \$ 2,251  |
| Accrued expenses and other current liabilities   | 4,874        | 6,907     |
| Total current liabilities  | 6,555        | 9,158     |
| Deferred rent, net of current portion  | 2,962        | 15,981    |
| Total liabilities  | 9,517        | 25,139    |
| Commitments and contingencies (Note 11)  |              |           |
| Convertible preferred stock (Series A and B), \$0.0001 par value; 26,425,664 shares authorized at December 31, 2018 and 2019; 26,425,664 shares issued and outstanding at December 31, 2018 and 2019; liquidation preference of \$115,822 at December 31, 2019 | 115,593      | 115,593   |
| Stockholders' deficit:   |              |           |
| Common stock, \$0.0001 par value; 46,750,000 shares authorized at December 31, 2018 and 2019; 12,142,331 and 12,321,881 shares issued and 7,161,448 and 9,310,006 shares outstanding at December 31, 2018 and 2019, respectively                               | 1            | 1         |
| Additional paid-in capital   | 5,552        | 9,859     |
| Accumulated other comprehensive loss   | (9)          | —         |
| Accumulated deficit  | (47,135)     | (108,452) |
| Total stockholders' deficit  | (41,591)     | (98,592)  |
| Total liabilities, convertible preferred stock and stockholders' deficit   | \$ 83,519    | \$ 42,140 |

The accompanying notes are an integral part of these consolidated financial statements.

# Generation Bio Co.

## Consolidated statements of operations and comprehensive loss

| (In thousands, except share and per share amounts)  | Year ended December 31, |             |
|---|-------------------------|-------------|
|   | 2018                    | 2019        |
| Revenue   | \$ 36                   | \$ —        |
| Operating expenses:   |                         |             |
| Research and development  | 28,152                  | 50,134      |
| General and administrative  | 9,178                   | 12,168      |
| Total operating expenses  | 37,330                  | 62,302      |
| Loss from operations  | (37,294)                | (62,302)    |
| Other income (expense):   |                         |             |
| Interest income and other income (expense), net   | 1,491                   | 985         |
| Net loss and net loss attributable to common stockholders                                       | \$ (35,803)             | \$ (61,317) |
| Net loss per share attributable to common stockholders, basic and diluted                       | \$ (6.05)               | \$ (7.34)   |
| Weighted average common shares outstanding, basic and diluted                                   | 5,918,054               | 8,357,283   |
| Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited) |                         | \$ (1.76)   |
| Pro forma weighted average common shares outstanding, basic and diluted (unaudited)             |                         | 34,782,947  |
| Comprehensive loss:   |                         |             |
| Net loss  | \$ (35,803)             | \$ (61,317) |
| Other comprehensive income (loss):  |                         |             |
| Unrealized gains (losses) on marketable securities  | (9)                     | 9           |
| Comprehensive loss  | \$ (35,812)             | \$ (61,308) |

The accompanying notes are an integral part of these consolidated financial statements.



# Generation Bio Co.

## Consolidated statements of convertible preferred stock and stockholders' deficit

| (in thousands,<br>except share amounts)  | Series A and B<br>convertible<br>preferred stock |                   | Common stock     |             | Additional<br>paid-in<br>capital | Accumulated<br>other<br>comprehensive<br>loss | Accumulated<br>deficit | Total<br>stockholders'<br>deficit |
|--|--|-------------------|------------------|-------------|----------------------------------|---|------------------------|-----------------------------------|
|  | Shares   | Amount            | Shares           | Amount      |                                  |   |                        |                                   |
| <b>Balances at December 31, 2017</b>   | <b>15,451,020</b>                                | <b>\$ 15,404</b>  | <b>5,153,500</b> | <b>\$ 1</b> | <b>\$ 2,064</b>                  | <b>\$ —</b>                                   | <b>\$ (11,332)</b>     | <b>\$ (9,267)</b>                 |
| Issuance of Series B convertible preferred stock, net of issuance costs of \$181 | 10,974,644                                       | 100,189           | —                | —           | —                                | —   | —                      | —                                 |
| Vesting of restricted common stock   | —  | —                 | 2,007,948        | —           | —                                | —   | —                      | —                                 |
| Stock-based compensation expense   | —  | —                 | —                | —           | 3,488                            | —   | —                      | 3,488                             |
| Unrealized losses on marketable securities                                       | —  | —                 | —                | —           | —                                | (9)   | —                      | (9)                               |
| Net loss   | —  | —                 | —                | —           | —                                | —   | (35,803)               | (35,803)                          |
| <b>Balances at December 31, 2018</b>   | <b>26,425,664</b>                                | <b>115,593</b>    | <b>7,161,448</b> | <b>1</b>    | <b>5,552</b>                     | <b>(9)</b>                                    | <b>(47,135)</b>        | <b>(41,591)</b>                   |
| Issuance of common stock for license   | —  | —                 | 221,985          | —           | —                                | —   | —                      | —                                 |
| Issuance of common stock upon exercise of stock options                          | —  | —                 | 61,365           | —           | 100                              | —   | —                      | 100                               |
| Vesting of restricted common stock   | —  | —                 | 1,865,208        | —           | —                                | —   | —                      | —                                 |
| Stock-based compensation expense   | —  | —                 | —                | —           | 4,207                            | —   | —                      | 4,207                             |
| Unrealized gains on marketable securities  | —  | —                 | —                | —           | —                                | 9   | —                      | 9                                 |
| Net loss   | —  | —                 | —                | —           | —                                | —   | (61,317)               | (61,317)                          |
| <b>Balances at December 31, 2019</b>   | <b>26,425,664</b>                                | <b>\$ 115,593</b> | <b>9,310,006</b> | <b>\$ 1</b> | <b>\$ 9,859</b>                  | <b>\$ —</b>                                   | <b>\$ (108,452)</b>    | <b>\$ (98,592)</b>                |

The accompanying notes are an integral part of these consolidated financial statements.

# Generation Bio Co.

## Consolidated statements of cash flows

| (in thousands)  | Year ended<br>December 31, |                  |
|---|----------------------------|------------------|
|   | 2018                       | 2019             |
| <b>Cash flows from operating activities:</b>  |                            |                  |
| Net loss  | \$ (35,803)                | \$ (61,317)      |
| Adjustments to reconcile net loss to net cash used in operating activities:           |                            |                  |
| Stock-based compensation expense  | 3,488                      | 4,207            |
| Depreciation and amortization expense   | 347                        | 1,864            |
| Accretion of discount on marketable securities  | (834)                      | (317)            |
| Gain on sale of property and equipment  | —                          | (10)             |
| Changes in operating assets and liabilities:  |                            |                  |
| Tenant receivable   | (1,288)                    | 840              |
| Prepaid expenses and other current assets   | (498)                      | (1,863)          |
| Other noncurrent assets   | 17                         | —                |
| Accounts payable  | 223                        | 761              |
| Accrued expenses and other current liabilities  | 3,263                      | 2,470            |
| Deferred rent   | 2,966                      | 13,019           |
| Net cash used in operating activities   | <u>(28,119)</u>            | <u>(40,346)</u>  |
| <b>Cash flows from investing activities:</b>  |                            |                  |
| Purchases of property and equipment   | (3,021)                    | (19,986)         |
| Proceeds from sale of property and equipment  | —                          | 80               |
| Purchases of marketable securities  | (109,940)                  | (20,789)         |
| Sales and maturities of marketable securities   | 43,200                     | 88,680           |
| Net cash provided by (used in) investing activities                                   | <u>(69,761)</u>            | <u>47,985</u>    |
| <b>Cash flows from financing activities:</b>  |                            |                  |
| Proceeds from issuance of convertible preferred stock, net of issuance costs          | 100,189                    | —                |
| Proceeds from exercise of stock options   | —                          | 100              |
| Payments of deferred offering costs   | —                          | (22)             |
| Net cash provided by financing activities   | <u>100,189</u>             | <u>78</u>        |
| <b>Net increase in cash, cash equivalents and restricted cash</b>                     | <b>2,309</b>               | <b>7,717</b>     |
| Cash, cash equivalents and restricted cash at beginning of period                     | 7,157                      | 9,466            |
| Cash, cash equivalents and restricted cash at end of period                           | <u>\$ 9,466</u>            | <u>\$ 17,183</u> |
| <b>Supplemental disclosure of noncash investing and financing information:</b>        |                            |                  |
| Purchases of property and equipment included in accounts payable and accrued expenses | \$ 1,337                   | \$ 644           |
| Deferred offering costs included in accrued expenses                                  | \$ —                       | \$ 65            |

The accompanying notes are an integral part of these consolidated financial statements.

# Generation Bio Co.

## Notes to consolidated financial statements

### 1. Nature of the business and basis of presentation

The Company was incorporated on October 21, 2016 as Torus Therapeutics, Inc. and subsequently changed its name to Generation Bio Co. (the “Company” or “Generation Bio”). The Company is an innovative genetic medicines company creating a new class of gene therapy utilizing its proprietary non-viral gene therapy platform to provide durable, redosable treatments for millions of patients living with rare and prevalent diseases. The Company’s non-viral gene therapy platform incorporates its high-capacity DNA construct called closed-ended DNA (“ceDNA”), its cell-targeted lipid nanoparticle delivery system (“ctLNP”) and its established, scalable capsid-free manufacturing process. Using its approach, the Company is developing novel gene therapies to provide targeted delivery of genetic payloads that include large and multiple genes to a range of tissues across a broad array of diseases. The Company is also engineering its gene therapies to be redosable, which may enable individualized patient titration to reach the desired therapeutic expression and to maintain efficacy throughout a patient’s life. The Company is headquartered in Cambridge, Massachusetts.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations, the ability to establish clinical- and commercial-scale manufacturing processes and the ability to secure additional capital to fund operations. Programs currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization of a product. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company’s development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

The accompanying consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities and commitments in the ordinary course of business. Through December 31, 2019, the Company has funded its operations with the proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017) and the sale of convertible preferred stock. Since inception, the Company has incurred recurring losses, including net losses of \$61.3 million for the year ended December 31, 2019. As of December 31, 2019, the Company had an accumulated deficit of \$108.5 million. The Company expects to continue to generate operating losses in the foreseeable future. The Company expects that its cash and cash equivalents, including gross proceeds of \$111.5 million it received in January 2020 from the sale of Series C convertible preferred stock (see Note 13), will be sufficient to fund its operating expenses and capital expenditure requirements for at least 12 months from the issuance date of the consolidated financial statements.

The Company is seeking to complete an initial public offering (“IPO”) of its common stock. Upon the completion of a qualified public offering on specified terms, the Company’s outstanding convertible preferred stock will automatically convert into shares of common stock (see Note 6).

The Company will need to obtain additional funding through public or private equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. The Company

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may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into collaborative or strategic alliances or licensing arrangements. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. Arrangements with collaborators or others may require the Company to relinquish rights to certain of its technologies or programs. If the Company is unable to obtain funding, the Company could be forced to delay, reduce or eliminate some or all of its research and development programs, pipeline expansion or commercialization efforts, which could adversely affect its business prospects.

Although management will continue to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations when needed or at all.

The accompanying consolidated financial statements reflect the operations of the Company and the Company's wholly-owned subsidiary, Generation Bio Securities Corporation. Intercompany balances and transactions have been eliminated in consolidation. The accompanying consolidated financial statements have been prepared in conformity with generally accepted accounting principles ("GAAP") in the United States of America. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

## **2. Summary of significant accounting policies**

### ***Use of estimates***

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenue and expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the accrual of research and development expenses and stock-based compensation expense. The Company bases its estimates on historical experience, known trends and other market-specific or other relevant factors that it believes to be reasonable under the circumstances. On an ongoing basis, management evaluates its estimates, as there are changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results may differ from those estimates or assumptions.

### ***Unaudited pro forma information***

In the accompanying consolidated statements of operations and comprehensive loss, the unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2019 have been prepared to give effect, upon a qualified IPO, to the automatic conversion of all shares of convertible preferred stock outstanding into shares of common stock as if the proposed IPO had occurred on the later of January 1, 2019 or the issuance date of the convertible preferred stock. The unaudited pro forma basic and diluted weighted average common shares outstanding used in the calculation of the unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2019 do not give effect to the issuance of shares of Series C convertible preferred stock and the resulting adjustment to the Series B convertible preferred stock conversion ratio that occurred in January 2020 (see Notes 6 and 13).

***Concentrations of credit risk and of significant suppliers***

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. The Company has not experienced any other-than-temporary losses with respect to its cash and cash equivalents and does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on a small number of third-party suppliers for its drug substance and drug product. In particular, the Company relies, and expects to continue to rely, on third-party suppliers for certain materials and components required for the production of any product candidates it may develop for its programs. These programs could be adversely affected by a significant interruption in the supply process.

***Deferred offering costs***

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded as a reduction of the carrying value of the preferred stock or, for issuances of common stock, in stockholder's equity (deficit) as a reduction of additional paid-in capital generated as a result of the offering. Should the planned equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expenses in the consolidated statement of operations and comprehensive loss. As of December 31, 2019, the Company recorded \$0.1 million of deferred offering costs related to its Series C convertible preferred stock financing (see Note 13).

***Cash equivalents***

The Company considers all highly liquid investments with a remaining maturity when purchased of three months or less to be cash equivalents.

***Restricted cash***

Amounts included in restricted cash represent amounts pledged as collateral for letters of credit required for security deposits on the Company's leased facilities. These amounts are classified as restricted cash (current and noncurrent) on the Company's consolidated balance sheets.

***Marketable securities***

The Company's marketable securities, which consisted of debt securities as of December 31, 2018, are classified as available-for-sale and are reported at fair value. Unrealized gains and losses on available-for-sale debt securities are reported as a component of accumulated other comprehensive income (loss) in stockholders' equity (deficit). Realized gains and losses and declines in value determined to be other than temporary are based on the specific identification method and are included as a component of other income (expense), net in the consolidated statements of operations and comprehensive loss.

The Company evaluates its marketable securities with unrealized losses for other-than-temporary impairment. When assessing marketable securities for other-than-temporary declines in value, the Company considers such factors as, among other things, how significant the decline in value is as a percentage of the original cost, how long the market value of the investment has been less than its original cost, the Company's ability and intent to retain the investment for a period of

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time sufficient to allow for any anticipated recovery in fair value and market conditions in general. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company reduces the investment to fair value through a charge to the statement of operations and comprehensive loss. No such adjustments were necessary during the periods presented.

### **Fair value measurements**

Certain assets and liabilities of the Company are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and financial liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities, or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents and marketable securities are carried at fair value, determined according to the fair value hierarchy described above (see Note 3). The carrying values of the Company's tenant receivable, accounts payable and accrued expenses approximate their fair values due to the short-term nature of these assets and liabilities.

### **Deferred rent**

Payment escalations, rent holidays and other lease incentives that may be included in lease agreements are accrued or deferred as appropriate such that rent expense for each lease is recognized on a straight-line basis over the respective lease term. Adjustments for such items are recorded as deferred rent and amortized over the respective lease terms.

### **Property and equipment**

Property and equipment are stated at cost less accumulated depreciation and amortization. Depreciation and amortization expense is recognized using the straight-line method over the estimated useful life of each asset as follows:

|                                 | <b>Estimated useful life</b>                      |
|---------------------------------|---|
| Laboratory equipment            | 5 years   |
| Computer equipment and software | 3 years   |
| Furniture and fixtures          | 5 years   |
| Leasehold improvements          | Shorter of remaining life of lease or useful life |

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Costs for capital assets not yet placed into service are capitalized as construction-in-progress and depreciated once placed into service. Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation and amortization are removed from the accounts and any resulting gain or loss is included in loss from operations. Expenditures for repairs and maintenance which do not improve or extend the life of the respective assets are charged to expense as incurred.

### ***Impairment of long-lived assets***

Long-lived assets consist of property and equipment. The Company evaluates the recoverability of its long-lived assets when circumstances indicate that an event of impairment may have occurred. The Company recognizes an impairment loss only if the carrying amount of a long-lived asset is not recoverable based on its undiscounted future cash flows. Impairment is measured based on the difference between the carrying value of the related assets and the fair value of such assets. The Company did not record any impairment losses on long-lived assets during the years ended December 31, 2018 or 2019.

### ***Classification and accretion of convertible preferred stock***

The Company's convertible preferred stock is classified outside of stockholders' equity (deficit) on the consolidated balance sheets because the holders of such shares have redemption rights in the event of a deemed liquidation that, in certain situations, is not solely within the control of the Company and would require the redemption of the then-outstanding convertible preferred stock. A deemed liquidation includes Liquidation Events as that term is defined in Note 6. The Company's Series A and Series B convertible preferred stock are not redeemable, except in the event of a deemed liquidation (see Note 6). Because the occurrence of a deemed liquidation event is not currently probable, the carrying values of the convertible preferred stock are not being accreted to their redemption values. If a deemed liquidation event became probable, the carrying values of the convertible preferred stock would be accreted to redemption values.

### ***Segment information***

The Company has determined that its chief executive officer is the chief operating decision maker ("CODM"). The CODM reviews financial information presented on a consolidated basis. Resource allocation decisions are made by the CODM based on consolidated results. There are no segment managers who are held accountable by the CODM for operations, operating results, and planning for levels or components below the consolidated unit level. As such, the Company has concluded that it operates as one segment. All long-lived assets are located in the United States.

### ***Revenue recognition***

To date, the Company has derived its revenue by providing services to pharmaceutical and life sciences companies. On January 1, 2019, the Company adopted ASU 2014-09, discussed below under the heading "Recently Adopted Accounting Pronouncements", which amended revenue recognition principles and provides a single, comprehensive set of criteria for revenue recognition across all industries. The new revenue standard provides a five-step framework whereby revenue is recognized when control of promised goods or services is transferred to a customer at an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. To determine revenue recognition for arrangements that the Company determines are within the scope of the new revenue standard, the Company performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the

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performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the Company satisfies a performance obligation.

The Company only applies the five-step model to contracts when collectability of the consideration to which the Company is entitled in exchange for the goods or services it transfers to the customer is determined to be probable. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional. The Company does not assess whether a contract has a significant financing component if the expectation at contract inception is that the period between payment by the customer and the transfer of the promised goods or services to the customer will be one year or less. The Company recognizes revenue when it satisfies its performance obligations by delivering the services to its customers in an amount that reflects the consideration to which it expects to be entitled in exchange for those services.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue on the accompanying balance sheets. Amounts expected to be recognized as revenue within 12 months of the balance sheet date are classified as current deferred revenue. The Company had no deferred revenue as of December 31, 2018 or 2019. The Company expenses incremental costs of obtaining a contract as and when incurred if the expected amortization period of the asset that would have been recognized is one year or less or the amount is immaterial. As of December 31, 2019, the Company had not capitalized any costs to obtain contracts.

### ***Research and development costs***

Research and development costs are expensed as incurred. Research and development expenses consist of costs incurred in performing research and development activities, including salaries and bonuses, stock-based compensation, employee benefits, facilities costs, laboratory supplies, depreciation and amortization, manufacturing expenses and external costs of vendors engaged to conduct preclinical development activities as well as the cost of licensing technology.

Upfront payments and milestone payments made for the licensing of technology are expensed as research and development expenses in the period in which they are incurred. Advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses. The prepaid amounts are expensed as the related goods are delivered or the services are performed.

### ***Research and manufacturing contract costs and accruals***

The Company has entered into various research and development and manufacturing contracts. These agreements are generally cancelable, and related payments are recorded as the corresponding expenses are incurred. The Company records accruals for estimated ongoing costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the research studies and manufacturing activities, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.



**Patent costs**

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

**Stock-based compensation**

The Company measures stock options with service-based vesting or performance-based vesting granted to employees, non-employees and directors based on the fair value on the date of grant using the Black-Scholes option-pricing model. The Company measures restricted common stock awards using the difference between the purchase price per share of the award, if any, and the fair value of the Company's common stock at the date of grant. The Company's board of directors values the Company's common stock, taking into consideration its most recently available valuation of common stock performed by third parties as well as additional factors which may have changed since the date of the most recent contemporaneous valuation through the date of grant.

Compensation expense for awards with service-based vesting is generally recognized over the vesting period of the award using the straight-line method to record the expense. The Company uses the graded-vesting method to record the expense of awards with both service-based and performance-based vesting conditions, commencing once achievement of the performance condition becomes probable. The Company accounts for forfeitures of share-based awards as they occur. The Company classifies stock-based compensation expense in its consolidated statements of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Prior to the adoption of ASU 2018-07 on January 1, 2019 discussed below, the Company measured the fair value of stock-based awards granted to non-employees on the date that the related service was complete, which was generally the vesting date of the award. Prior to the service completion date, compensation expense was recognized over the period during which services were rendered by such non-employees. At the end of each financial reporting period prior to the service completion date, the fair value of the unvested awards was remeasured using the then-current fair value of the Company's common stock and updated assumption inputs in the Black-Scholes option-pricing model for options or the then-current fair value of the Company's common stock for restricted common stock awards.

**Comprehensive loss**

Comprehensive loss includes net loss as well as other changes in stockholders' equity (deficit) that result from transactions and economic events other than those with stockholders. For the years ended December 31, 2018 and 2019, the Company's only element of other comprehensive loss was unrealized gains (losses) on marketable securities.

**Income taxes**

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements or in the Company's tax returns. Deferred tax assets and liabilities are determined on the basis of the differences

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between the financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence, that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies.

The Company accounts for uncertainty in income taxes recognized in the consolidated financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the consolidated financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties. The Company's policy is to recognize interest and/or penalties related to income tax matters in income tax expense. The Company had accrued no amounts for interest and penalties on its consolidated balance sheets at December 31, 2018 and 2019.

### ***Net loss per share***

The Company follows the two-class method when computing net income (loss) per share as the Company has issued shares that meet the definition of participating securities. The two-class method determines net income (loss) per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income (loss) available to common stockholders for the period to be allocated between common stock and participating securities based upon their respective rights to share in the earnings as if all income (loss) for the period had been distributed.

Basic net income (loss) per share attributable to common stockholders is computed by dividing net income (loss) attributable to common stockholders by the weighted average number of common shares outstanding for the period. Diluted net income (loss) per share attributable to common stockholders is computed by adjusting net income (loss) attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net income (loss) per share attributable to common stockholders is computed by dividing the diluted net income (loss) attributable to common stockholders by the weighted average number of common shares outstanding for the period, including potential dilutive common shares.

The Company's participating securities contractually entitle the holders of such shares to participate in dividends but do not contractually require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reports a net loss, such losses are not allocated to such participating securities. In periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to

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common stockholders is the same as basic net loss per share attributable to common stockholders, since dilutive common shares are not assumed to have been issued if their effect is anti-dilutive. The Company reported a net loss attributable to common stockholders for the years ended December 31, 2018 and 2019.

### ***Recently adopted accounting pronouncements***

In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes all existing revenue recognition requirements, including most industry-specific guidance. The new standard requires a company to recognize revenue when it transfers goods or services to customers in an amount that reflects the consideration that the Company expects to receive for those goods or services. The FASB has subsequently issued several amendments to ASU 2014-09 that have the same effective date and transition date. For public entities, the guidance was effective for annual reporting periods beginning after December 15, 2017 and for interim periods within that reporting period. For nonpublic entities, the guidance is effective for annual reporting periods beginning after December 15, 2018. The Company adopted ASU 2014-09 on January 1, 2019 using the modified retrospective transition method. The adoption did not have an impact on its consolidated financial statements.

In June 2018, the FASB issued ASU 2018-07, Compensation-Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting. The new standard largely aligns the accounting for share-based payment awards issued to employees and nonemployees by expanding the scope of ASC 718 to apply to nonemployee share-based transactions, as long as the transaction is not effectively a form of financing. For public entities, ASU 2018-07 was required to be adopted for annual periods beginning after December 15, 2018, including interim periods within those fiscal years. For non-public entities, ASU 2018-07 is effective for annual periods beginning after December 15, 2019. Early adoption is permitted for all entities but no earlier than the Company's adoption of ASU 2014-09. The Company early adopted ASU 2018-07 on January 1, 2019 and the adoption did not have a material impact on the Company's financial statements.

### ***Recently issued accounting pronouncements***

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies that the Company adopts as of the specified effective date. The Company qualifies as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012 and has elected not to "opt out" of the extended transition related to complying with new or revised accounting standards, which means that when a standard is issued or revised and it has different application dates for public and nonpublic companies, the Company can adopt the new or revised standard at the time nonpublic companies adopt the new or revised standard and can do so until such time that the Company either (i) irrevocably elects to "opt out" of such extended transition period or (ii) no longer qualifies as an emerging growth company.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). The new standard requires that all lessees recognize the assets and liabilities that arise from leases on the balance sheet and disclose qualitative and quantitative information about its leasing arrangements. In July 2018, the FASB issued ASU 2018-11, which provides entities with an additional transition method to adopt Topic 842. Under the new transition method, an entity initially applies the new lease requirements at the adoption date, not the earliest period presented, and recognizes a cumulative effect adjustment to the opening balance of retained earnings in the period of

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adoption. For public entities, the guidance was effective for annual reporting periods beginning after December 15, 2018 and for interim periods within those fiscal years. For nonpublic entities, the guidance was effective for annual reporting periods beginning after December 15, 2019. Early adoption is permitted for all entities. In November 2019, the FASB issued ASU 2019-10, which deferred the effective date for nonpublic entities to annual reporting periods beginning after December 15, 2020, and interim periods within fiscal years beginning after December 15, 2021. Early adoption continues to be allowed. The Company will adopt ASU 2016-02 on January 1, 2021 using the modified retrospective approach transition method as of the date of adoption such that prior periods will not be restated. The Company will also elect a package of practical expedients, under which an entity need not reassess whether any expired or existing contracts are or contain leases, the lease classification for any expired or existing leases, or initial direct costs for any existing leases. The adoption of the new standard is expected to result in the recognition of material liabilities and right-of-use assets, however, the Company has not completed its assessment.

In June 2016, the FASB issued ASU 2016-13, Financial Instruments – Credit Losses (Topic 326). The new standard adjusts the accounting for assets held at amortized costs basis, including marketable securities accounted for as available-for-sale. The standard eliminates the probable initial recognition threshold and requires an entity to reflect its current estimate of all expected credit losses. The allowance for credit losses is a valuation account that is deducted from the amortized cost basis of the financial assets to present the net amount expected to be collected. For public entities, the guidance is effective for annual reporting periods beginning after December 15, 2019 and for interim periods within those fiscal years. For nonpublic entities, the guidance was effective for annual reporting periods beginning after December 15, 2020. Early adoption is permitted for all entities. In November 2019, the FASB issued ASU 2019-10, which deferred the effective date for nonpublic entities to annual reporting periods beginning after December 15, 2022, including interim periods within those fiscal years. The Company is currently evaluating when to adopt the guidance and the impact the guidance will have on its consolidated financial statements.

In August 2018, the FASB issued ASU 2018-13, Fair Value Measurement (Topic 820): Disclosure Requirements for Fair Value Measurement. The new standard added, modified or removed disclosure requirements under Topic 820 for clarity and consistency. ASU 2018-13 is effective for all entities for fiscal years, and interim periods within those fiscal years, beginning after December 15, 2019. The Company will adopt ASU 2018-03 on January 1, 2020 and does not believe the guidance will have a material impact on its consolidated financial statements.

### 3. Marketable securities and fair value measurements

Marketable securities by security type consisted of the following:

| (in thousands)                | December 31, 2018 |                        |                         |            |
|-------------------------------|-------------------|------------------------|-------------------------|------------|
|                               | Amortized cost    | Gross unrealized gains | Gross unrealized losses | Fair value |
| U.S. treasury bills and notes | \$ 67,574         | \$ —                   | \$ (9)                  | \$67,565   |
|                               | \$ 67,574         | \$ —                   | \$ (9)                  | \$67,565   |

The Company did not have marketable securities as of December 31, 2019.

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The following tables present the Company's fair value hierarchy for its assets that are measured at fair value on a recurring basis and indicate the level within the fair value hierarchy of the valuation techniques the Company utilized to determine such fair value, which is described further within Note 2, Summary of Significant Accounting Policies:

| (in thousands)                | Fair value measurements at<br>December 31, 2018 using: |          |         |          |
|-------------------------------|--|----------|---------|----------|
|                               | Level 1  | Level 2  | Level 3 | Total    |
| Cash equivalents:             |  |          |         |          |
| Money market funds            | \$ 7,244   | \$ —     | \$ —    | \$ 7,244 |
| Marketable securities         |  |          |         |          |
| U.S. treasury bills and notes | —  | 67,565   | —       | 67,565   |
| Totals                        | \$ 7,244   | \$67,565 | \$ —    | \$74,809 |

| (in thousands)     | Fair value measurements at<br>December 31, 2019 using: |         |         |          |
|--------------------|--|---------|---------|----------|
|                    | Level 1  | Level 2 | Level 3 | Total    |
| Cash equivalents:  |  |         |         |          |
| Money market funds | \$15,076   | \$ —    | \$ —    | \$15,076 |
| Totals             | \$15,076   | \$ —    | \$ —    | \$15,076 |

U.S. government money market funds were valued by the Company based on quoted market prices, which represent a Level 1 measurement within the fair value hierarchy. U.S. treasury bills and notes were valued by the Company using quoted prices in active markets for similar securities, which represent a Level 2 measurement within the fair value hierarchy. During the years ended December 31, 2018 and 2019, there were no transfers between Level 1, Level 2 and Level 3.

## 4. Property and equipment, net

Property and equipment, net consisted of the following:

| (in thousands)                                  | December 31, |           |
|---|--------------|-----------|
|   | 2018         | 2019      |
| Laboratory equipment                            | \$ 3,053     | \$ 5,029  |
| Computer equipment and software                 | 261          | 740       |
| Furniture and fixtures                          | —            | 826       |
| Leasehold improvements                          | 1,561        | 12,993    |
| Construction in progress                        | —            | 4,487     |
|   | 4,875        | 24,075    |
| Less: Accumulated depreciation and amortization | (389)        | (2,230)   |
|   | \$ 4,486     | \$ 21,845 |

Depreciation and amortization expense for the years ended December 31, 2018 and 2019 was \$0.3 million and \$1.9 million, respectively. At December 31, 2019, construction in progress was related to the build-out of leasehold improvements in the Company's new facility.

## 5. Accrued expenses

Accrued expenses consisted of the following:

| (in thousands)                                     | December 31,   |                |
|--|----------------|----------------|
|  | 2018           | 2019           |
| Accrued employee compensation and benefits         | \$2,572        | \$3,255        |
| Accrued external research and development expenses | 1,284          | 1,344          |
| Accrued professional fees                          | 102            | 619            |
| Deferred rent                                      | 4              | 1,370          |
| Other  | 912            | 319            |
|  | <u>\$4,874</u> | <u>\$6,907</u> |

## 6. Convertible preferred stock

The Company has issued Series A convertible preferred stock (the "Series A") and Series B convertible preferred stock (the "Series B"). Collectively the Series A and Series B are referred to as the Preferred Stock. On February 22, 2018, the Company issued and sold 10,974,644 shares of Series B at a price of \$9.1457 per share for gross proceeds of \$100.4 million. The Company incurred issuance costs in connection with this transaction of \$0.2 million.

Upon issuance of each class of Preferred Stock, the Company assessed the embedded conversion and liquidation features of the shares and determined that such features did not require the Company to separately account for these features. The Company also concluded that no beneficial conversion feature existed on the issuance date of each class of Preferred Stock.

Preferred Stock consisted of the following at December 31, 2018 and 2019:

| (in thousands except share amounts) | Preferred stock authorized | Preferred stock issued and outstanding | Carrying value    | Liquidation preference | Common stock issuable upon conversion |
|-------------------------------------|----------------------------|--|-------------------|------------------------|---------------------------------------|
| Series A Preferred Stock            | 15,451,020                 | 15,451,020                             | \$ 15,404         | \$ 15,451              | 15,451,020                            |
| Series B Preferred Stock            | 10,974,644                 | 10,974,644                             | 100,189           | 100,371                | 10,974,644(1)                         |
|                                     | <u>26,425,664</u>          | <u>26,425,664</u>                      | <u>\$ 115,593</u> | <u>\$ 115,822</u>      | <u>26,425,664(1)</u>                  |

(1) Upon issuance and sale of Series C convertible preferred stock in January 2020 (see Note 13), the 10,974,644 outstanding shares of Series B became convertible into 12,469,030 shares of common stock and the 26,425,664 outstanding shares of Preferred Stock became convertible into 27,920,050 shares of common stock.

The holders of Preferred Stock have the following rights and preferences:

### Voting

The holders of Preferred Stock are entitled to vote, together with the holders of common stock, on matters submitted to stockholders for a vote. The holders of Preferred Stock are entitled to the number of votes equal to the number of shares of common stock into which each such share of Preferred Stock could convert. In addition, the holders of Series A, voting exclusively and as a separate class, are entitled to elect two directors of the Company. The holders of Series B, voting exclusively and as a separate class, are entitled to elect one director of the Company.

### **Conversion**

Each share of Preferred Stock is convertible at the option of the holder at any time after the date of issuance. Each share of Preferred Stock will be automatically converted into shares of common stock at the applicable conversion ratio then in effect upon the closing of a firm commitment public offering of common stock with a price of at least \$9.1457 per share and at least \$40.0 million of gross proceeds to the Company. Shares of Series A will be automatically converted into shares of common stock at the applicable conversion ratio then in effect upon written consent of the holders of at least a majority of the then-outstanding shares of Series A. Shares of Series B will be automatically converted into shares of common stock at the applicable conversion ratio then in effect upon written consent of the holders of at least 57% of the then-outstanding shares of Series B.

The conversion ratio of each series of Preferred Stock is determined by dividing the Original Issue Price of each series by the Conversion Price of each series. The Original Issue Price is \$1.00 per share for Series A and \$9.1457 per share for Series B. The Conversion Price at issuance was \$1.00 per share for Series A and \$9.1457 per share for Series B, subject to appropriate adjustment in the event of any stock split, stock dividend, combination or other similar recapitalization and other adjustments as set forth in the Company's certificate of incorporation, as amended and restated. As a result, as of December 31, 2018 and 2019, each outstanding share of Preferred Stock was convertible into common stock on a one for one basis. Upon the issuance and sale of Series C convertible preferred stock in January 2020 at a price of \$5.5914 per share, the Conversion Price of the Series B was adjusted (see Note 13).

### **Dividends**

The holders of Preferred Stock are entitled to receive noncumulative dividends if and when declared by the Company's board of directors at a rate of 6.0% of the Original Issuance Price per share of each series of Preferred Stock (subject to appropriate adjustment in the event of any stock split, stock dividend, combination or other similar recapitalization with respect to such shares) per annum. The Company may not declare, pay or set aside any dividends on shares of any other series of capital stock of the Company, other than dividends on common stock payable in common stock, unless the holders of the Preferred Stock first receive, or simultaneously receive, a dividend on each outstanding share of Preferred Stock in an amount equal to the greater of (i) 6.0% of the Original Issuance Price of each series of Preferred Stock (subject to appropriate adjustment in the event of any stock split, stock dividend, combination or other similar recapitalization with respect to such shares) per annum from the date of issuance of such shares, and (ii) (A) in the case of a dividend on common stock or any class or series of stock that is convertible into common stock, that dividend per share of Preferred Stock as would equal the product of (1) the dividend payable on each share of such class or series determined, if applicable, as if all shares of such class or series had been converted into common stock and (2) the number of shares of common stock issuable upon conversion of each share of Preferred Stock, or (B) in the case of a dividend on any class or series that is not convertible into common stock, at a rate per share of Preferred Stock determined by (1) dividing the amount of the dividend payable on each share of such class or series of capital stock by the Original Issue Price of such class or series of capital stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination of or other similar recapitalization affecting such shares) and (2) multiplying such fraction by an amount equal to the Original Issue Price of each series of Preferred Stock. If the Company declares, pays or sets aside, on the same date, a dividend on shares of more than one class or series of capital stock of the Company, the dividend payable to

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the holders of the Preferred Stock shall be calculated based upon the dividend on the class or series of capital stock that would result in the highest Preferred Stock dividend. No dividends were declared or paid during the years ended December 31, 2018 or 2019.

### **Liquidation**

In the event of any voluntary or involuntary liquidation, dissolution or winding-up of the Company or Liquidating Event (as described below), the holders of shares of Preferred Stock will receive, in preference to the common stockholders, an amount equal to the greater of (i) the Original Issue Price per share of the respective share of Preferred Stock, plus all dividends declared but unpaid on such shares, or (ii) the amount the holders would receive if the Preferred Stock were converted into common stock prior to such liquidation event. In the event that the assets available for distribution to the Company's stockholders are not sufficient to permit payment to the holders of Preferred Stock in the full amount to which they are entitled, the assets available for distribution will be distributed on a pro rata basis among the holders of the Series A and Series B. After the payment of all preferential amounts to the holders of the Preferred Stock then, to the extent available, the remaining assets available for distribution shall be distributed among the holders of the common stock ratably based on the number of shares of common stock held by each holder.

Unless the holders of (i) at least a majority of the then-outstanding shares of Preferred Stock, voting together as a single class on an as-converted basis and (ii) at least 57% of the then-outstanding shares of Series B, voting together as a separate series on an as-converted basis, elect otherwise, a Liquidating Event shall include a merger or consolidation (other than one in which stockholders of the Company own a majority by voting power of the outstanding shares of the surviving or acquiring corporation) or a sale, lease, transfer, exclusive license or other disposition of all or substantially all of the assets of the Company.

## **7. Common stock**

The voting, dividend and liquidation rights of the holders of the Company's common stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth above. Each share of common stock entitles the holder to one vote, together with the holders of the Preferred Stock, on all matters submitted to the stockholders for a vote.

## **8. Stock-based compensation**

### **2017 Stock Incentive Plan**

The Company's 2017 Stock Incentive Plan (the "2017 Plan") provides for the Company to grant incentive stock options or nonqualified stock options, restricted stock, restricted stock units and other equity awards to employees, directors and consultants of the Company. The 2017 Plan is administered by the board of directors or, at the discretion of the board of directors, by a committee of the board of directors. The exercise prices, vesting and other restrictions on any award under the 2017 Plan are determined at the discretion of the board of directors, or its committee if so delegated.

Stock options granted under the 2017 Plan with service-based vesting conditions generally vest over four years and expire after ten years. The exercise price for stock options granted is not less than the fair value of common stock as determined by the board of directors as of the date of grant.



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The total number of shares of common stock that may be issued under the 2017 Plan was 14,850,000 shares as of December 31, 2019, of which 1,366,502 shares remained available for future issuance. Shares that are expired, terminated, surrendered or canceled without having been fully exercised will be available for future awards under the 2017 Plan.

### **Stock option valuation**

The fair value of stock option grants is estimated using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

The following table presents, on a weighted average basis, the assumptions used in the Black-Scholes option-pricing model to determine the fair value of stock options granted:

### **Stock options**

|                          | Year ended<br>December 31, |       |
|--------------------------|----------------------------|-------|
|                          | 2018                       | 2019  |
| Risk-free interest rate  | 2.70%                      | 2.00% |
| Expected volatility      | 76.0%                      | 75.7% |
| Expected dividend yield  | —                          | —     |
| Expected term (in years) | 6.1                        | 6.0   |

The following table summarizes the Company's stock option activity since December 31, 2018:

|   | Number of<br>shares | Weighted<br>average<br>exercise<br>price | Weighted<br>average<br>contractual<br>term<br>(in years) | Aggregate<br>intrinsic value<br>(in thousands) |
|---|---------------------|--|--|--|
| Outstanding as of December 31, 2018                 | 4,918,750           | \$ 2.29                                  | 9.4  | \$ 1,403                                       |
| Granted   | 2,121,250           | 3.35                                     |  |  |
| Exercised   | (61,365)            | 1.63                                     |  |  |
| Forfeited   | (1,019,033)         | 2.20                                     |  |  |
| Outstanding as of December 31, 2019                 | 5,959,602           | \$ 2.69                                  | 8.79   | \$ 8,492                                       |
| Vested and expected to vest as of December 31, 2019 | 5,959,602           | \$ 2.69                                  | 8.79   | \$ 8,492                                       |
| Options exercisable as of December 31, 2019         | 1,506,032           | \$ 2.29                                  | 8.38   | \$ 2,735                                       |

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The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had strike prices lower than the fair value of the Company's common stock.

The weighted average grant-date fair value of awards granted during the years ended December 31, 2018 and 2019 was \$2.03 per share and \$2.52 per share, respectively.

There were no stock options exercised during the year ended December 31, 2018. The total intrinsic value of stock options exercised during the year ended December 31, 2019 was \$0.1 million.

### **Restricted common stock**

The Company issued 696,300 shares of service-based restricted common stock awards during the year ended December 31, 2018. The Company did not issue any restricted common stock awards during the year ended December 31, 2019.

The following table summarizes the Company's restricted common stock activity since December 31, 2018:

|  | Shares      | Weighted<br>average<br>grant<br>date fair<br>value |
|--|-------------|--|
| Unvested restricted common stock as of December 31, 2018 | 4,980,883   | \$ 0.70  |
| Issued   | —           | \$ —   |
| Vested   | (1,865,208) | \$ 0.73  |
| Forfeited  | (103,800)   | \$ 0.41  |
| Unvested restricted common stock as of December 31, 2019 | 3,011,875   | \$ 0.69  |

The total fair value of restricted common stock vested during the years ended December 31, 2018 and 2019 was approximately \$1.2 million and \$7.0 million, respectively.

### **Stock-Based Compensation**

The Company records compensation cost for all share-based payment arrangements, including employee, director and consultant stock options and restricted common stock. The Company recorded stock-based compensation expense in the following expense categories of its consolidated statements of operations and comprehensive loss:

| <b>(in thousands)</b>               | <b>Year ended<br/>December 31,</b> |                |
|-------------------------------------|------------------------------------|----------------|
|                                     | <b>2018</b>                        | <b>2019</b>    |
| Research and development expenses   | \$2,093                            | \$2,753        |
| General and administrative expenses | 1,395                              | 1,454          |
|                                     | <b>\$3,488</b>                     | <b>\$4,207</b> |

As of December 31, 2019, total unrecognized compensation cost related to the unvested stock-based awards was \$10.9 million, which is expected to be recognized over a weighted average period of 2.4 years.

## 9. Income taxes

For the years ended December 31, 2018 and 2019, the Company recorded no income tax benefits for the net operating losses incurred or for the research and development tax credits generated in each year, due to its uncertainty of realizing a benefit from those items. All of the Company's operating losses since inception have been generated in the United States.

A reconciliation of the U.S. federal statutory income tax rate to the Company's effective income tax rate is as follows:

|  | Year ended<br>December 31, |         |
|--|----------------------------|---------|
|  | 2018                       | 2019    |
| Federal statutory income tax rate                      | (21.0)%                    | (21.0)% |
| State income taxes, net of federal benefit             | (5.6)                      | (5.9)   |
| Federal and state research and development tax credits | (6.5)                      | (5.1)   |
| Stock-based compensation expense                       | 1.6                        | 1.0     |
| Other  | —                          | 0.2     |
| Change in deferred tax asset valuation allowance       | 31.5                       | 30.8    |
| Effective income tax rate                              | 0.0%                       | 0.0%    |

Net deferred tax assets as of December 31, 2018 and 2019 consisted of the following:

| (in thousands)                                    | December 31, |           |
|---|--------------|-----------|
|   | 2018         | 2019      |
| Deferred tax assets:                              |              |           |
| Net operating loss carryforwards                  | \$ 10,183    | \$ 25,023 |
| Research and development tax credit carryforwards | 2,878        | 5,998     |
| Deferred rent                                     | —            | 4,740     |
| Other   | 1,701        | 1,469     |
| Total deferred tax assets                         | 14,762       | 37,230    |
| Deferred tax liabilities:                         |              |           |
| Property and equipment                            | (516)        | (4,117)   |
| Total deferred tax liabilities                    | (516)        | (4,117)   |
| Valuation allowance                               | (14,246)     | (33,113)  |
| Net deferred tax assets                           | \$ —         | \$ —      |

As of December 31, 2019, the Company had federal net operating loss carryforwards of \$91.9 million, which may be available to offset future taxable income, of which \$8.2 million of the total net operating loss carryforwards expire at various dates beginning in 2036, while the remaining \$83.7 million do not expire but may be limited in their usage to an annual deduction equal to 80% of annual taxable income. As of December 31, 2019, the Company had state net operating loss carryforwards of \$90.6 million, which may be available to offset future taxable income and expire at various dates beginning in 2036. In addition, as of December 31, 2019, the Company also had federal and state research and development tax credit carryforwards of \$4.0 million and \$2.5 million, respectively, which may be available to reduce future tax liabilities and expire at various dates beginning in 2036 and 2032, respectively.

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Utilization of the U.S. federal and state net operating loss carryforwards and research and development tax credit carryforwards may be subject to a substantial annual limitation under Section 382 and Section 383 of the Internal Revenue Code of 1986, and corresponding provisions of state law, due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income and tax liabilities. In general, an ownership change, as defined by Section 382, results from transactions increasing the ownership of certain stockholders or public groups in the stock of a corporation by more than 50% over a three-year period. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382, at any time since inception, utilization of the net operating loss carryforwards or research and development tax credit carryforwards may be subject to an annual limitation, which is determined by first multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before their utilization. Further, until a study is completed by the Company and any limitation is known, no amounts are being presented as an uncertain tax position.

The Company has evaluated the positive and negative evidence bearing upon its ability to realize the deferred tax assets, which consist primarily of net operating loss carryforwards and research and development tax credit carryforwards. Management has considered the Company's history of cumulative net losses incurred since inception, estimated future taxable income and prudent and feasible tax planning strategies and has concluded that it is more likely than not that the Company will not realize the benefits of federal and state net deferred tax assets. Accordingly, a full valuation allowance has been established against the net deferred tax assets as of December 31, 2018 and 2019. The Company reevaluates the positive and negative evidence at each reporting period.

The changes in the valuation allowance for deferred tax assets during the year ended December 31, 2018 and 2019 related primarily to the increases in net operating loss carryforwards and research and development tax credit carryforwards. The changes in the valuation allowance for 2018 and 2019 were as follows:

| <b>(in thousands)</b>                       | <b>Year ended<br/>December 31,</b> |             |
|---|------------------------------------|-------------|
|   | <b>2018</b>                        | <b>2019</b> |
| Valuation allowance as of beginning of year | \$ 2,976                           | \$14,246    |
| Increases recorded to income tax provision  | 11,270                             | 18,867      |
| Valuation allowance as of end of year       | \$14,246                           | \$33,113    |

The Company assesses the uncertainty in its income tax positions to determine whether a tax position of the Company is more likely than not to be sustained upon examination, including resolution of any related appeals or litigation processes, based on the technical merits of the position. For tax positions meeting the more-likely-than-not threshold, the tax amount recognized in the financial statements is reduced by the largest benefit that has a greater than 50% likelihood of being realized upon the ultimate settlement with the relevant taxing

authority. No reserve for uncertain tax positions or related interest and penalties has been recorded at December 31, 2018 and 2019.

The Company files income tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending tax examinations. The Company is open to future tax examination under statute from 2016 to the present.

## 10. License agreements

### *NIH*

The Company has an agreement with the U.S. Department of Health and Human Services, as represented by The National Heart, Lung, and Blood Institute, an Institute of the National Institutes of Health ("NIH"), entered into in 2017, pursuant to which NIH granted the Company a non-exclusive license, with the right to grant sublicenses, under certain NIH intellectual property related to the Company's ceDNA construct. In July 2019, the agreement was amended to include Association Institut de Myologie, Universite Pierre et Marie Curie, Centre National de la Recherche Scientifique, and Inserm Transfert SA, collectively referred to as the French Institutions, as a licensor.

The Company is obligated to make future milestone payments of up to \$0.4 million per licensed product upon the achievement of specified milestones as well as royalties on a licensed product-by-licensed product and country-by-country basis of a low single digit percentage of annual net sales of licensed products. The Company is obligated to pay a high single-digit royalty percentage of all sublicensing income. The royalties on net sales may be reduced by up to 25% in certain circumstances as defined in the agreement. The Company's royalty obligation expires on a licensed product-by-licensed product and country-by-country basis upon the expiration of the last-to-expire licensed intellectual property rights in such country. Additionally, the Company is required to reimburse the French Institutions for a portion of certain past and ongoing patent related expenses related to the licensed technology. The agreement requires the Company to use reasonable commercial efforts to meet certain performance milestones and execute a commercial development plan within specified timeframes.

Unless terminated earlier, the agreement remains in effect until the last to expire of the licensed patent rights on a licensed product-by-licensed product and country-by-country basis. NIH and the French Institutions may terminate the agreement if the Company fails to perform its material obligations, including but not limited to its failure to meet the applicable performance milestones despite using commercially reasonable efforts, and has not remediated such deficiency within a specified time period. NIH and the French Institutions can terminate the agreement in the event the Company becomes insolvent, files a petition in bankruptcy, has such a petition filed against it, or determines to file a petition in bankruptcy. In addition, NIH and the French Institutions may terminate the agreement in the event of a material breach by the Company and failure to cure such breach within a certain period of time. The company is currently in compliance with the terms of the agreement. The Company can voluntarily terminate the agreement with prior notice to NIH and the French Institutions.

During each of the years ended December 31, 2018 and 2019, the Company recorded research and development expense of less than \$0.1 million under this agreement.

### **UMass**

The Company has an agreement with the University of Massachusetts as represented by and solely on behalf of its Medical School (“UMass”), entered into in 2017, pursuant to which UMass granted the Company an exclusive license, with the right to grant sublicenses, under the UMass intellectual property related to the Company’s ceDNA construct.

The Company is obligated to make future milestone payments of up to \$0.8 million per licensed product upon the achievement of specified milestones as well as royalties on a licensed product-by-licensed product and country-by-country basis of a low single digit percentage of annual net sales of licensed products, subject to annual minimum royalties as defined in the agreement. Additionally, the Company has agreed to pay a low-to-mid single-digit royalty percentage of all sublicensing income, which will vary depending on when the sublicense agreement to a third party was executed. The milestone payments are non-refundable and non-creditable against any other payments due to UMass under the agreement. The Company’s royalty obligation expires on a licensed product-by-licensed product and country-by-country basis upon the expiration of the last-to-expire licensed intellectual property rights in such country. The agreement requires the Company to use diligent efforts to meet certain performance milestones within specified timeframes.

As part of the arrangement, the Company agreed to issue 221,985 shares of its common stock to UMass. The Company recorded \$0.1 million as research and development expense and additional-paid-in capital at the time of the agreement in 2017, representing the fair value of the common stock at that time. The shares were issued in August 2019.

Unless terminated earlier, the agreement will continue until the last-to-expire valid claim of the licensed patents. UMass may terminate the agreement if the Company fails to perform its material obligations, including but not limited to its failure to meet the applicable performance milestones despite using commercially reasonable efforts, and has not remediated such deficiency within a specified time period or negotiated a revised performance timeline. UMass can terminate the agreement if the Company fails to make any payments within a specified period after receiving written notice of such failure, or in the event of a material breach by the Company and failure to cure such breach within a certain period of time. The Company is currently in compliance with the terms of the agreement. The Company can voluntarily terminate the agreement with prior notice to UMass.

During each of the years ended December 31, 2018 and 2019, the Company recorded research and development expense of less than \$0.1 million under this agreement.

### ***Other license agreements***

The Company has other license agreements under which it may become subject to future additional fees and milestone payments.

## **11. Commitments and contingencies**

The Company leases its office and laboratory space under a noncancelable operating lease that was entered into in August 2018, as amended in July 2019, and expires in 2029. The Company has an option to extend for one additional term of five years at the greater of the then-current base rent, or the then-current fair market value. The lease agreement includes provisions for a free-rent period and annual rent increases, which are accrued or deferred as appropriate such that

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rent expense for the lease is recognized on a straight-line basis over the lease term (see Note 2). The Company is responsible for real estate taxes, maintenance, and other operating expenses applicable to the leased premises. The lease provides for a tenant improvement allowance, at the cost of the lessor, not to exceed \$14.7 million. Costs incurred by the Company for tenant improvements but not yet reimbursed by the landlord are presented on the accompanying consolidated balance sheets as tenant receivable. As of December 31, 2018 and 2019, the Company had a tenant receivable of \$1.3 million and \$0.4 million, respectively. The Company posted a customary letter of credit in the amount of approximately \$2.1 million as a security deposit. The letter of credit is subject to increase if the Company were to sublease any portion of the leased premises.

Previously, the Company leased its office and laboratory space under noncancelable operating leases that expired during 2019.

Rent expense for the years ended December 31, 2018 and 2019 was \$3.5 million and \$7.9 million, respectively.

Future minimum lease payments as of December 31, 2019 are as follows:

| <b>Year ending December 31,</b> | <b>(in thousands)</b> |
|---------------------------------|-----------------------|
| 2020                            | \$ 6,867              |
| 2021                            | 7,053                 |
| 2022                            | 7,251                 |
| 2023                            | 7,441                 |
| 2024                            | 7,679                 |
| Thereafter                      | 35,451                |
|                                 | <u>\$ 71,742</u>      |

### ***401(k) Plan***

The Company has a defined-contribution plan under Section 401(k) of the Internal Revenue Code of 1986 (the "401(k) Plan"). The 401(k) Plan covers all employees who meet defined minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. As currently established, the Company is not required to make, and to date has not made, any contributions to the 401(k) Plan.

### ***Indemnification agreements***

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, contract research organizations, business partners and other parties with respect to certain matters including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its board of directors and its executive officers that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as directors or officers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. The Company has not incurred any material costs as a result of such indemnifications and is not currently aware of any indemnification claims.

**Legal proceedings**

The Company, from time to time, may be party to litigation arising in the ordinary course of business. The Company was not subject to any material legal proceedings during the years ended December 31, 2018 and 2019.

**12. Net loss and unaudited pro forma net loss per share****Net loss per share**

The Company has generated a net loss in all periods presented, therefore the basic and diluted net loss per share attributable to common stockholders are the same as the inclusion of the potentially dilutive securities would be anti-dilutive. The Company excluded the following potential common shares, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to common stockholders for the periods indicated:

|  | <b>Year ended December 31,</b> |                   |
|--|--------------------------------|-------------------|
|  | <b>2018</b>                    | <b>2019</b>       |
| Convertible preferred stock (as converted to common stock) | 26,425,664                     | 26,425,664        |
| Unvested restricted common stock                           | 4,980,883                      | 3,011,875         |
| Stock options to purchase common stock                     | 4,918,750                      | 5,959,602         |
|  | <b>36,325,297</b>              | <b>35,397,141</b> |

**Unaudited pro forma net loss per share**

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2019 has been prepared to give effect to adjustments arising upon the completion of a qualified IPO. The unaudited pro forma basic and diluted weighted average common shares outstanding used in the calculation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2019 has been prepared to give effect, upon a qualified IPO, to the automatic conversion of all outstanding shares of Series A and Series B convertible preferred stock into common stock as if the proposed IPO had occurred on the later of January 1, 2019 or the issuance date of the Series A and Series B convertible preferred stock. The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2019 do not give effect to the issuance of shares of Series C convertible preferred stock and the resulting adjustment to the Series B convertible preferred stock conversion ratio that occurred in January 2020 (see Notes 6 and 13).



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Unaudited pro forma basic and diluted net loss per share attributable to common stockholders were calculated as follows:

|   | Year ended<br>December 31,<br>2019<br>(unaudited) |
|---|---|
| <b>(in thousands except share and per share amounts)</b>  |   |
| Numerator:  |   |
| Net loss attributable to common stockholders  | \$ (61,317)                                       |
| Pro forma net loss attributable to common stockholders  | \$ (61,317)                                       |
| Denominator:  |   |
| Weighted average common shares outstanding, basic and diluted   | 8,357,283   |
| Pro forma adjustment to reflect automatic conversion of convertible preferred stock to common stock upon the completion of the proposed IPO | 26,425,664  |
| Pro forma weighted average common shares outstanding, basic and diluted   | 34,782,947  |
| Pro forma net loss per share attributable to common stockholders, basic and diluted   | \$ (1.76)   |

### 13. Subsequent events

The Company has completed an evaluation of all subsequent events after the audited balance sheet date of December 31, 2019 through April 9, 2020, the date these consolidated financial statements were issued, to ensure that these consolidated financial statements include appropriate disclosure of events both recognized in the consolidated financial statements as of December 31, 2019, and events which occurred subsequently but were not recognized in the consolidated financial statements.

#### ***Issuance and sale of Series C convertible preferred stock***

In January 2020, the Company issued and sold 19,936,296 shares of Series C convertible preferred stock (the "Series C"), at a price of \$5.5914 per share, for gross proceeds of \$111.5 million. The terms of the Series C Preferred Stock are substantially the same as the terms of the Series A and Series B Preferred Stock, except for the liquidation preference per share, which is equal to the per share price paid. In connection with the issuance, the Company increased the number of authorized shares of preferred stock from 26,425,664 shares to 46,361,960 shares. Because the price per share of the Series C in this transaction was lower than the Conversion Price of the Company's Series B, in accordance with the Company's certificate of incorporation, as amended and restated, the Conversion Price of Series B was adjusted from \$9.1457 to \$8.0496 per share. As a result, the 10,974,644 outstanding shares of Series B as of December 31, 2019 became convertible into 12,469,030 shares of common stock.

#### ***Increase in shares available for issuance under the 2017 Plan***

In January 2020, the number of shares of common stock authorized for issuance under the 2017 Plan was increased from 14,850,000 shares to 18,150,000 shares.

#### ***Grant of stock options under the 2017 Plan***

On March 5, 2020, the Company granted options with service-based vesting criteria for the purchase of an aggregate of 1,673,413 shares of common stock, at an exercise price of \$2.92 per

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share. The aggregate grant-date fair value of these options was \$3.4 million, which is expected to be recognized over approximately four years.

On March 5, 2020, the Company granted options with performance-based vesting criteria for the purchase of an aggregate of 1,266,787 shares of common stock, at an exercise price of \$2.92 per share. The aggregate grant-date fair value of these options was \$2.3 million, which will be recognized once it becomes probable that the milestone will be achieved and the shares will vest.

On March 31, 2020, the Company granted options with service-based vesting criteria for the purchase of an aggregate of 112,750 shares of common stock, at an exercise price of \$2.97 per share. The aggregate grant-date fair value of these options was \$0.2 million, which is expected to be recognized over approximately four years.

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Through and including \_\_\_\_\_, 2020, (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

**shares**

**generation bio™**

***Common stock***

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**Prospectus**

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*Joint Book-Running Managers*

**J.P. Morgan**

**Jefferies**

**Cowen**

*Lead Manager*

**Wedbush PacGrow**

**, 2020**

## PART II

### Information not required in prospectus

#### Item 13. Other expenses of issuance and distribution

The following table sets forth the expenses to be incurred in connection with the offering described in this registration statement, other than underwriting discounts and commissions, all of which will be paid by the registrant. All amounts are estimates except the Securities and Exchange Commission, or SEC, registration fee, the Financial Industry Regulatory Authority, Inc. filing fee and the Nasdaq Global Market initial listing fee.

|  | <b>Amount</b> |
|--|---------------|
| SEC registration fee                                     |               |
| Financial Industry Regulatory Authority, Inc. filing fee |               |
| Nasdaq Global Stock Market initial listing fee           |               |
| Accountants' fees and expenses                           |               |
| Legal fees and expenses                                  |               |
| Blue Sky fees and expenses                               |               |
| Transfer agent's fees and expenses                       |               |
| Printing and engraving expenses                          |               |
| Miscellaneous  |               |
| Total expenses   |               |

#### Item 14. Indemnification of directors and officers

Section 102 of the General Corporation Law of the State of Delaware, or the DGCL, permits a corporation to eliminate the personal liability of its directors or its stockholders for monetary damages for a breach of fiduciary duty as a director, except where the director breached his or her duty of loyalty, failed to act in good faith, engaged in intentional misconduct or knowingly violated a law, authorized the payment of a dividend or approved a stock repurchase in violation of Delaware corporate law or obtained an improper personal benefit. Our certificate of incorporation that will be effective upon the closing of this offering provides that no director shall be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duty as a director, notwithstanding any provision of law imposing such liability, except to the extent that the DGCL prohibits the elimination or limitation of liability of directors for breaches of fiduciary duty.

Section 145 of the DGCL provides that a corporation has the power to indemnify a director, officer, employee, or agent of the corporation, and certain other persons serving at the request of the corporation in related capacities against expenses (including attorneys' fees), judgments, fines and amounts paid in settlements actually and reasonably incurred by the person in connection with an action, suit or proceeding to which he or she is or is threatened to be made a party by reason of such position, if such person acted in good faith and in a manner he or she reasonably believed to be in or not opposed to the best interests of the corporation, and, in any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful, except that, in the case of actions brought by or in the right of the corporation, no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to the corporation unless and only to the extent that the Court of Chancery or other adjudicating court determines that, despite the adjudication of liability but in view of all of the circumstances of the case, such person is fairly and reasonably entitled to indemnification for such expenses which the Court of Chancery or such other court shall deem proper.

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Our certificate of incorporation that will be effective upon the closing of this offering provides that we will indemnify each person who was or is a party or threatened to be made a party to any threatened, pending or completed action, suit or proceeding, whether civil, criminal, administrative or investigative (other than an action by or in the right of us), by reason of the fact that he or she is or was, or has agreed to become, our director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (all such persons being referred to as an Indemnitee), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees), liabilities, losses, judgments, fines (including excise taxes and penalties arising under the Employee Retirement Income Security Act of 1974) and amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding and any appeal therefrom if such Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, and, with respect to any criminal action or proceeding, he or she had no reasonable cause to believe his or her conduct was unlawful.

Our certificate of incorporation that will be effective upon the closing of this offering also provides that we will indemnify any Indemnitee who was or is a party or threatened to be made a party to any threatened, pending or completed action or suit by or in the right of us to procure a judgment in our favor by reason of the fact that the Indemnitee is or was, or has agreed to become, our director or officer, or is or was serving, or has agreed to serve, at our request as a director, officer, partner, employee or trustee of, or in a similar capacity with, another corporation, partnership, joint venture, trust or other enterprise (including any employee benefit plan), or by reason of any action alleged to have been taken or omitted in such capacity, against all expenses (including attorneys' fees) and, to the extent permitted by law, amounts paid in settlement actually and reasonably incurred in connection with such action, suit or proceeding, and any appeal therefrom, if the Indemnitee acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, our best interests, except that no indemnification shall be made with respect to any claim, issue or matter as to which such person shall have been adjudged to be liable to us, unless and only to the extent that the Court of Chancery of Delaware or the court in which such action or suit was brought determines that, despite such adjudication but in view of all of the circumstances, he or she is fairly and reasonably entitled to indemnification of such expenses (including attorney's fees). Notwithstanding the foregoing, to the extent that any Indemnitee has been successful, on the merits or otherwise, he or she will be indemnified by us against all expenses (including attorneys' fees) actually and reasonably incurred by him or her or on his or her behalf in connection therewith. If we do not assume the defense, expenses must be advanced to an Indemnitee under certain circumstances.

In addition, we intend to enter into new indemnification agreements with all of our directors and executive officers prior to the completion of this offering. In general, these agreements provide that we will indemnify the directors or executive officers to the fullest extent permitted by law for claims arising in his or her capacity as a director or executive officer of our company or in connection with his or her service at our request for another corporation or entity. The indemnification agreements also provide for procedures that will apply in the event that a director or executive officer makes a claim for indemnification and establish certain presumptions that are favorable to the executive officer or director.

We maintain a general liability insurance policy that covers certain liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers.

The underwriting agreement we will enter into in connection with the offering of common stock being registered hereby provides that the underwriters will indemnify, under certain conditions, our directors and officers (as well as certain other persons) against certain liabilities arising in connection with such offering.

Insofar as the foregoing provisions permit indemnification of directors, executive officers or persons controlling us for liability arising under the Securities Act of 1933, as amended, or the Securities Act, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

## **Item 15. Recent sales of unregistered securities**

Set forth below is information regarding shares of our common stock, shares of our preferred stock and stock options granted by us within the past three years that were not registered under the Securities Act. Also included is the consideration, if any, received by us for such shares and options and information relating to the section of the Securities Act, or rule of the SEC, under which exemption from registration was claimed.

### ***(a) Issuances of convertible promissory notes and preferred stock***

Between November 2016 and February 2017, we issued convertible promissory notes in an aggregate principal amount of \$1,000,000. The notes accrued interest at a rate of 6% per annum. On November 20, 2017, all principal and accrued but unpaid interest under the notes were converted into 1,051,020 shares of our Series A preferred stock.

Between April and September 2017, we issued Simple Agreements for Future Equity in an aggregate principal amount of \$6,000,000. On November 20, 2017, these agreements converted into 6,000,000 shares of our Series A preferred stock.

Between November and December 2017, we issued and sold 8,400,000 shares of our Series A preferred stock to four investors at a price per share of \$1.00 in cash, for an aggregate purchase price of \$8,400,000.

On February 21, 2018, we issued and sold 10,974,644 shares of our Series B preferred stock to 26 investors at a price per share of \$9.1457 in cash, for an aggregate purchase price of \$100,370,801.68.

On January 9, 2020, we issued and sold 19,936,296 shares of our Series C preferred stock to 54 investors at a price per share of \$5.5914 in cash, for an aggregate purchase price of \$111,471,805.56.

No underwriters were involved in the foregoing issuances of securities. The securities described in this section (a) of Item 15 were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(a)(2) under the Securities Act and, in certain cases, Regulation D thereunder, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All purchasers received written disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration statement or an available exemption from such registration.

### ***(b) Issuances of common stock***

Between April 9, 2017 and April 9, 2020, we issued an aggregate of 8,096,800 shares of restricted common stock and 4,576,000 shares of common stock.

On August 20, 2019, in connection with our entry into a license agreement, we issued 221,985 shares of common stock to the University of Massachusetts.

No underwriters were involved in the foregoing issuances of securities. The issuances of shares of common stock described in this section (b) of Item 15 were issued pursuant to written compensatory plans or arrangements with our employees, directors and consultants, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act or pursuant to Section 4(a)(2) under the Securities Act. All recipients either received adequate information about our company or had access, through employment or other relationships, to such information.

**(c) Stock option grants and exercises**

Between April 9, 2017 and April 9, 2020, we granted options to purchase an aggregate of 10,352,452 shares of common stock, with exercise prices ranging from \$0.34 to \$4.11 per share, to our employees, advisors and consultants pursuant to our 2017 Stock Incentive Plan. Between April 9, 2017 and April 9, 2020, we issued 122,888 shares of our common stock upon the exercise of stock options outstanding under our 2017 Stock Incentive Plan for aggregate consideration of \$254,055.

No underwriters were involved in the foregoing issuances of securities. The stock options and the shares of common stock issued upon the exercise of the options described in this paragraph (c) of Item 15 were issued pursuant to written compensatory plans or arrangements with our employees, advisors and consultants, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All recipients either received adequate information about our company or had access, through employment or other relationships, to such information.

## Item 16. Exhibits and financial statement schedules

(a) Exhibits.

| <b>Exhibit number</b> | <b>Description of exhibit</b>   |
|-----------------------|---|
| 1.1*                  | Form of Underwriting Agreement  |
| 3.1                   | Third Amended and Restated Certificate of Incorporation of the registrant   |
| 3.2                   | By-laws of the registrant   |
| 3.3*                  | Form of Certificate of Incorporation of the registrant (to be effective upon the closing of this offering)  |
| 3.4*                  | Form of Bylaws of the registrant (to be effective upon the closing of this offering)  |
| 4.1*                  | Specimen Stock Certificate evidencing the shares of common stock  |
| 4.2                   | Second Amended and Restated Investors' Rights Agreement dated as of January 9, 2020 by and among the registrant and the other parties thereto   |
| 5.1*                  | Opinion of Wilmer Cutler Pickering Hale and Dorr LLP  |
| 10.1                  | 2017 Stock Incentive Plan   |
| 10.2                  | Form of Stock Option Agreement under 2017 Stock Incentive Plan  |
| 10.3                  | Form of Restricted Stock Agreement under 2017 Stock Incentive Plan  |
| 10.4*                 | 2020 Stock Incentive Plan   |
| 10.5*                 | Form of Stock Option Agreement under 2020 Stock Incentive Plan  |
| 10.6*                 | Form of Restricted Stock Agreement under 2020 Stock Incentive Plan  |
| 10.7*                 | Form of Restricted Stock Unit Agreement under 2020 Stock Incentive Plan   |
| 10.8*                 | 2020 Employee Stock Purchase Plan   |
| 10.9                  | Lease, dated August 2, 2018, by and between the registrant and BMR-Rogers Street LLC, as amended  |
| 10.10*                | Form of Indemnification Agreement between the registrant and each of its Executive Officers and Directors   |
| 10.11†                | Exclusive License Agreement, dated June 28, 2017, by and between the registrant and the University of Massachusetts   |
| 10.12†                | Public Health Service Patent License Agreement Nonexclusive Sublicensable, dated February 2, 2017, by and between the registrant and the U.S. Department of Health and Human Services, as represented by the National Heart, Lung, and Blood Institute, as amended on June 20, 2019 |
| 21.1                  | Subsidiaries of the registrant  |
| 23.1*                 | Consent of Ernst & Young LLP  |
| 23.2*                 | Consent of Wilmer Cutler Pickering Hale and Dorr LLP (included in Exhibit 5.1)  |
| 24.1*                 | Power of Attorney (included on signature page)  |

\* To be filed by amendment.

† Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.



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(b) Financial statement schedules

No financial statement schedules are provided because the information called for is not required or is shown either in the financial statements or the related notes.

**Item 17. Undertakings**

The undersigned registrant hereby undertakes to provide to the underwriters, at the closing specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

## Signatures

Pursuant to the requirements of the Securities Act of 1933, as amended, the registrant has duly caused this registration statement to be signed on its behalf by the undersigned, thereunto duly authorized, in Cambridge, Commonwealth of Massachusetts, on this \_\_\_\_\_ day of \_\_\_\_\_, 2020.

**GENERATION BIO CO.**

By: \_\_\_\_\_

Geoff McDonough, M.D.  
President and Chief Executive Officer

## Signatures and power of attorney

We, the undersigned officers and directors of Generation Bio Co., hereby severally constitute and appoint Geoff McDonough, M.D. and Jennifer Elliott, Ph.D., J.D. and each of them singly (with full power to each of them to act alone), our true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution in each of them for him or her and in his or her name, place and stead, and in any and all capacities, to sign any and all amendments (including post-effective amendments) to this registration statement (or any other registration statement for the same offering that is to be effective upon filing pursuant to Rule 462(b) under the Securities Act of 1933), and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite or necessary to be done in and about the premises, as full to all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents or any of them, or their, his or her substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, this Registration Statement has been signed by the following persons in the capacities held on the dates indicated.

| <u>Signature</u>                        | <u>Title</u>   | <u>Date</u> |
|---|--|-------------|
| <u>Geoff McDonough, M.D.</u>            | President, Chief Executive Officer and Director<br>(principal executive officer) | , 2020      |
| <u>Catherine Stehman-Breen, M.D.</u>    | Director   | , 2020      |
| <u>Gustav Christensen</u>               | Director   | , 2020      |
| <u>Jeffrey Jonas, M.D.</u>              | Director   | , 2020      |
| <u>Donald Nicolson, Ph.D.</u>           | Director   | , 2020      |
| <u>Jason Rhodes</u>                     | Director   | , 2020      |
| <u>Charles Rowland</u>                  | Director   | , 2020      |
| <u>Anthony Quinn, M.B. Ch.B., Ph.D.</u> | Director   | , 2020      |

THIRD AMENDED AND RESTATED  
CERTIFICATE OF INCORPORATION  
OF  
GENERATION BIO CO.

(Pursuant to Sections 242 and 245 of the  
General Corporation Law of the State of Delaware)

Generation Bio Co., a corporation organized and existing under and by virtue of the provisions of the General Corporation Law of the State of Delaware (the “**General Corporation Law**”),

**DOES HEREBY CERTIFY:**

**1.** That the name of this corporation is Generation Bio Co., and that this corporation was originally incorporated pursuant to the General Corporation Law on October 21, 2016 under the name Torus Therapeutics, Inc.

**2.** That the Board of Directors duly adopted resolutions proposing to amend and restate the Amended and Restated Certificate of Incorporation of this corporation, declaring said amendment and restatement to be advisable and in the best interests of this corporation and its stockholders, and authorizing the appropriate officers of this corporation to solicit the consent of the stockholders therefor, which resolution setting forth the proposed amendment and restatement is as follows:

**RESOLVED**, that the Amended and Restated Certificate of Incorporation of this corporation be amended and restated in its entirety to read as follows:

**FIRST:** The name of this corporation is Generation Bio Co. (the “**Corporation**”).

**SECOND:** The address of the registered office of the Corporation in the State of Delaware is 1209 Orange Street, in the City of Wilmington, County of New Castle, Delaware 19801. The name of its registered agent at such address is The Corporation Trust Company.

**THIRD:** The nature of the business or purposes to be conducted or promoted is to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law.

**FOURTH:** The total number of shares of all classes of stock which the Corporation shall have authority to issue is (i) 75,000,000 shares of Common Stock, \$0.0001 par value per share (“**Common Stock**”) and (ii) 46,361,960 shares of Preferred Stock, \$0.0001 par value per share (“**Preferred Stock**”).

The following is a statement of the designations and the powers, privileges and rights, and the qualifications, limitations or restrictions thereof in respect of each class of capital stock of the Corporation.

#### A. COMMON STOCK

1. General. The voting, dividend and liquidation rights of the holders of the Common Stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth herein.

2. Voting. The holders of the Common Stock are entitled to one vote for each share of Common Stock held at all meetings of stockholders (and written actions in lieu of meetings); provided, however, that, except as otherwise required by law, holders of Common Stock, as such, shall not be entitled to vote on any amendment to the Certificate of Incorporation that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to the Certificate of Incorporation or pursuant to the General Corporation Law. There shall be no cumulative voting. The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by (in addition to any vote of the holders of one or more series of Preferred Stock that may be required by the terms of the Certificate of Incorporation) the affirmative vote of the holders of shares of capital stock of the Corporation representing a majority of the votes represented by all outstanding shares of capital stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the General Corporation Law.

#### B. PREFERRED STOCK

15,451,020 shares of the authorized Preferred Stock of the Corporation are hereby designated “**Series A Preferred Stock**,” 10,974,644 shares of the authorized Preferred Stock of the Corporation are hereby designated “**Series B Preferred Stock**” and 19,936,296 shares of the authorized and unissued Preferred Stock of the Corporation are hereby designated “**Series C Preferred Stock**,” in each case with the following rights, preferences, powers, privileges and restrictions, qualifications and limitations. Unless otherwise indicated, references to “sections” or “subsections” in this Part B of this Article Fourth refer to sections and subsections of Part B of this Article Fourth.

1. Dividends. The holders of Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock shall be entitled to receive dividends, out of any assets legally available therefor, on a pari passu basis and prior and in preference to any declaration or payment of any dividend (payable other than in Common Stock or other securities and rights convertible into or entitling the holder thereof to receive, directly or indirectly, additional shares of Common Stock of the Corporation) at the rate of six percent (6%) of the Series A Original Issue Price (as defined below) per share of Series A Preferred Stock per annum (the “**Series A Dividend**”), six percent (6%) of the Series B Original Issue Price (as defined below) per share of Series B Preferred Stock per annum (the “**Series B Dividend**”) and six percent (6%) of the Series C Original Issue Price (as defined below) per share of Series C Preferred Stock per annum (the “**Series C Dividend**”) and together with the Series A Dividend and the Series B Dividend, the “**Preferred Dividend**”), as applicable, and payable only when, as and if declared by the Board of Directors of the Corporation. The Corporation shall not declare, pay or set aside any dividends on shares of any other class or series of capital stock of the Corporation (other than

dividends on shares of Common Stock payable in shares of Common Stock) unless (in addition to the obtaining of any consents required elsewhere in the Certificate of Incorporation) the holders of the Preferred Stock then outstanding shall first receive, or simultaneously receive, a dividend on each outstanding share of Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock in an amount at least equal to the greater of (i) the Series A Dividend, Series B Dividend and Series C Dividend, as applicable, from the date of issuance of such share of Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock (to the extent not paid) and (ii) (A) in the case of a dividend on Common Stock or any class or series that is convertible into Common Stock, that dividend per share of such series of Preferred Stock as would equal the product of (I) the dividend payable on each share of such class or series determined, if applicable, as if all shares of such class or series had been converted into Common Stock and (II) the number of shares of Common Stock issuable upon conversion of a share of such series of Preferred Stock, in each case calculated on the record date for determination of holders entitled to receive such dividend or (B) in the case of a dividend on any class or series that is not convertible into Common Stock, at a rate per share of Preferred Stock determined by (I) dividing the amount of the dividend payable on each share of such class or series of capital stock by the original issuance price of such class or series of capital stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to such class or series) and (II) multiplying such fraction by an amount equal to the Series A Original Issue Price, Series B Original Issue Price or Series C Original Issue Price, as applicable; provided that, if the Corporation declares, pays or sets aside, on the same date, a dividend on shares of more than one class or series of capital stock of the Corporation, the dividend payable to the holders of Preferred Stock pursuant to this Section 1 shall be calculated based upon the dividend on the class or series of capital stock that would result in the highest Preferred Stock dividend. The “**Series A Original Issue Price**” shall mean \$1.00 per share, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A Preferred Stock. The “**Series B Original Issue Price**” shall mean \$9.1457 per share, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series B Preferred Stock. The “**Series C Original Issue Price**” shall mean \$5.5914 per share, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series C Preferred Stock.

2. Liquidation, Dissolution or Winding Up; Certain Mergers, Consolidations and Asset Sales.

2.1 Preferential Payments to Holders of Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the holders of shares of Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders before any payment shall be made to the holders of Common Stock by reason of their ownership thereof, on a pari passu basis, an amount per share equal to the greater of (i) the Series A Original Issue Price, Series B Original Issue Price or Series C Original Issue Price, as applicable, plus any dividends declared but unpaid thereon or (ii) such amount per share as would have been payable had all shares of such series of Preferred Stock been converted into Common Stock pursuant to Section 4 immediately

prior to such liquidation, dissolution, winding up or Deemed Liquidation Event (the amount payable pursuant to this sentence to the holders of Series A Preferred Stock is hereinafter referred to as the “**Series A Liquidation Amount**,” the amount payable pursuant to this sentence to the holders of Series B Preferred Stock is hereinafter referred to as the “**Series B Liquidation Amount**” and the amount payable pursuant to this sentence to the holders of Series C Preferred Stock is hereinafter referred to as the “**Series C Liquidation Amount**”). If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1, the holders of shares of Preferred Stock shall share ratably in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.2 Distribution of Remaining Assets. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, after the payment of all preferential amounts required to be paid to the holders of shares of Preferred Stock, the remaining assets of the Corporation available for distribution to its stockholders shall be distributed among the holders of shares of Common Stock, pro rata based on the number of shares held by each such holder.

### 2.3 Deemed Liquidation Events.

2.3.1 Definition. Each of the following events shall be considered a “**Deemed Liquidation Event**” unless the holders of (i) a majority of the shares of Preferred Stock (as calculated on an as-converted to Common Stock basis) then outstanding (the “**Requisite Holders**”), (ii) at least 57% of the shares of Series B Preferred Stock (as calculated on an as-converted to Common Stock basis) then outstanding, voting together as a separate series (the “**Series B Majority**”) and (iii) a majority of the shares of Series C Preferred Stock (as calculated on an as-converted to Common Stock basis) then outstanding, voting together as a separate series (the “**Series C Majority**”), elect otherwise by written notice sent to the Corporation at least ten (10) days prior to the effective date of any such event:

- (a) a merger or consolidation in which
  - (i) the Corporation is a constituent party; or
  - (ii) a subsidiary of the Corporation is a constituent party and the Corporation issues shares of its capital stock pursuant to such merger or consolidation,

except any such merger or consolidation involving the Corporation or a subsidiary in which the shares of capital stock of the Corporation outstanding immediately prior to such merger or consolidation continue to represent, or are converted into or exchanged for shares of capital stock that represent, immediately following such merger or consolidation, a majority, by voting power, of the capital stock of (1) the surviving or resulting corporation; or (2) if the surviving or resulting corporation is a wholly owned subsidiary of another corporation immediately following such merger or consolidation, the parent corporation of such surviving or resulting corporation; or

(b) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by the Corporation or any subsidiary of the Corporation of all or substantially all the assets of the Corporation and its subsidiaries taken as a whole, or the sale or disposition (whether by merger, consolidation or otherwise) of one or more subsidiaries of the Corporation if substantially all of the assets of the Corporation and its subsidiaries taken as a whole are held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition is to a wholly owned subsidiary of the Corporation.

### 2.3.2 Effecting a Deemed Liquidation Event.

(a) The Corporation shall not have the power to effect a Deemed Liquidation Event referred to in Subsection 2.3.1(a) (i) unless the agreement or plan of merger or consolidation for such transaction (the “**Merger Agreement**”) provides that the consideration payable to the stockholders of the Corporation shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2.

(b) In the event of a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(ii) or 2.3.1(b), if the Corporation does not effect a dissolution of the Corporation under the General Corporation Law within twenty (20) days after such Deemed Liquidation Event, then (i) the Corporation shall send a written notice to each holder of Preferred Stock no later than the twentieth (20<sup>th</sup>) day after the Deemed Liquidation Event advising such holders of their right (and the requirements to be met to secure such right) pursuant to the terms of the following clause to require the redemption of such shares of Preferred Stock, and (ii) unless each of the Requisite Holders, the Series B Majority and the Series C Majority request otherwise in a written instrument delivered to the Corporation not later than forty-five (45) days after such Deemed Liquidation Event, the Corporation shall use the consideration received by the Corporation for such Deemed Liquidation Event (net of any retained liabilities associated with the assets sold or technology licensed, as determined in good faith by the Board of Directors of the Corporation), together with any other assets of the Corporation available for distribution to its stockholders, all to the extent permitted by Delaware law governing distributions to stockholders (the “**Available Proceeds**”), on the sixtieth (60<sup>th</sup>) day after such Deemed Liquidation Event (the “**Redemption Date**”), to redeem all outstanding shares of Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock at a price per share equal to the Series A Liquidation Amount, Series B Liquidation Amount and Series C Liquidation Amount, respectively. Notwithstanding the foregoing, in the event of a redemption pursuant to the preceding sentence, if the Available Proceeds are not sufficient to redeem all outstanding shares of Preferred Stock, the Corporation shall ratably redeem each holder’s shares of Preferred Stock to the fullest extent of such Available Proceeds, and shall redeem the remaining shares as soon as it may lawfully do so under Delaware law governing distributions to stockholders. Prior to the distribution or redemption provided for in this Subsection 2.3.2(b), the Corporation shall not expend or dissipate the consideration received for such Deemed Liquidation Event, except to discharge expenses incurred in connection with such Deemed Liquidation Event or in the ordinary course of business.



(c) Following an election of holders of Preferred Stock to demand redemption as provided in Subsection 2.3.2(b), the Corporation shall promptly and no more than thirty (30) days thereafter send a notice (the “**Redemption Notice**”) to each holder of Preferred Stock stating (i) the number and series of shares of Preferred Stock held by such holder as of the date of such election, (ii) the price at which such shares of Preferred Stock will be redeemed (the “**Redemption Price**”), (iii) the date upon which the holder’s right to convert such shares terminates (as determined in accordance with Subsection 4.1), and (iv) that such holder is to surrender to the Corporation, in the manner and at the place designated, his, her or its certificate or certificates representing the shares of Preferred Stock to be redeemed.

(d) On or before the Redemption Date, each holder of Preferred Stock to be redeemed on such Redemption Date, unless such holder has exercised his, her or its right to convert such shares as provided in Section 4, shall surrender the certificate or certificates representing such shares (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation, in the manner and at the place designated in the Redemption Notice, and thereupon the Redemption Price for such shares shall be payable to the order of the person whose name appears on such certificate or certificates as the owner thereof.

(e) If the Redemption Notice shall have been duly given to each holder of Preferred Stock, and if on the Redemption Date the Redemption Price payable upon redemption of the shares of Preferred Stock is paid or tendered for payment or deposited with an independent payment agent so as to be available therefor in a timely manner, then notwithstanding that the certificates evidencing any of the shares of Preferred Stock so called for redemption shall not have been surrendered, dividends with respect to such shares of Preferred Stock shall cease after the Redemption Date and all rights with respect to such shares shall forthwith after the Redemption Date terminate, except only the right of the holders to receive the Redemption Price without interest upon surrender of their certificate or certificates therefor.

2.3.3 Amount Deemed Paid or Distributed. The amount deemed paid or distributed to the holders of capital stock of the Corporation upon any such merger, consolidation, sale, transfer, exclusive license, other disposition or redemption shall be the cash or the value of the property, rights or securities paid or distributed to such holders by the Corporation or the acquiring person, firm or other entity. The value of such property, rights or securities shall be determined in good faith by the Board of Directors of the Corporation.

2.3.4 Allocation of Escrow and Contingent Consideration. In the event of a Deemed Liquidation Event pursuant to Subsection 2.3.1(a)(i), if any portion of the consideration payable to the stockholders of the Corporation is payable only upon satisfaction of contingencies (the “**Additional Consideration**”), the Merger Agreement shall provide that (a) the portion of such consideration that is not Additional Consideration (such portion, the “**Initial**

**Consideration**") shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2 as if the Initial Consideration were the only consideration payable in connection with such Deemed Liquidation Event; and (b) any Additional Consideration which becomes payable to the stockholders of the Corporation upon satisfaction of such contingencies shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2 after taking into account the previous payment of the Initial Consideration as part of the same transaction. For the purposes of this Subsection 2.3.4, consideration placed into escrow or retained as holdback to be available for satisfaction of indemnification or similar obligations in connection with such Deemed Liquidation Event shall be deemed to be Additional Consideration.

3. Voting.

3.1 General. On any matter presented to the stockholders of the Corporation for their action or consideration at any meeting of stockholders of the Corporation (or by written consent of stockholders in lieu of meeting), each holder of outstanding shares of Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock shall be entitled to cast the number of votes equal to the number of whole shares of Common Stock into which the shares of Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock held by such holder are convertible as of the record date for determining stockholders entitled to vote on such matter. Except as provided by law or by the other provisions of the Certificate of Incorporation, holders of Series A Preferred Stock, holders of Series B Preferred Stock and holders of Series C Preferred Stock shall vote together with the holders of Common Stock as a single class.

3.2 Election of Directors. The holders of record of the shares of Series A Preferred Stock, exclusively and as a separate class, shall be entitled to elect two directors of the Corporation (the "**Series A Directors**") and the holders of record of the shares of Series B Preferred Stock, exclusively and as a separate class, shall be entitled to elect one director of the Corporation (the "**Series B Director**" and together with the Series A Directors, the "**Preferred Directors**"). Any director elected as provided in the preceding sentence may be removed without cause by, and only by, the affirmative vote of the holders of the shares of the class or series of capital stock entitled to elect such director or directors, given either at a special meeting of such stockholders duly called for that purpose or pursuant to a written consent of stockholders. If the holders of shares of Series A Preferred Stock or Series B Preferred Stock fail to elect a sufficient number of directors to fill all directorships for which they are entitled to elect directors, voting exclusively and as a separate class, pursuant to the first sentence of this Subsection 3.2, then any directorship not so filled shall remain vacant until such time as the holders of the Series A Preferred Stock or Series B Preferred Stock, as the case may be, elect a person to fill such directorship by vote or written consent in lieu of a meeting; and no such directorship may be filled by stockholders of the Corporation other than by the stockholders of the Corporation that are entitled to elect a person to fill such directorship, voting exclusively and as a separate class. The holders of record of the shares of Common Stock and of any other class or series of voting stock (including the Series A Preferred Stock and Series B Preferred Stock), exclusively and voting together as a single class, shall be entitled to elect the balance of the total number of directors of the Corporation. At any meeting held for the purpose of electing a

director, the presence in person or by proxy of the holders of a majority of the outstanding shares of the class or series entitled to elect such director shall constitute a quorum for the purpose of electing such director. Except as otherwise provided in this Subsection 3.2, a vacancy in any directorship filled by the holders of any class or series shall be filled only by vote or written consent in lieu of a meeting of the holders of such class or series or by any remaining director or directors elected by the holders of such class or series pursuant to this Subsection 3.2. The rights of the holders of the Series A Preferred Stock under the first sentence of this Subsection 3.2 shall terminate on the first date following the Series C Original Issue Date (as defined below) on which there are issued and outstanding less than twenty percent (20%) of the shares of Series A Preferred Stock ever issued (subject to appropriate adjustment in the event of any stock dividend, stock split, combination, or other similar recapitalization with respect to the Series A Preferred Stock). The rights of the holders of the Series B Preferred Stock under the first sentence of this Subsection 3.2 shall terminate on the first date following the Series C Original Issue Date on which there are issued and outstanding less than 2,200,000 shares of Series B Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination, or other similar recapitalization with respect to the Series B Preferred Stock).

3.3 Preferred Stock Protective Provisions. At any time when at least 9,272,392 shares of Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock) are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote of the Requisite Holders, given in writing or by vote at a meeting, consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect:

3.3.1 liquidate, dissolve or wind-up the business and affairs of the Corporation, effect any merger or consolidation or any other Deemed Liquidation Event, or consent to any of the foregoing;

3.3.2 amend, alter or repeal any provision of the Certificate of Incorporation or Bylaws of the Corporation;

3.3.3 create, or authorize the creation of, or issue or obligate itself to issue shares of, any class or series of capital stock that is senior to or pari passu with any series of Preferred Stock, or increase the authorized number of shares of Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock or increase the authorized number of shares of any additional class or series of capital stock;

3.3.4 (i) reclassify, alter or amend any existing security of the Corporation that is pari passu with any series of Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to such series of Preferred Stock in respect of any such right, preference, or privilege or (ii) reclassify, alter or amend any existing security of the Corporation that is junior to any series of Preferred Stock in respect of the distribution of assets on the liquidation, dissolution

or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to or pari passu with such series of Preferred Stock in respect of any such right, preference or privilege;

3.3.5 purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of capital stock of the Corporation other than (i) redemptions of or dividends or distributions on the Preferred Stock as expressly authorized herein, (ii) dividends or other distributions payable on the Common Stock solely in the form of additional shares of Common Stock and (iii) repurchases of stock from former employees, officers, directors, consultants or other persons who performed services for the Corporation or any subsidiary in connection with the cessation of such employment or service at the lower of the original purchase price or the then-current fair market value thereof;

3.3.6 create, or authorize the creation of, or issue, or authorize the issuance of any debt security, or permit any subsidiary to take any such action with respect to any debt security;

3.3.7 incur any aggregate indebtedness in excess of \$250,000 without the approval of the Board of Directors, including the approval of a majority of the Preferred Directors, other than trade credit incurred in the ordinary course of business;

3.3.8 create, or hold capital stock in, any subsidiary that is not wholly owned (either directly or through one or more other subsidiaries) by the Corporation, or sell, transfer or otherwise dispose of any capital stock of any direct or indirect subsidiary of the Corporation, or permit any direct or indirect subsidiary to sell, lease, transfer, exclusively license or otherwise dispose (in a single transaction or series of related transactions) of all or substantially all of the assets of such subsidiary;

3.3.9 increase or decrease the authorized number of directors constituting the Board of Directors;

3.3.10 sell, assign, license, pledge, or encumber material technology or intellectual property without the approval of the Board of Directors, including the approval of a majority of the Preferred Directors, other than licenses granted in the ordinary course of business;

3.3.11 guarantee, directly or indirectly, or permit any subsidiary to guarantee, directly or indirectly, any indebtedness without the approval of the Board of Directors, except for trade accounts of the Company or any subsidiary arising in the ordinary course of business;

3.3.12 grant a security interest in the assets of the Corporation (other than in the ordinary course of business) without the approval of the Board of Directors;

3.3.13 make, or permit any subsidiary to make, any loan or advance to any subsidiary or other corporation, partnership, or other entity unless it is wholly owned by the Company;

3.3.14 enter into any transaction with any director, officer or employee of the Corporation or any “associate” (as defined in Rule 12b-2 promulgated under the Securities Exchange Act of 1934, as amended) of any such person other than transactions made in the ordinary course of business and pursuant to reasonable requirements of the Corporation’s business and upon fair and reasonable terms that are approved by the Corporation’s Board of Directors;

3.3.15 hire, terminate, or change the compensation of the Chief Executive Officer, including approving any option grants or stock awards to the Chief Executive Officer, unless such actions have received the prior approval of the Board of Directors, including the approval of a majority of the Preferred Directors; or

3.3.16 establish any equity plan or arrangement for the benefit of the employees, officers, directors, consultants or other persons who perform services for the Corporation or any subsidiary, or increase the number of shares of Common Stock subject to issuance under any such equity plan or arrangement for the benefit of such service providers.

3.4 Series B Preferred Stock Protective Provisions. At any time when at least 2,200,000 shares of Series B Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series B Preferred Stock) are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote of holders of at least 57% of the outstanding shares of Series B Preferred Stock, given in writing or by vote at a meeting, consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect:

3.4.1 amend, alter or repeal any provision of the Certificate of Incorporation or Bylaws of the Corporation in a manner that adversely affects the Series B Preferred Stock;

3.4.2 purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of Common Stock, Series A Preferred Stock or Series C Preferred Stock other than (i) dividends or other distributions payable on the Common Stock solely in the form of additional shares of Common Stock or (ii) repurchases of stock from former employees, officers, directors, consultants or other persons who performed services for the Corporation or any subsidiary in connection with the cessation of such employment or service at the lower of the original purchase price or the then-current fair market value thereof;

3.4.3 create, or hold capital stock in, any subsidiary that is not wholly owned (either directly or through one or more other subsidiaries) by the Corporation;

3.4.4 authorize or consummate any Deemed Liquidation Event if such transaction would result in consideration to the holders of the Series B Preferred Stock in an amount less than the amount they would receive pursuant to Sections 2.1 and 2.2 had the proceeds from such transaction been distributed to stockholders in accordance therewith; or

3.4.5 increase or decrease the authorized number of shares of Series B Preferred Stock.

3.5 Series C Preferred Stock Protective Provisions. At any time when at least 3,987,259 shares of Series C Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series C Preferred Stock) are outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or the Certificate of Incorporation) the written consent or affirmative vote of the Series C Majority, given in writing or by vote at a meeting, consenting or voting (as the case may be) separately as a class, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect:

3.5.1 amend, alter or repeal any provision of the Certificate of Incorporation or Bylaws of the Corporation in a manner that adversely affects the Series C Preferred Stock;

3.5.2 purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of Common Stock, Series A Preferred Stock or Series B Preferred Stock other than (i) dividends or other distributions payable on the Common Stock solely in the form of additional shares of Common Stock or (ii) repurchases of stock from former employees, officers, directors, consultants or other persons who performed services for the Corporation or any subsidiary in connection with the cessation of such employment or service at the lower of the original purchase price or the then-current fair market value thereof;

3.5.3 create, or hold capital stock in, any subsidiary that is not wholly owned (either directly or through one or more other subsidiaries) by the Corporation;

3.5.4 authorize or consummate any Deemed Liquidation Event if such transaction would result in consideration to the holders of the Series C Preferred Stock in an amount less than the amount they would receive pursuant to Sections 2.1 and 2.2 had the proceeds from such transaction been distributed to stockholders in accordance therewith; or

3.5.5 increase or decrease the authorized number of shares of Series C Preferred Stock.

#### 4. Optional Conversion.

The holders of the Preferred Stock shall have conversion rights as follows (the “**Conversion Rights**”):

##### 4.1 Right to Convert.

4.1.1 Conversion Ratio. Each share of Preferred Stock shall be convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into such number of fully paid and non-assessable shares of Common Stock as is determined by dividing the Series A Original Issue Price by the Series A Conversion Price (as defined below) in effect at the time of conversion, in the case of the Series A Preferred Stock, by dividing the Series B Original Issue Price by the Series B Conversion Price (as defined below) in effect at the time of conversion, in the case of the Series B Preferred Stock or by dividing the Series C Original Issue Price by the Series C Conversion Price (as defined below) in effect at the time of conversion, in the case of the Series C Preferred Stock. The “**Series A Conversion Price**” shall initially be equal to \$1.00. The “**Series B Conversion Price**” shall initially be equal to \$8.0496. The “**Series C Conversion Price**” shall initially be equal to \$5.5914. Each of the Series A Conversion Price, the Series B Conversion Price and the Series C Conversion Price is referred to as a “**Conversion Price**.” Such initial Series A Conversion Price, Series B Conversion Price and Series C Conversion Price, and the rate at which shares of Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock, respectively, may be converted into shares of Common Stock, shall be subject to adjustment as provided below.

4.1.2 Termination of Conversion Rights. In the event of an election of redemption of any shares of Preferred Stock pursuant to Subsection 2.3.2(c), the Conversion Rights of the shares designated for redemption shall terminate at the close of business on the last full day preceding the date fixed for redemption, unless the redemption price is not fully paid on such redemption date, in which case the Conversion Rights for such shares shall continue until such price is paid in full. In the event of a liquidation, dissolution or winding up of the Corporation or a Deemed Liquidation Event, the Conversion Rights shall terminate at the close of business on the last full day preceding the date fixed for the payment of any such amounts distributable on such event to the holders of Preferred Stock.

4.2 Fractional Shares. No fractional shares of Common Stock shall be issued upon conversion of the Preferred Stock. In lieu of any fractional shares to which the holder would otherwise be entitled, the Corporation shall pay cash equal to such fraction multiplied by the fair market value of a share of Common Stock as determined in good faith by the Board of Directors of the Corporation. Whether or not fractional shares would be issuable upon such conversion shall be determined on the basis of the total number of shares of Preferred Stock the holder is at the time converting into Common Stock and the aggregate number of shares of Common Stock issuable upon such conversion.

##### 4.3 Mechanics of Conversion.

4.3.1 Notice of Conversion. In order for a holder of Preferred Stock to voluntarily convert shares of Preferred Stock into shares of Common Stock, such holder shall (a) provide written notice to the Corporation’s transfer agent at the office of the transfer agent for the Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent) that such holder elects to convert all or any number of such holder’s shares of Preferred Stock and, if applicable, any event on which such conversion is

contingent and (b), if such holder's shares are certificated, surrender the certificate or certificates for such shares of Preferred Stock (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate), at the office of the transfer agent for the Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent). Such notice shall state such holder's name or the names of the nominees in which such holder wishes the shares of Common Stock to be issued. If required by the Corporation, any certificates surrendered for conversion shall be endorsed or accompanied by a written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or his, her or its attorney duly authorized in writing. The close of business on the date of receipt by the transfer agent (or by the Corporation if the Corporation serves as its own transfer agent) of such notice and, if applicable, certificates (or lost certificate affidavit and agreement) shall be the time of conversion (the "**Conversion Time**"), and the shares of Common Stock issuable upon conversion of the specified shares shall be deemed to be outstanding of record as of such date. The Corporation shall, as soon as practicable after the Conversion Time (i) issue and deliver to such holder of Preferred Stock, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable upon such conversion in accordance with the provisions hereof and a certificate for the number (if any) and series of the shares of Preferred Stock represented by the surrendered certificate that were not converted into Common Stock, (ii) pay in cash such amount as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and (iii) pay all declared but unpaid dividends on the shares of Preferred Stock converted.

4.3.2 Reservation of Shares. The Corporation shall at all times when the Preferred Stock shall be outstanding, reserve and keep available out of its authorized but unissued capital stock, for the purpose of effecting the conversion of the Preferred Stock, such number of its duly authorized shares of Common Stock as shall from time to time be sufficient to effect the conversion of all outstanding Preferred Stock; and if at any time the number of authorized but unissued shares of Common Stock shall not be sufficient to effect the conversion of all then outstanding shares of the Preferred Stock, the Corporation shall take such corporate action as may be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes, including, without limitation, engaging in best efforts to obtain the requisite stockholder approval of any necessary amendment to the Certificate of Incorporation. Before taking any action which would cause an adjustment reducing the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price below the then par value of the shares of Common Stock issuable upon conversion of the Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock, respectively, the Corporation will take any corporate action which may, in the opinion of its counsel, be necessary in order that the Corporation may validly and legally issue fully paid and non-assessable shares of Common Stock at such adjusted Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, respectively.



4.3.3 Effect of Conversion. All shares of Preferred Stock which shall have been surrendered for conversion as herein provided shall no longer be deemed to be outstanding and all rights with respect to such shares shall immediately cease and terminate at the Conversion Time, except only the right of the holders thereof to receive shares of Common Stock in exchange therefor, to receive payment in lieu of any fraction of a share otherwise issuable upon such conversion as provided in Subsection 4.2 and to receive payment of any dividends declared but unpaid thereon. Any shares of Preferred Stock so converted shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of the respective series of Preferred Stock accordingly.

4.3.4 No Further Adjustment. Upon any such conversion, no adjustment to the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price shall be made for any declared but unpaid dividends on the Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock, respectively, surrendered for conversion or on the Common Stock delivered upon conversion.

4.3.5 Taxes. The Corporation shall pay any and all issue and other similar taxes that may be payable in respect of any issuance or delivery of shares of Common Stock upon conversion of shares of Preferred Stock pursuant to this Section 4. The Corporation shall not, however, be required to pay any tax which may be payable in respect of any transfer involved in the issuance and delivery of shares of Common Stock in a name other than that in which the shares of Preferred Stock so converted were registered, and no such issuance or delivery shall be made unless and until the person or entity requesting such issuance has paid to the Corporation the amount of any such tax or has established, to the satisfaction of the Corporation, that such tax has been paid.

4.4 Adjustments to Conversion Price for Diluting Issues.

4.4.1 Special Definitions. For purposes of this Article Fourth, the following definitions shall apply:

(a) **“Option”** shall mean rights, options or warrants to subscribe for, purchase or otherwise acquire Common Stock or Convertible Securities.

(b) **“Series C Original Issue Date”** shall mean the date on which the first share of Series C Preferred Stock was issued.

(c) **“Convertible Securities”** shall mean any evidences of indebtedness, shares or other securities directly or indirectly convertible into or exchangeable for Common Stock, but excluding Options.

(d) “**Additional Shares of Common Stock**” shall mean all shares of Common Stock issued (or, pursuant to Subsection 4.4.3 below, deemed to be issued) by the Corporation after the Series C Original Issue Date, other than (1) the following shares of Common Stock and (2) shares of Common Stock deemed issued pursuant to the following Options and Convertible Securities (clauses (1) and (2), collectively, “**Exempted Securities**”):

- (i) shares of Common Stock, Options or Convertible Securities issued as a dividend or distribution on Preferred Stock;
- (ii) shares of Common Stock, Options or Convertible Securities issued by reason of a dividend, stock split, split-up or other distribution on shares of Common Stock that is covered by Subsection 4.5, 4.6, 4.7 or 4.8;
- (iii) shares of Common Stock or Options issued to employees or directors of, or consultants or advisors to, the Corporation or any of its subsidiaries pursuant to a plan, agreement or arrangement approved by the Board of Directors of the Corporation;
- (iv) shares of Common Stock or Convertible Securities actually issued upon the exercise of Options or shares of Common Stock actually issued upon the conversion or exchange of Convertible Securities, in each case provided such issuance is pursuant to the terms of such Option or Convertible Security and has previously been taken into account under (or constitutes an Exempted Securities under) this Section 4.4;
- (v) shares of Common Stock, Options or Convertible Securities issued to banks, equipment lessors or other financial institutions, or to real property lessors, pursuant to a debt financing, equipment leasing or real property leasing transaction approved by the Board of Directors of the Corporation;
- (vi) shares of Common Stock, Options or Convertible Securities issued to suppliers or third party service providers as consideration, in whole or in part, for the provision of goods or services pursuant to transactions approved by the Board of Directors of the Corporation;

- (vii) shares of Common Stock, Options or Convertible Securities issued as consideration, in whole or in part, for the acquisition of another corporation by the Corporation by merger, purchase of substantially all of the assets or other reorganization or to a joint venture agreement, provided that such issuances are approved by the Board of Directors of the Corporation;
- (viii) shares of Common Stock, Options or Convertible Securities issued as consideration, in whole or in part, for sponsored research, collaboration, technology license, development, OEM, marketing or other similar agreements or strategic partnerships approved by the Board of Directors of the Corporation; or
- (ix) shares of Series C Preferred Stock issued pursuant to that certain Series C Preferred Stock Purchase Agreement, dated as of January 9, 2020, among the Corporation and the signatories thereto.

4.4.2 No Adjustment of Conversion Price. No adjustment in the Series A Conversion Price shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the holders of a majority of the outstanding shares of Series A Preferred Stock agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock. No adjustment in the Series B Conversion Price shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the holders of at least 57% of the outstanding shares of Series B Preferred Stock agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock. No adjustment in the Series C Conversion Price shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the Series C Majority agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock.

4.4.3 Deemed Issue of Additional Shares of Common Stock.

(a) If the Corporation at any time or from time to time after the Series C Original Issue Date shall issue any Options or Convertible Securities (excluding Options or Convertible Securities which are themselves Exempted Securities) or shall fix a record date for the determination of holders of any class of securities entitled to receive any such Options or Convertible Securities, then the maximum number of shares of Common Stock (as set forth in the instrument relating thereto, assuming the satisfaction of any conditions to

exercisability, convertibility or exchangeability but without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or, in the case of Convertible Securities and Options therefor, the conversion or exchange of such Convertible Securities, shall be deemed to be Additional Shares of Common Stock issued as of the time of such issue or, in case such a record date shall have been fixed, as of the close of business on such record date.

(b) If the terms of any Option or Convertible Security, the issuance of which resulted in an adjustment to the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price pursuant to the terms of Subsection 4.4.4, are revised as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase or decrease in the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any such Option or Convertible Security or (2) any increase or decrease in the consideration payable to the Corporation upon such exercise, conversion and/or exchange, then, effective upon such increase or decrease becoming effective, the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, computed upon the original issue of such Option or Convertible Security (or upon the occurrence of a record date with respect thereto) shall be readjusted to such Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, respectively, as would have obtained had such revised terms been in effect upon the original date of issuance of such Option or Convertible Security. Notwithstanding the foregoing, no readjustment pursuant to this clause (b) shall have the effect of increasing the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price to an amount which exceeds the lower of (i) the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, respectively, in effect immediately prior to the original adjustment made as a result of the issuance of such Option or Convertible Security, or (ii) the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, respectively, that would have resulted from any issuances of Additional Shares of Common Stock (other than deemed issuances of Additional Shares of Common Stock as a result of the issuance of such Option or Convertible Security) between the original adjustment date and such readjustment date.

(c) If the terms of any Option or Convertible Security (excluding Options or Convertible Securities which are themselves Exempted Securities), the issuance of which did not result in an adjustment to the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price pursuant to the terms of Subsection 4.4.4 (either because the consideration per share (determined pursuant to Subsection 4.4.5) of the Additional Shares of Common Stock subject thereto was equal to or greater than the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, then in effect, or because such Option or Convertible Security was issued before the Series C Original Issue Date), are revised after the Series C Original Issue Date as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase in the number of shares of Common Stock issuable upon the exercise, conversion or exchange of any such Option

or Convertible Security or (2) any decrease in the consideration payable to the Corporation upon such exercise, conversion or exchange, then such Option or Convertible Security, as so amended or adjusted, and the Additional Shares of Common Stock subject thereto (determined in the manner provided in Subsection 4.4.3(a)) shall be deemed to have been issued effective upon such increase or decrease becoming effective.

(d) Upon the expiration or termination of any unexercised Option or unconverted or unexchanged Convertible Security (or portion thereof) which resulted (either upon its original issuance or upon a revision of its terms) in an adjustment to the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price pursuant to the terms of Subsection 4.4.4, the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, shall be readjusted to such Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, respectively, as would have obtained had such Option or Convertible Security (or portion thereof) never been issued.

(e) If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, is calculable at the time such Option or Convertible Security is issued or amended but is subject to adjustment based upon subsequent events, any adjustment to the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price provided for in this Subsection 4.4.3 shall be effected at the time of such issuance or amendment based on such number of shares or amount of consideration without regard to any provisions for subsequent adjustments (and any subsequent adjustments shall be treated as provided in clauses (b) and (c) of this Subsection 4.4.3). If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, cannot be calculated at all at the time such Option or Convertible Security is issued or amended, any adjustment to the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price that would result under the terms of this Subsection 4.4.3 at the time of such issuance or amendment shall instead be effected at the time such number of shares and/or amount of consideration is first calculable (even if subject to subsequent adjustments), assuming for purposes of calculating such adjustment to the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, respectively, that such issuance or amendment took place at the time such calculation can first be made.

4.4.4 Adjustment of Conversion Price Upon Issuance of Additional Shares of Common Stock. In the event the Corporation shall at any time or from time to time after the Series C Original Issue Date issue Additional Shares of Common Stock (including Additional Shares of Common Stock deemed to be issued pursuant to Subsection 4.4.3), without consideration or for a consideration per share less than the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price in effect immediately prior to such issue, as the case may be, then the applicable Conversion Price shall be reduced, concurrently with such issue, to a price (calculated to the nearest one-hundredth of a cent) determined in accordance with the following formula:

$$CP_2 = CP_1 * (A + B) \div (A + C).$$

For purposes of the foregoing formula, the following definitions shall apply:

- (a) "CP<sub>2</sub>" shall mean the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, in effect immediately after such issue of Additional Shares of Common Stock;
- (b) "CP<sub>1</sub>" shall mean the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, in effect immediately prior to such issue of Additional Shares of Common Stock;
- (c) "A" shall mean the number of shares of Common Stock outstanding immediately prior to such issue of Additional Shares of Common Stock (treating for this purpose as outstanding all shares of Common Stock issuable upon exercise of Options outstanding immediately prior to such issue or upon conversion or exchange of Convertible Securities (including the Preferred Stock) outstanding (assuming exercise of any outstanding Options therefor) immediately prior to such issue);
- (d) "B" shall mean the number of shares of Common Stock that would have been issued if such Additional Shares of Common Stock had been issued at a price per share equal to CP<sub>1</sub> (determined by dividing the aggregate consideration received by the Corporation in respect of such issue by CP<sub>1</sub>); and
- (e) "C" shall mean the number of such Additional Shares of Common Stock issued in such transaction.

4.4.5 Determination of Consideration. For purposes of this Subsection 4.4, the consideration received by the Corporation for the issue of any Additional Shares of Common Stock shall be computed as follows:

- (a) Cash and Property: Such consideration shall:
  - (i) insofar as it consists of cash, be computed at the aggregate amount of cash received by the Corporation, excluding amounts paid or payable for accrued interest;
  - (ii) insofar as it consists of property other than cash, be computed at the fair market value thereof at the time of such issue, as determined in good faith by the Board of Directors of the Corporation; and
  - (iii) in the event Additional Shares of Common Stock are issued together with other shares or securities or other assets of the Corporation for consideration which covers both, be the proportion of such consideration so received, computed as provided in clauses (i) and (ii) above, as determined in good faith by the Board of Directors of the Corporation.

(b) Options and Convertible Securities. The consideration per share received by the Corporation for Additional Shares of Common Stock deemed to have been issued pursuant to Subsection 4.4.3, relating to Options and Convertible Securities, shall be determined by dividing:

- (i) the total amount, if any, received or receivable by the Corporation as consideration for the issue of such Options or Convertible Securities, plus the minimum aggregate amount of additional consideration (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such consideration) payable to the Corporation upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities, by
- (ii) the maximum number of shares of Common Stock (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities.

4.4.6 Multiple Closing Dates. In the event the Corporation shall issue on more than one date Additional Shares of Common Stock that are a part of one transaction or a series of related transactions and that would result in an adjustment to the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price pursuant to the terms of Subsection 4.4.4, and such issuance dates occur within a period of no more than

ninety (90) days from the first such issuance to the final such issuance, then, upon the final such issuance, the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, shall be readjusted to give effect to all such issuances as if they occurred on the date of the first such issuance (and without giving effect to any additional adjustments as a result of any such subsequent issuances within such period).

4.5 Adjustment for Stock Splits and Combinations. If the Corporation shall at any time or from time to time after the Series C Original Issue Date effect a subdivision of the outstanding Common Stock, the Series A Conversion Price, Series B Conversion Price and Series C Conversion Price in effect immediately before that subdivision shall be proportionately decreased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be increased in proportion to such increase in the aggregate number of shares of Common Stock outstanding. If the Corporation shall at any time or from time to time after the Series C Original Issue Date combine the outstanding shares of Common Stock, the Series A Conversion Price, Series B Conversion Price and Series C Conversion Price in effect immediately before the combination shall be proportionately increased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be decreased in proportion to such decrease in the aggregate number of shares of Common Stock outstanding. Any adjustment under this subsection shall become effective at the close of business on the date the subdivision or combination becomes effective.

4.6 Adjustment for Certain Dividends and Distributions. In the event the Corporation at any time or from time to time after the Series C Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable on the Common Stock in additional shares of Common Stock, then and in each such event the Series A Conversion Price, Series B Conversion Price and Series C Conversion Price in effect immediately before such event shall be decreased as of the time of such issuance or, in the event such a record date shall have been fixed, as of the close of business on such record date, by multiplying the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, then in effect by a fraction:

(1) the numerator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date, and

(2) the denominator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date plus the number of shares of Common Stock issuable in payment of such dividend or distribution.

Notwithstanding the foregoing, (a) if such record date shall have been fixed and such dividend is not fully paid or if such distribution is not fully made on the date fixed therefor, the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, shall be recomputed accordingly as of the close of business on such record date and thereafter the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, respectively, shall be adjusted pursuant to this subsection as of the time of actual payment of such dividends or distributions; and (b) that no such adjustment shall be made if the holders of



Preferred Stock simultaneously receive a dividend or other distribution of shares of Common Stock in a number equal to the number of shares of Common Stock as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event.

4.7 Adjustments for Other Dividends and Distributions. In the event the Corporation at any time or from time to time after the Series C Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable in securities of the Corporation (other than a distribution of shares of Common Stock in respect of outstanding shares of Common Stock) or in other property and the provisions of Section 1 do not apply to such dividend or distribution, then and in each such event the holders of Preferred Stock shall receive, simultaneously with the distribution to the holders of Common Stock, a dividend or other distribution of such securities or other property in an amount equal to the amount of such securities or other property as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event.

4.8 Adjustment for Merger or Reorganization, etc. Subject to the provisions of Subsection 2.3, if there shall occur any reorganization, recapitalization, reclassification, consolidation or merger involving the Corporation in which the Common Stock (but not the Preferred Stock) is converted into or exchanged for securities, cash or other property (other than a transaction covered by Subsections 4.4, 4.6 or 4.7), then, following any such reorganization, recapitalization, reclassification, consolidation or merger, each share of Preferred Stock shall thereafter be convertible in lieu of the Common Stock into which it was convertible prior to such event into the kind and amount of securities, cash or other property which a holder of the number of shares of Common Stock of the Corporation issuable upon conversion of one share of such series of Preferred Stock immediately prior to such reorganization, recapitalization, reclassification, consolidation or merger would have been entitled to receive pursuant to such transaction; and, in such case, appropriate adjustment (as determined in good faith by the Board of Directors of the Corporation) shall be made in the application of the provisions in this Section 4 with respect to the rights and interests thereafter of the holders of the Preferred Stock, to the end that the provisions set forth in this Section 4 (including provisions with respect to changes in and other adjustments of the Series A Conversion Price, Series B Conversion Price and Series C Conversion Price) shall thereafter be applicable, as nearly as reasonably may be, in relation to any securities or other property thereafter deliverable upon the conversion of the Preferred Stock. For the avoidance of doubt, nothing in this Subsection 4.8 shall be construed as preventing the holders of Preferred Stock from seeking any appraisal rights to which they are otherwise entitled under the DGCL in connection with a merger triggering an adjustment hereunder, nor shall this Subsection 4.8 be deemed conclusive evidence of the fair value of the shares of any series of Preferred Stock in any such appraisal proceeding.

4.9 Certificate as to Adjustments. Upon the occurrence of each adjustment or readjustment of the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price pursuant to this Section 4, the Corporation at its expense shall, as promptly as reasonably practicable but in any event not later than ten (10) days thereafter, compute such adjustment or readjustment in accordance with the terms hereof and furnish to

each holder of Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock, respectively, a certificate setting forth such adjustment or readjustment (including the kind and amount of securities, cash or other property into which the Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock, respectively, is convertible) and showing in detail the facts upon which such adjustment or readjustment is based. The Corporation shall, as promptly as reasonably practicable after the written request at any time of any holder of Preferred Stock (but in any event not later than ten (10) days thereafter), furnish or cause to be furnished to such holder a certificate setting forth (i) the Series A Conversion Price, Series B Conversion Price or Series C Conversion Price, as applicable, then in effect, and (ii) the number of shares of Common Stock and the amount, if any, of other securities, cash or property which then would be received upon the conversion of Series A Preferred Stock, Series B Preferred Stock or Series C Preferred Stock, as applicable.

4.10 Notice of Record Date. In the event:

(a) the Corporation shall take a record of the holders of its Common Stock (or other capital stock or securities at the time issuable upon conversion of the Preferred Stock) for the purpose of entitling or enabling them to receive any dividend or other distribution, or to receive any right to subscribe for or purchase any shares of capital stock of any class or any other securities, or to receive any other security; or

(b) of any capital reorganization of the Corporation, any reclassification of the Common Stock of the Corporation, or any Deemed Liquidation Event; or

(c) of the voluntary or involuntary dissolution, liquidation or winding-up of the Corporation,

then, and in each such case, the Corporation will send or cause to be sent to the holders of the Preferred Stock a notice specifying, as the case may be, (i) the record date for such dividend, distribution or right, and the amount and character of such dividend, distribution or right, or (ii) the effective date on which such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up is proposed to take place, and the time, if any is to be fixed, as of which the holders of record of Common Stock (or such other capital stock or securities at the time issuable upon the conversion of the Preferred Stock) shall be entitled to exchange their shares of Common Stock (or such other capital stock or securities) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up, and the amount per share and character of such exchange applicable to the Preferred Stock and the Common Stock. Such notice shall be sent at least ten (10) days prior to the record date or effective date for the event specified in such notice.

5. Mandatory Conversion.

5.1 Trigger Events.

5.1.1 Upon the closing of the sale of shares of Common Stock to the public at a price of at least \$8.0496 per share (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Common Stock), in a firm-commitment underwritten public offering pursuant to an effective registration statement under the Securities Act of 1933, as amended, resulting in at least \$40,000,000 of proceeds, net of the underwriting discount and commissions, to the Corporation (a “**Qualified Public Offering**,” and the time of such closing is referred to herein as the “**QPO Mandatory Conversion Time**”), then all outstanding shares of Preferred Stock shall automatically be converted into shares of Common Stock, at the then effective conversion rate as calculated pursuant to Subsection 4.1.1.

5.1.2 Notwithstanding Subsection 5.1.1, all outstanding shares of Series A Preferred Stock shall automatically be converted into shares of Common Stock, at the then effective conversion rate as calculated pursuant to Subsection 4.1.1 upon the date and time, or the occurrence of an event, specified by vote or written consent of the holders of at least a majority of the then outstanding shares of Series A Preferred Stock (the date and time specified or the time of the event specified in such vote or written consent is referred to herein as the “**Series A Mandatory Conversion Time**”).

5.1.3 Notwithstanding Subsection 5.1.1, all outstanding shares of Series B Preferred Stock shall automatically be converted into shares of Common Stock, at the then effective conversion rate as calculated pursuant to Subsection 4.1.1 upon the date and time, or the occurrence of an event, specified by vote or written consent of the holders of at least 57% of the then outstanding shares of Series B Preferred Stock (the date and time specified or the time of the event specified in such vote or written consent is referred to herein as the “**Series B Mandatory Conversion Time**”).

5.1.4 Notwithstanding Subsection 5.1.1, all outstanding shares of Series C Preferred Stock shall automatically be converted into shares of Common Stock, at the then effective conversion rate as calculated pursuant to Subsection 4.1.1 upon the date and time, or the occurrence of an event, specified by vote or written consent of the Series C Majority (the date and time specified or the time of the event specified in such vote or written consent is referred to herein as the “**Series C Mandatory Conversion Time**” and, together with the QPO Mandatory Conversion Time, the Series A Mandatory Conversion Time and the Series B Mandatory Conversion Time, the “**Mandatory Conversion Time**”).

5.1.5 Any shares converted pursuant to Subsection 5.1.1, 5.1.2, 5.1.3 or 5.1.4 may not be reissued by the Corporation.

5.2 Procedural Requirements. All holders of record of shares of Preferred Stock being converted shall be sent written notice of the Mandatory Conversion Time and the place designated for mandatory conversion of all applicable shares of Preferred Stock pursuant to this Section 5. Such notice need not be sent in advance of the occurrence of the Mandatory Conversion Time. Upon receipt of such notice, each holder of shares of Preferred Stock affected by such conversion shall surrender his, her or its certificate or certificates for all such shares (or, if such holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation at the place designated in

such notice. If so required by the Corporation, any certificates surrendered for conversion shall be endorsed or accompanied by written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or by his, her or its attorney duly authorized in writing. All rights with respect to the Preferred Stock converted pursuant to Subsection 5.1, including the rights, if any, to receive notices and vote (other than as a holder of Common Stock), will terminate at the Mandatory Conversion Time (notwithstanding the failure of the holder or holders thereof to surrender any certificates at or prior to such time), except only the rights of the holders thereof, upon surrender of any certificate or certificates of such holders (or lost certificate affidavit and agreement) therefor, to receive the items provided for in the next sentence of this Subsection 5.2. As soon as practicable after the Mandatory Conversion Time and, if applicable, the surrender of any certificate or certificates (or lost certificate affidavit and agreement) for Preferred Stock so converted, the Corporation shall (a) issue and deliver to such holder, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable on such conversion in accordance with the provisions hereof and (b) pay cash as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and the payment of any declared but unpaid dividends on the shares of the Preferred Stock converted. Such converted Preferred Stock shall be retired and cancelled and may not be reissued as shares of such series, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of the affected Preferred Stock accordingly.

6. Redeemed or Otherwise Acquired Shares. Any shares of Preferred Stock that are redeemed or otherwise acquired by the Corporation or any of its subsidiaries shall be automatically and immediately cancelled and retired and shall not be reissued, sold or transferred. Neither the Corporation nor any of its subsidiaries may exercise any voting or other rights granted to the holders of Preferred Stock following redemption.

7. Notices. Any notice required or permitted by the provisions of this Article Fourth to be given to a holder of shares of Preferred Stock shall be mailed, postage prepaid, to the post office address last shown on the records of the Corporation, or given by electronic communication in compliance with the provisions of the General Corporation Law, and shall be deemed sent upon such mailing or electronic transmission.

**FIFTH:** Subject to any additional vote required by the Certificate of Incorporation or Bylaws, in furtherance and not in limitation of the powers conferred by statute, the Board of Directors is expressly authorized to make, repeal, alter, amend and rescind any or all of the Bylaws of the Corporation.

**SIXTH:** Subject to any additional vote required by the Certificate of Incorporation, the number of directors of the Corporation shall be determined in the manner set forth in the Bylaws of the Corporation.

**SEVENTH:** Elections of directors need not be by written ballot unless the Bylaws of the Corporation shall so provide.

**EIGHTH:** Meetings of stockholders may be held within or without the State of Delaware, as the Bylaws of the Corporation may provide. The books of the Corporation may be kept outside the State of Delaware at such place or places as may be designated from time to time by the Board of Directors or in the Bylaws of the Corporation.

**NINTH:** To the fullest extent permitted by law, a director of the Corporation shall not be personally liable to the Corporation or its stockholders for monetary damages for breach of fiduciary duty as a director. If the General Corporation Law or any other law of the State of Delaware is amended after approval by the stockholders of this Article Ninth to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of a director of the Corporation shall be eliminated or limited to the fullest extent permitted by the General Corporation Law as so amended.

Any repeal or modification of the foregoing provisions of this Article Ninth by the stockholders of the Corporation shall not adversely affect any right or protection of a director of the Corporation existing at the time of, or increase the liability of any director of the Corporation with respect to any acts or omissions of such director occurring prior to, such repeal or modification.

**TENTH:** To the fullest extent permitted by applicable law, the Corporation is authorized to provide indemnification of (and advancement of expenses to) directors, officers and agents of the Corporation (and any other persons to which General Corporation Law permits the Corporation to provide indemnification) through Bylaw provisions, agreements with such agents or other persons, vote of stockholders or disinterested directors or otherwise, in excess of the indemnification and advancement otherwise permitted by Section 145 of the General Corporation Law.

Any amendment, repeal or modification of the foregoing provisions of this Article Tenth shall not adversely affect any right or protection of any director, officer or other agent of the Corporation existing at the time of such amendment, repeal or modification.

**ELEVENTH:** The Corporation renounces, to the fullest extent permitted by law, any interest or expectancy of the Corporation in, or in being offered an opportunity to participate in, any Excluded Opportunity. An “**Excluded Opportunity**” is any matter, transaction or interest that is presented to, or acquired, created or developed by, or which otherwise comes into the possession of (i) any director of the Corporation who is not an employee of the Corporation or any of its subsidiaries, or (ii) any holder of Preferred Stock or any partner, member, director, stockholder, employee, affiliate or agent of any such holder, other than someone who is an employee of the Corporation or any of its subsidiaries (collectively, “**Covered Persons**”), unless such matter, transaction or interest is presented to, or acquired, created or developed by, or otherwise comes into the possession of, a Covered Person expressly and solely in such Covered Person’s capacity as a director of the Corporation. Any repeal or modification of this Article Eleventh will only be prospective and will not affect the rights under this Article Eleventh in effect at the time of the occurrence of any actions or omissions to act giving rise to liability. Notwithstanding anything to the contrary contained elsewhere in this Certificate of Incorporation, the written consent or affirmative vote of the Requisite Holders will be required to amend or repeal, or to adopt any provisions inconsistent with, this Article Eleventh.

**TWELFTH:** Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery in the State of Delaware shall be the sole and exclusive forum for any stockholder (including a beneficial owner) to bring (i) any derivative action or proceeding brought on behalf of the Corporation, (ii) any action asserting a claim of breach of fiduciary duty owed by any director, officer or other employee of the Corporation to the Corporation or the Corporation's stockholders, (iii) any action asserting a claim against the Corporation, its directors, officers or employees arising pursuant to any provision of the Delaware General Corporation Law or the Corporation's certificate of incorporation or bylaws or (iv) any action asserting a claim against the Corporation, its directors, officers or employees governed by the internal affairs doctrine, except for, as to each of (i) through (iv) above, any claim as to which the Court of Chancery determines that there is an indispensable party not subject to the jurisdiction of the Court of Chancery (and the indispensable party does not consent to the personal jurisdiction of the Court of Chancery within ten days following such determination), which is vested in the exclusive jurisdiction of a court or forum other than the Court of Chancery, or for which the Court of Chancery does not have subject matter jurisdiction. If any provision or provisions of this Article Twelfth shall be held to be invalid, illegal or unenforceable as applied to any person or entity or circumstance for any reason whatsoever, then, to the fullest extent permitted by law, the validity, legality and enforceability of such provisions in any other circumstance and of the remaining provisions of this Article Twelfth (including, without limitation, each portion of any sentence of this Article Twelfth containing any such provision held to be invalid, illegal or unenforceable that is not itself held to be invalid, illegal or unenforceable) and the application of such provision to other persons or entities and circumstances shall not in any way be affected or impaired thereby.

\* \* \*

3. That the foregoing amendment and restatement was approved by the holders of the requisite number of shares of this corporation in accordance with Section 228 of the General Corporation Law.

4. That this Third Amended and Restated Certificate of Incorporation, which restates and integrates and further amends the provisions of this Corporation's Certificate of Incorporation, has been duly adopted in accordance with Sections 242 and 245 of the General Corporation Law.

**IN WITNESS WHEREOF**, this Third Amended and Restated Certificate of Incorporation has been executed by a duly authorized officer of this corporation on this 9th day of January, 2020.

By: /s/ Geoffrey McDonough

Name: Geoffrey McDonough

Title: President and Chief Executive Officer

**BY-LAWS**  
**of**  
**TORUS THERAPEUTICS, INC.**  
(the "Corporation")

1. Stockholders

(a) Annual Meeting. The annual meeting of stockholders shall be held for the election of directors each year at such place, date and time as shall be designated by the Board of Directors. Any other proper business may be transacted at the annual meeting. If no date for the annual meeting is established or said meeting is not held on the date established as provided above, a special meeting in lieu thereof may be held or there may be action by written consent of the stockholders on matters to be voted on at the annual meeting, and such special meeting or written consent shall have for the purposes of these By-laws or otherwise all the force and effect of an annual meeting.

(b) Special Meetings. Special meetings of stockholders may be called by the Chief Executive Officer, if one is elected, or, if there is no Chief Executive Officer, a President, or by the Board of Directors, but such special meetings may not be called by any other person or persons. The call for the meeting shall state the place, date, hour and purposes of the meeting. Only the purposes specified in the notice of special meeting shall be considered or dealt with at such special meeting.

(c) Notice of Meetings. Whenever stockholders are required or permitted to take any action at a meeting, a notice stating the place, if any, date and hour of the meeting, the means of remote communications, if any, by which stockholders and proxyholders may be deemed to be present and vote at such meeting, and, in the case of a special meeting, the purpose or purposes of the meeting, shall be given by the Secretary (or other person authorized by these By-laws or by law) not less than ten (10) nor more than sixty (60) days before the meeting to each stockholder entitled to vote thereat and to each stockholder who, under the Certificate of Incorporation or under these By-laws is entitled to such notice. If mailed, notice is given when deposited in the mail, postage prepaid, directed to such stockholder at such stockholder's address as it appears in the records of the Corporation. Without limiting the manner by which notice otherwise may be effectively given to stockholders, any notice to stockholders may be given by electronic transmission in the manner provided in Section 232 of the Delaware General Corporation Law (the "DGCL").

If a meeting is adjourned to another time or place, notice need not be given of the adjourned meeting if the time and place, if any, and the means of remote communications, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such adjourned meeting are announced at the meeting at which the adjournment is taken, except that if the adjournment is for more than thirty (30) days, or if after the adjournment a new record date is fixed for the adjourned meeting, notice of the adjourned meeting shall be given to each stockholder of record entitled to vote at the meeting.



(d) Quorum. The holders of a majority in interest of all stock issued, outstanding and entitled to vote at a meeting, present in person or represented by proxy, shall constitute a quorum. Any meeting may be adjourned from time to time by a majority of the votes properly cast upon the question, whether or not a quorum is present. The stockholders present at a duly constituted meeting may continue to transact business until adjournment notwithstanding the withdrawal of enough stockholders to reduce the voting shares below a quorum.

(e) Voting and Proxies. Except as otherwise provided by the Certificate of Incorporation or by law, each stockholder entitled to vote at any meeting of stockholders shall be entitled to one vote for each share of stock held by such stockholder which has voting power upon the matter in question. Each stockholder entitled to vote at a meeting of stockholders or to express consent or dissent to corporate action in writing without a meeting may authorize another person or persons to act for such stockholder by either written proxy or by a transmission permitted by Section 212(c) of the DGCL, but no proxy shall be voted or acted upon after three years from its date, unless the proxy provides for a longer period or is irrevocable and coupled with an interest. Proxies shall be filed with the Secretary of the meeting, or of any adjournment thereof. Except as otherwise limited therein, proxies shall entitle the persons authorized thereby to vote at any adjournment of such meeting.

(f) Action at Meeting. When a quorum is present, any matter before the meeting shall be decided by vote of the holders of a majority of the shares of stock voting on such matter except where a larger vote is required by law, by the Certificate of Incorporation or by these By-laws. Any election of directors by stockholders shall be determined by a plurality of the votes cast, except where a larger vote is required by law, by the Certificate of Incorporation or by these By-laws. The Corporation shall not directly or indirectly vote any share of its own stock; provided, however, that the Corporation may vote shares which it holds in a fiduciary capacity to the extent permitted by law.

(g) Presiding Officer. Meetings of stockholders shall be presided over by the Chairman of the Board, if one is elected, or in his or her absence, the Vice Chairman of the Board, if one is elected, or if neither is elected or in their absence, a President. The Board of Directors shall have the authority to appoint a temporary presiding officer to serve at any meeting of the stockholders if the Chairman of the Board, the Vice Chairman of the Board or a President is unable to do so for any reason.

(h) Conduct of Meetings. The Board of Directors may adopt by resolution such rules and regulations for the conduct of the meeting of stockholders as it shall deem appropriate. Except to the extent inconsistent with such rules and regulations as adopted by the Board of Directors, the presiding officer of any meeting of stockholders shall have the right and authority to prescribe such rules, regulations and procedures and to do all such acts as, in the judgment of such chairman, are appropriate for the proper conduct of the meeting. Such rules, regulations or procedures, whether adopted by the Board of Directors or prescribed by the presiding officer of the meeting, may include, without limitation, the following: (i) the

establishment of an agenda or order of business for the meeting; (ii) rules and procedures for maintaining order at the meeting and the safety of those present; (iii) limitations on attendance at or participation in the meeting to stockholders of record of the Corporation, their duly authorized and constituted proxies or such other persons as the chairman of the meeting shall determine; (iv) restrictions on entry to the meeting after the time fixed for the commencement thereof; and (v) limitations on the time allotted to questions or comments by participants. Unless and to the extent determined by the Board of Directors or the presiding officer of the meeting, meetings of stockholders shall not be required to be held in accordance with the rules of parliamentary procedure.

(i) Action without a Meeting. Unless otherwise provided in the Certificate of Incorporation, any action required or permitted by law to be taken at any annual or special meeting of stockholders, may be taken without a meeting, without prior notice and without a vote, if a consent or consents in writing, setting forth the action so taken, shall be signed by the holders of outstanding stock having not less than the minimum number of votes that would be necessary to authorize or take such action at a meeting at which all shares entitled to vote thereon were present and voted and shall be delivered to the Corporation by delivery to its registered office, by hand or by certified mail, return receipt requested, or to the Corporation's principal place of business or to the officer of the Corporation having custody of the minute book. Every written consent shall bear the date of signature and no written consent shall be effective unless, within sixty (60) days of the earliest dated consent delivered pursuant to these By-laws, written consents signed by a sufficient number of stockholders entitled to take action are delivered to the Corporation in the manner set forth in these By-laws. Prompt notice of the taking of the corporate action without a meeting by less than unanimous written consent shall be given to those stockholders who have not consented in writing.

(j) Stockholder Lists. The officer who has charge of the stock ledger of the Corporation shall prepare and make, at least ten (10) days before every meeting of stockholders, a complete list of the stockholders entitled to vote at the meeting, arranged in alphabetical order, and showing the address of each stockholder and the number of shares registered in the name of each stockholder. Nothing contained in this Section 1(j) shall require the Corporation to include electronic mail addresses or other electronic contact information on such list. Such list shall be open to the examination of any stockholder, for any purpose germane to the meeting, for a period of at least ten (10) days prior to the meeting in the manner provided by law. The list shall also be open to the examination of any stockholder during the whole time of the meeting as provided by law.

## 2. Directors

(a) Powers. The business of the Corporation shall be managed by or under the direction of a Board of Directors who may exercise all the powers of the Corporation except as otherwise provided by law, by the Certificate of Incorporation or by these By-laws. In the event of a vacancy in the Board of Directors, the remaining directors, except as otherwise provided by law, may exercise the powers of the full Board until the vacancy is filled.

(b) Number and Qualification. Unless otherwise provided in the Certificate of Incorporation or in these By-laws, the number of directors which shall constitute the whole board shall be determined from time to time by resolution of the Board of Directors. Directors need not be stockholders.

(c) Vacancies; Reduction of Board. A majority of the directors then in office, although less than a quorum, or a sole remaining Director, may fill vacancies in the Board of Directors occurring for any reason and newly created directorships resulting from any increase in the authorized number of directors. In lieu of filling any vacancy, the Board of Directors may reduce the number of directors.

(d) Tenure. Except as otherwise provided by law, by the Certificate of Incorporation or by these By-laws, directors shall hold office until their successors are elected and qualified or until their earlier resignation or removal. Any director may resign at any time upon notice given in writing or by electronic transmission to the Corporation. Such resignation shall be effective upon receipt unless it is specified to be effective at some other time or upon the happening of some other event.

(e) Removal. To the extent permitted by law, a director may be removed from office with or without cause by vote of the holders of a majority of the shares of stock entitled to vote in the election of directors.

(f) Meetings. Regular meetings of the Board of Directors may be held without notice at such time, date and place as the Board of Directors may from time to time determine. Special meetings of the Board of Directors may be called, orally or in writing, by the Chief Executive Officer, if one is elected, or, if there is no Chief Executive Officer, the President, or by any Director, designating the time, date and place thereof. Directors may participate in meetings of the Board of Directors by means of conference telephone or other communications equipment by means of which all directors participating in the meeting can hear each other, and participation in a meeting in accordance herewith shall constitute presence in person at such meeting.

(g) Notice of Meetings. Notice of the time, date and place of all special meetings of the Board of Directors shall be given to each director by the Secretary, or Assistant Secretary, or in case of the death, absence, incapacity or refusal of such persons, by the officer or one of the directors calling the meeting. Notice shall be given to each director in person, by telephone, or by facsimile, electronic mail or other form of electronic communications, sent to such director's business or home address at least twenty-four (24) hours in advance of the meeting, or by written notice mailed to such director's business or home address at least forty-eight (48) hours in advance of the meeting.

(h) Quorum. At any meeting of the Board of Directors, a majority of the total number of directors shall constitute a quorum for the transaction of business. Less than a quorum may adjourn any meeting from time to time and the meeting may be held as adjourned without further notice.

(i) Action at Meeting. At any meeting of the Board of Directors at which a quorum is present, unless otherwise provided in the following sentence, a majority of the directors present may take any action on behalf of the Board of Directors, unless a larger number is required by law, by the Certificate of Incorporation or by these By-laws. So long as there are two (2) or fewer Directors, any action to be taken by the Board of Directors shall require the approval of all Directors.

(j) Action by Consent. Any action required or permitted to be taken at any meeting of the Board of Directors may be taken without a meeting if all members of the Board of Directors consent thereto in writing or by electronic transmission, and the writing or writings or electronic transmission or transmissions are filed with the records of the meetings of the Board of Directors. Such filing shall be in paper form if the minutes are maintained in paper form and shall be in electronic form if the minutes are maintained in electronic form.

(k) Committees. The Board of Directors may, by resolution passed by a majority of the whole Board of Directors, establish one or more committees, each committee to consist of one or more directors. The Board of Directors may designate one or more directors as alternate members of any committee, who may replace any absent or disqualified member at any meeting of the committee. In the absence or disqualification of a member of a committee, the member or members thereof present at any meeting and not disqualified from voting, whether or not such member or members constitute a quorum, may unanimously appoint another member of the Board of Directors to act at the meeting in the place of any such absent or disqualified member.

Any such committee, to the extent permitted by law and to the extent provided in the resolution of the Board of Directors, shall have and may exercise all the powers and authority of the Board of Directors in the management of the business and affairs of the Corporation, and may authorize the seal of the Corporation to be affixed to all papers which may require it; but no such committee shall have the power or authority in reference to the following: (i) approving or adopting, or recommending to the stockholders, any action or matter expressly required by the DGCL to be submitted to stockholders for approval or (ii) adopting, amending or repealing any provision of these By-laws.

Except as the Board of Directors may otherwise determine, any such committee may make rules for the conduct of its business, but in the absence of such rules its business shall be conducted so far as possible in the same manner as is provided in these By-laws for the Board of Directors. All members of such committees shall hold their committee offices at the pleasure of the Board of Directors, and the Board may abolish any committee at any time.

### 3. Officers

(a) Enumeration. The officers of the Corporation shall consist of one or more Presidents (who, if there is more than one, shall be referred to as Co-Presidents), a Secretary, and such other officers, including, without limitation, a Treasurer, a Chief Executive Officer and one or more Vice Presidents (including Executive Vice Presidents or Senior Vice Presidents), Assistant Vice Presidents, Assistant Treasurers and Assistant Secretaries, as the Board of Directors may determine. The Board of Directors may elect from among its members a Chairman of the Board and a Vice Chairman of the Board.

(b) Election. The Presidents, Treasurer and Secretary shall be elected annually by the Board of Directors at their first meeting following the annual meeting of stockholders. Other officers may be chosen by the Board of Directors at such meeting or at any other meeting.

(c) Qualification. No officer need be a stockholder or Director. Any two or more offices may be held by the same person. Any officer may be required by the Board of Directors to give bond for the faithful performance of such officer's duties in such amount and with such sureties as the Board of Directors may determine.

(d) Tenure. Except as otherwise provided by the Certificate of Incorporation or by these By-laws, each of the officers of the Corporation shall hold office until the first meeting of the Board of Directors following the next annual meeting of stockholders and until such officer's successor is elected and qualified or until such officer's earlier resignation or removal. Any officer may resign by delivering his or her written resignation to the Corporation, and such resignation shall be effective upon receipt unless it is specified to be effective at some other time or upon the happening of some other event.

(e) Removal. The Board of Directors may remove any officer with or without cause by a vote of a majority of the directors then in office.

(f) Vacancies. Any vacancy in any office may be filled for the unexpired portion of the term by the Board of Directors.

(g) Chairman of the Board and Vice Chairman. Unless otherwise provided by the Board of Directors, the Chairman of the Board of Directors, if one is elected, shall preside, when present, at all meetings of the stockholders and the Board of Directors. The Chairman of the Board shall have such other powers and shall perform such duties as the Board of Directors may from time to time designate.

Unless otherwise provided by the Board of Directors, in the absence of the Chairman of the Board, the Vice Chairman of the Board, if one is elected, shall preside, when present, at all meetings of the stockholders and the Board of Directors. The Vice Chairman of the Board shall have such other powers and shall perform such duties as the Board of Directors may from time to time designate.

(h) Chief Executive Officer. The Chief Executive Officer, if one is elected, shall have such powers and shall perform such duties as the Board of Directors may from time to time designate.

(i) Presidents. The Presidents shall, subject to the direction of the Board of Directors, each have general supervision and control of the Corporation's business and any action that would typically be taken by a President may be taken by any Co-President. If there is no Chairman of the Board or Vice Chairman of the Board, a President shall preside, when present, at all meetings of stockholders and the Board of Directors. The Presidents shall have such other powers and shall perform such duties as the Board of Directors may from time to time designate.

(j) Vice Presidents and Assistant Vice Presidents. Any Vice President (including any Executive Vice President or Senior Vice President) and any Assistant Vice President shall have such powers and shall perform such duties as the Board of Directors may from time to time designate.

(k) Treasurer and Assistant Treasurers. The Treasurer shall, subject to the direction of the Board of Directors, have general charge of the financial affairs of the Corporation and shall cause to be kept accurate books of account. The Treasurer shall have custody of all funds, securities, and valuable documents of the Corporation, except as the Board of Directors may otherwise provide. The Treasurer shall have such other powers and shall perform such duties as the Board of Directors may from time to time designate.

Any Assistant Treasurer shall have such powers and perform such duties as the Board of Directors may from time to time designate.

(l) Secretary and Assistant Secretaries. The Secretary shall record the proceedings of all meetings of the stockholders and the Board of Directors (including committees of the Board) in books kept for that purpose. In the absence of the Secretary from any such meeting an Assistant Secretary, or if such person is absent, a temporary secretary chosen at the meeting, shall record the proceedings thereof. The Secretary shall have charge of the stock ledger (which may, however, be kept by any transfer or other agent of the Corporation) and shall have such other duties and powers as may be designated from time to time by the Board of Directors.

Any Assistant Secretary shall have such powers and perform such duties as the Board of Directors may from time to time designate.

(m) Other Powers and Duties. Subject to these By-laws, each officer of the Corporation shall have in addition to the duties and powers specifically set forth in these By-laws, such duties and powers as are customarily incident to such officer's office, and such duties and powers as may be designated from time to time by the Board of Directors.

#### 4. Capital Stock

(a) Certificates of Stock. Each stockholder shall be entitled to a certificate of the capital stock of the Corporation in such form as may from time to time be prescribed by the Board of Directors. Such certificate shall be signed by a President or a Vice President, and by the Treasurer or an Assistant Treasurer, or the Secretary or an Assistant Secretary. Such signatures may be a facsimile. In case any officer, transfer agent or registrar who has signed or whose facsimile signature has been placed on such certificate shall have ceased to be such officer, transfer agent or registrar before such certificate is issued, it may be issued by the Corporation with the same effect as if such person were such officer, transfer agent or registrar at the time of its issue. Every certificate for shares of stock which are subject to any restriction on transfer and every certificate issued when the Corporation is authorized to issue more than one class or series of stock shall contain such legend with respect thereto as is required by law. The Corporation shall be permitted to issue fractional shares.

(b) Transfers. Subject to any restrictions on transfer, shares of stock may be transferred on the books of the Corporation by the surrender to the Corporation or its transfer agent of the certificate therefor properly endorsed or accompanied by a written assignment or power of attorney properly executed, with transfer stamps (if necessary) affixed, and with such proof of the authenticity of signature as the Corporation or its transfer agent may reasonably require.

(c) Record Holders. Except as may otherwise be required by law, by the Certificate of Incorporation or by these By-laws, the Corporation shall be entitled to treat the record holder of stock as shown on its books as the owner of such stock for all purposes, including the payment of dividends and the right to vote with respect thereto, regardless of any transfer, pledge or other disposition of such stock, until the shares have been transferred on the books of the Corporation in accordance with the requirements of these By-laws.

It shall be the duty of each stockholder to notify the Corporation of such stockholder's post office address.

(d) Record Date. In order that the Corporation may determine the stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof, or to consent to corporate action in writing without a meeting, or entitled to receive payment of any dividend or other distribution or allotment of any rights, or entitled to exercise any rights in respect of any change, conversion or exchange of stock or for the purpose of any other lawful action, the Board of Directors may fix, in advance, a record date, which shall not precede the date on which it is established, and which shall not be more than sixty (60) nor less than ten (10) days before the date of such meeting, more than ten (10) days after the date on which the record date for stockholder consent without a meeting is established, nor more than sixty (60) days prior to any other action. In such case only stockholders of record on such record date shall be so entitled notwithstanding any transfer of stock on the books of the Corporation after the record date.

If no record date is fixed, (i) the record date for determining stockholders entitled to notice of or to vote at a meeting of stockholders shall be at the close of business on the day next preceding the day on which notice is given, or, if notice is waived, at the close of business on the day next preceding the day on which the meeting is held, (ii) the record date for determining stockholders entitled to consent to corporate action in writing without a meeting, when no prior action by the Board of Directors is necessary, shall be the first date on which a signed written consent setting forth the action taken or proposed to be taken is delivered to the Corporation by delivery to its registered office in this state, to its principal place of business, or to an officer or agent of the Corporation having custody of the book in which proceedings of meetings of stockholders are recorded, and (iii) the record date for determining stockholders for any other purpose shall be at the close of business on the day on which the Board of Directors adopts the resolution relating thereto.

(e) Lost Certificates. The Corporation may issue a new certificate of stock in the place of any certificate theretofore issued by it, alleged to have been lost, stolen or destroyed, and the Corporation may require the owner of the lost, stolen or destroyed certificate, or his legal representative, to give the Corporation a bond sufficient to indemnify it against any claim that may be made against it on account of the alleged loss, theft or destruction of any such certificate or the issuance of such new certificate.

## 5. Indemnification

(a) Definitions. For purposes of this Section 5:

(i) "Corporate Status" describes the status of a person who is serving or has served (A) as a Director of the Corporation, (B) as an Officer of the Corporation, (C) as a Non-Officer Employee of the Corporation, or (D) as a director, partner, trustee, officer, employee or agent of any other corporation, partnership, limited liability company, joint venture, trust, employee benefit plan, foundation, association, organization or other legal entity for which such person is or was serving at the request of the Corporation. For purposes of this Section 5(a)(i), a Director, Officer or Non-Officer Employee of the Corporation who is serving or has served as a director, partner, trustee, officer, employee or agent of a Subsidiary shall be deemed to be serving at the request of the Corporation. Notwithstanding the foregoing, "Corporate Status" shall not include the status of a person who is serving or has served as a director, officer, employee or agent of a constituent corporation absorbed in a merger or consolidation transaction with the Corporation with respect to such person's activities prior to said transaction, unless specifically authorized by the Board of Directors or the stockholders of the Corporation;

(ii) "Director" means any person who serves or has served the Corporation as a director on the Board of Directors of the Corporation;

(iii) "Disinterested Director" means, with respect to each Proceeding in respect of which indemnification is sought hereunder, a Director of the Corporation who is not and was not a party to such Proceeding;

(iv) "Expenses" means all reasonable attorneys fees, retainers, court costs, transcript costs, fees of expert witnesses, private investigators and professional advisors (including, without limitation, accountants and investment bankers), travel expenses, duplicating costs, printing and binding costs, costs of preparation of demonstrative evidence and other courtroom presentation aids and devices, costs incurred in connection with document review, organization, imaging and computerization, telephone charges, postage, delivery service fees, and all other disbursements, costs or expenses of the type customarily incurred in connection with prosecuting, defending, preparing to prosecute or defend, investigating, being or preparing to be a witness in, settling or otherwise participating in, a Proceeding;

(v) "Liabilities" means judgments, damages, liabilities, losses, penalties, excise taxes, fines and amounts paid in settlement;

(vi) "Non-Officer Employee" means any person who serves or has served as an employee or agent of the Corporation, but who is not or was not a Director or Officer;



(vii) "Officer" means any person who serves or has served the Corporation as an officer of the Corporation appointed by the Board of Directors of the Corporation;

(viii) "Proceeding" means any threatened, pending or completed action, suit, arbitration, alternate dispute resolution mechanism, inquiry, investigation, administrative hearing or other proceeding, whether civil, criminal, administrative, arbitrate or investigative; and

(ix) "Subsidiary" shall mean any corporation, partnership, limited liability company, joint venture, trust or other entity of which the Corporation owns (either directly or through or together with another Subsidiary of the Corporation) either (i) a general partner, managing member or other similar interest or (ii) (A) 50% or more of the voting power of the voting capital equity interests of such corporation, partnership, limited liability company, joint venture or other entity, or (B) 50% or more of the outstanding voting capital stock or other voting equity interests of such corporation, partnership, limited liability company, joint venture or other entity.

(b) Indemnification of Directors and Officers. Subject to the operation of Section 5(d) of these By-laws, each Director and Officer shall be indemnified and held harmless by the Corporation to the fullest extent authorized by the DGCL, as the same exists or may hereafter be amended (but, in the case of any such amendment, only to the extent that such amendment permits the Corporation to provide broader indemnification rights than such law permitted the Corporation to provide prior to such amendment), and to the extent authorized in subsections (i) through (iv) of this Section 5(b).

(i) Actions, Suits and Proceedings Other than By or In the Right of the Corporation. Each Director and Officer shall be indemnified and held harmless by the Corporation against any and all Expenses and Liabilities that are incurred or paid by such Director or Officer or on such Director's or Officer's behalf in connection with any Proceeding or any claim, issue or matter therein (other than an action by or in the right of the Corporation), which such Director or Officer is, or is threatened to be made, a party to or participant in by reason of such Director's or Officer's Corporate Status, if such Director or Officer acted in good faith and in a manner such Director or Officer reasonably believed to be in or not opposed to the best interests of the Corporation and, with respect to any criminal proceeding, had no reasonable cause to believe his or her conduct was unlawful.

(ii) Actions, Suits and Proceedings By or In the Right of the Corporation. Each Director and Officer shall be indemnified and held harmless by the Corporation against any and all Expenses that are incurred by such Director or Officer or on such Director's or Officer's behalf in connection with any Proceeding or any claim, issue or matter therein by or in the right of the Corporation, which such Director or Officer is, or is threatened to be made, a party to or participant in by reason of such Director's or Officer's Corporate Status, if such Director or Officer acted in good faith and in a manner such Director or Officer reasonably believed to be in or not opposed to the best interests of the Corporation; provided, however, that no indemnification shall be

made under this Section 5(b)(ii) in respect of any claim, issue or matter as to which such Director or Officer shall have been finally adjudged by a court of competent jurisdiction to be liable to the Corporation, unless, and only to the extent that, the Court of Chancery or another court in which such Proceeding was brought shall determine upon application that, despite adjudication of liability, but in view of all the circumstances of the case, such Director or Officer is fairly and reasonably entitled to indemnification for such Expenses that such court deems proper.

(iii) Survival of Rights. The rights of indemnification provided by this Section 5(b) shall continue as to a Director or Officer after he or she has ceased to be a Director or Officer and shall inure to the benefit of his or her heirs, executors, administrators and personal representatives.

(iv) Actions by Directors or Officers. Notwithstanding the foregoing, the Corporation shall indemnify any Director or Officer seeking indemnification in connection with a Proceeding initiated by such Director or Officer only if such Proceeding (including any parts of such Proceeding not initiated by such Director or Officer) was authorized in advance by the Board of Directors of the Corporation, unless such Proceeding was brought to enforce such Officer's or Director's rights to indemnification or, in the case of Directors, advancement of Expenses under these By-laws in accordance with the provisions set forth herein.

(c) Indemnification of Non-Officer Employees. Subject to the operation of Section 5(d) of these By-laws, each Non-Officer Employee may, in the discretion of the Board of Directors of the Corporation, be indemnified by the Corporation to the fullest extent authorized by the DGCL, as the same exists or may hereafter be amended, against any or all Expenses and Liabilities that are incurred by such Non-Officer Employee or on such Non-Officer Employee's behalf in connection with any threatened, pending or completed Proceeding, or any claim, issue or matter therein, which such Non-Officer Employee is, or is threatened to be made, a party to or participant in by reason of such Non-Officer Employee's Corporate Status, if such Non-Officer Employee acted in good faith and in a manner such Non-Officer Employee reasonably believed to be in or not opposed to the best interests of the Corporation and, with respect to any criminal proceeding, had no reasonable cause to believe his or her conduct was unlawful. The rights of indemnification provided by this Section 5(c) shall exist as to a Non-Officer Employee after he or she has ceased to be a Non-Officer Employee and shall inure to the benefit of his or her heirs, personal representatives, executors and administrators. Notwithstanding the foregoing, the Corporation may indemnify any Non-Officer Employee seeking indemnification in connection with a Proceeding initiated by such Non-Officer Employee only if such Proceeding was authorized in advance by the Board of Directors of the Corporation.

(d) Determination. Unless ordered by a court, no indemnification shall be provided pursuant to this Section 5 to a Director, to an Officer or to a Non-Officer Employee unless a determination shall have been made that such person acted in good faith and in a manner such person reasonably believed to be in or not opposed to the best interests of the Corporation and, with respect to any criminal Proceeding, such person had no reasonable cause to believe his or her conduct was unlawful. Such determination shall be made by (i) a majority vote of the Disinterested Directors, even though less than a quorum of the Board of Directors, (ii) a

committee comprised of Disinterested Directors, such committee having been designated by a majority vote of the Disinterested Directors (even though less than a quorum), (iii) if there are no such Disinterested Directors, or if a majority of Disinterested Directors so directs, by independent legal counsel in a written opinion, or (iv) by the stockholders of the Corporation.

(e) Advancement of Expenses to Directors Prior to Final Disposition.

(i) The Corporation shall advance all Expenses incurred by or on behalf of any Director in connection with any Proceeding in which such Director is involved by reason of such Director's Corporate Status within thirty (30) days after the receipt by the Corporation of a written statement from such Director requesting such advance or advances from time to time, whether prior to or after final disposition of such Proceeding. Such statement or statements shall reasonably evidence the Expenses incurred by such Director and shall be preceded or accompanied by an undertaking by or on behalf of such Director to repay any Expenses so advanced if it shall ultimately be determined that such Director is not entitled to be indemnified against such Expenses. Notwithstanding the foregoing, the Corporation shall advance all Expenses incurred by or on behalf of any Director seeking advancement of expenses hereunder in connection with a Proceeding initiated by such Director only if such Proceeding (including any parts of such Proceeding not initiated by such Director) was (A) authorized by the Board of Directors of the Corporation, or (B) brought to enforce such Director's rights to indemnification or advancement of Expenses under these By-laws.

(ii) If a claim for advancement of Expenses hereunder by a Director is not paid in full by the Corporation within thirty (30) days after receipt by the Corporation of documentation of Expenses and the required undertaking, such Director may at any time thereafter bring suit against the Corporation to recover the unpaid amount of the claim and if successful in whole or in part, such Director shall also be entitled to be paid the expenses of prosecuting such claim. The failure of the Corporation (including its Board of Directors or any committee thereof, independent legal counsel, or stockholders) to make a determination concerning the permissibility of such advancement of Expenses under this Section 5 shall not be a defense to an action brought by a Director for recovery of the unpaid amount of an advancement claim and shall not create a presumption that such advancement is not permissible. The burden of proving that a Director is not entitled to an advancement of expenses shall be on the Corporation.

(iii) In any suit brought by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the Corporation shall be entitled to recover such expenses upon a final adjudication that the Director has not met any applicable standard for indemnification set forth in the DGCL.

(f) Advancement of Expenses to Officers and Non-Officer Employees Prior to Final Disposition.

(i) The Corporation may, at the discretion of the Board of Directors of the Corporation, advance any or all Expenses incurred by or on behalf of any Officer or any Non-Officer Employee in connection with any Proceeding in which such person is

involved by reason of his or her Corporate Status as an Officer or Non-Officer Employee upon the receipt by the Corporation of a statement or statements from such Officer or Non-Officer Employee requesting such advance or advances from time to time, whether prior to or after final disposition of such Proceeding. Such statement or statements shall reasonably evidence the Expenses incurred by such Officer or Non-Officer Employee and shall be preceded or accompanied by an undertaking by or on behalf of such person to repay any Expenses so advanced if it shall ultimately be determined that such Officer or Non-Officer Employee is not entitled to be indemnified against such Expenses.

(ii) In any suit brought by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the Corporation shall be entitled to recover such expenses upon a final adjudication that the Officer or Non-Officer Employee has not met any applicable standard for indemnification set forth in the DGCL.

(g) Contractual Nature of Rights.

(i) The provisions of this Section 5 shall be deemed to be a contract between the Corporation and each Director and Officer entitled to the benefits hereof at any time while this Section 5 is in effect, in consideration of such person's past or current and any future performance of services for the Corporation. Neither amendment, repeal or modification of any provision of this Section 5 nor the adoption of any provision of the Certificate of Incorporation inconsistent with this Section 5 shall eliminate or reduce any right conferred by this Section 5 in respect of any act or omission occurring, or any cause of action or claim that accrues or arises or any state of facts existing, at the time of or before such amendment, repeal, modification or adoption of an inconsistent provision (even in the case of a proceeding based on such a state of facts that is commenced after such time), and all rights to indemnification and advancement of Expenses granted herein or arising out of any act or omission shall vest at the time of the act or omission in question, regardless of when or if any proceeding with respect to such act or omission is commenced. The rights to indemnification and to advancement of expenses provided by, or granted pursuant to, this Section 5 shall continue notwithstanding that the person has ceased to be a director or officer of the Corporation and shall inure to the benefit of the estate, heirs, executors, administrators, legatees and distributees of such person.

(ii) If a claim for indemnification hereunder by a Director or Officer is not paid in full by the Corporation within sixty (60) days after receipt by the Corporation of a written claim for indemnification, such Director or Officer may at any time thereafter bring suit against the Corporation to recover the unpaid amount of the claim, and if successful in whole or in part, such Director or Officer shall also be entitled to be paid the expenses of prosecuting such claim. The failure of the Corporation (including its Board of Directors or any committee thereof, independent legal counsel, or stockholders) to make a determination concerning the permissibility of such indemnification under this Section 5 shall not be a defense to an action brought by a Director or Officer for recovery of the unpaid amount of an indemnification claim and shall not create a presumption that such indemnification is not permissible. The burden of proving that a Director or Officer is not entitled to indemnification shall be on the Corporation.

(iii) In any suit brought by a Director or Officer to enforce a right to indemnification hereunder, it shall be a defense that such Director or Officer has not met any applicable standard for indemnification set forth in the DGCL.

(h) Non-Exclusivity of Rights. The rights to indemnification and advancement of Expenses set forth in this Section 5 shall not be exclusive of any other right which any Director, Officer, or Non-Officer Employee may have or hereafter acquire under any statute, provision of the Certificate or these By-laws, agreement, vote of stockholders or Disinterested Directors or otherwise.

(i) Insurance. The Corporation may maintain insurance, at its expense, to protect itself and any Director, Officer or Non-Officer Employee against any liability of any character asserted against or incurred by the Corporation or any such Director, Officer or Non-Officer Employee, or arising out of any such person's Corporate Status, whether or not the Corporation would have the power to indemnify such person against such liability under the DGCL or the provisions of this Section 5.

(j) Other Indemnification. The Corporation's obligation, if any, to indemnify or provide advancement of Expenses to any person under this Section 5 as a result of such person serving, at the request of the Corporation, as a director, partner, trustee, officer, employee or agent of another corporation, partnership, joint venture, trust, employee benefit plan or other enterprise shall be reduced by any amount such person may collect as indemnification or advancement of Expenses from such other corporation, partnership, joint venture, trust, employee benefit plan or enterprise (the "Primary Indemnitor"). Any indemnification or advancement of Expenses under this Section 5 owed by the Corporation as a result of a person serving, at the request of the Corporation, as a director, partner, trustee, officer, employee or agent of another corporation, partnership, joint venture, trust, employee benefit plan or other enterprise shall only be in excess of, and shall be secondary to, the indemnification or advancement of Expenses available from the applicable Primary Indemnitor(s) and any applicable insurance policies.

## 6. Miscellaneous Provisions

(a) Fiscal Year. Except as otherwise determined by the Board of Directors, the fiscal year of the Corporation shall end on December 31 of each year.

(b) Seal. The Board of Directors shall have power to adopt and alter the seal of the Corporation.

(c) Execution of Instruments. Subject to any limitations which may be set forth in a resolution of the Board of Directors, all deeds, leases, transfers, contracts, bonds, notes and other obligations to be entered into by the Corporation in the ordinary course of its business without director action may be executed on behalf of the Corporation by, a President, or by any other officer, employee or agent of the Corporation as the Board of Directors may authorize.

(d) Voting of Securities. Unless the Board of Directors otherwise provides, a President, any Vice President or the Treasurer may waive notice of and act on behalf of this Corporation, or appoint another person or persons to act as proxy or attorney in fact for this Corporation with or without discretionary power and/or power of substitution, at any meeting of stockholders or shareholders of any other corporation or organization, any of whose securities are held by this Corporation.

(e) Resident Agent. The Board of Directors may appoint a resident agent upon whom legal process may be served in any action or proceeding against the Corporation.

(f) Corporate Records. The original or attested copies of the Certificate of Incorporation, By-laws and records of all meetings of the incorporators, stockholders and the Board of Directors and the stock and transfer records, which shall contain the names of all stockholders, their record addresses and the amount of stock held by each, shall be kept at the principal office of the Corporation, at the office of its counsel, or at an office of its transfer agent.

(g) Certificate of Incorporation. All references in these By-laws to the Certificate of Incorporation shall be deemed to refer to the Certificate of Incorporation of the Corporation, as amended and in effect from time to time.

(h) Amendments. These By-laws may be altered, amended or repealed, and new By-laws may be adopted, by the stockholders or by the Board of Directors; provided, that (a) the Board of Directors may not alter, amend or repeal any provision of these By-laws which by law, by the Certificate of Incorporation or by these By-laws requires action by the stockholders and (b) any alteration, amendment or repeal of these By-laws by the Board of Directors and any new By-law adopted by the Board of Directors may be altered, amended or repealed by the stockholders.

(i) Waiver of Notice. Whenever notice is required to be given under any provision of these By-laws, a written waiver, signed by the person entitled to notice, or a waiver by electronic transmission by the person entitled to notice, whether before or after the time of the event for which notice is to be given, shall be deemed equivalent to notice. Attendance of a person at a meeting shall constitute a waiver of notice of such meeting, except when the person attends a meeting for the express purpose of objecting at the beginning of the meeting to the transaction of any business because the meeting is not lawfully called or convened. Neither the business to be transacted at, nor the purpose of, any meeting needs to be specified in any written waiver or any waiver by electronic transmission.

Adopted October 21, 2016

**GENERATION BIO CO.**

**SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

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Schedule A - Schedule of Investors

## SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

THIS SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT (this "**Agreement**"), is made as of the 9th day of January, 2020, by and among Generation Bio Co., a Delaware corporation (the "**Company**"), and each of the investors listed on Schedule A hereto, each of which is referred to in this Agreement as an "**Investor**".

### RECITALS

**WHEREAS**, certain of the Investors (the "**Existing Investors**") hold shares of the Company's Series A Preferred Stock (as defined below) and/or shares of the Company's Series B Preferred Stock (as defined below) and possess registration rights, information rights, rights of first offer, and other rights pursuant to an Amended and Restated Investors' Rights Agreement dated as of February 21, 2018, by and among the Company and such Investors (the "**Prior Agreement**"); and

**WHEREAS**, the undersigned Existing Investors are holders of a majority of the Registrable Securities of the Company (as defined in the Prior Agreement), and desire to amend and restate the Prior Agreement in its entirety and to accept the rights created pursuant to this Agreement in lieu of the rights granted to them under the Prior Agreement; and

**WHEREAS**, certain of the Investors are parties to that certain Series C Preferred Stock Purchase Agreement of even date herewith, by and among the Company and certain of the Investors (the "**Purchase Agreement**"), under which certain of the Company's and such Investors' obligations are conditioned upon the execution and delivery of this Agreement by such Investors, Existing Investors holding a majority of the Registrable Securities and the Company.

**NOW, THEREFORE**, the parties hereby agree as follows:

1. Definitions. For purposes of this Agreement:

1.1 "**Affiliate**" means, with respect to any specified Person, any other Person who, directly or indirectly, controls, is controlled by, or is under common control with such Person, including, without limitation, any general partner, managing member, limited partner, member, employee, officer or director of such Person or any venture capital fund or other investment fund now or hereafter existing that is controlled by one or more general partners or managing members of, or shares the same management company or investment adviser with, such Person. For purposes of this definition, the term "control" when used with respect to any Person means the power to direct the management or policies of such Person, directly or indirectly, whether through ownership of voting securities, by contract or otherwise, and the terms "controlling" and "controlled" shall have meanings correlative to the foregoing.

1.2 "**Common Stock**" means shares of the Company's common stock, par value \$0.0001 per share.

1.3 "**Damages**" means any loss, damage, claim or liability (joint or several) to which a party hereto may become subject under the Securities Act, the Exchange Act, or other

federal or state law, insofar as such loss, damage, claim or liability (or any action in respect thereof) arises out of or is based upon: (i) any untrue statement or alleged untrue statement of a material fact contained in any registration statement of the Company, including any preliminary prospectus or final prospectus contained therein or any amendments or supplements thereto; (ii) an omission or alleged omission to state therein a material fact required to be stated therein, or necessary to make the statements therein not misleading; or (iii) any violation or alleged violation by the indemnifying party (or any of its agents or Affiliates) of the Securities Act, the Exchange Act, any state securities law, or any rule or regulation promulgated under the Securities Act, the Exchange Act, or any state securities law.

1.4 “**Derivative Securities**” means any securities or rights convertible into, or exercisable or exchangeable for (in each case, directly or indirectly), Common Stock, including options and warrants.

1.5 “**Exchange Act**” means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

1.6 “**Excluded Registration**” means (i) a registration relating to the sale of securities to employees of the Company or a subsidiary pursuant to a stock option, stock purchase, or similar plan; (ii) a registration relating to an SEC Rule 145 transaction; (iii) a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities; or (iv) a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered.

1.7 “**Fidelity**” means Fidelity Management & Research Company and its Affiliates that hold shares of Registrable Securities.

1.8 “**Form S-1**” means such form under the Securities Act as in effect on the date hereof or any successor registration form under the Securities Act subsequently adopted by the SEC.

1.9 “**Form S-3**” means such form under the Securities Act as in effect on the date hereof or any registration form under the Securities Act subsequently adopted by the SEC that permits incorporation of substantial information by reference to other documents filed by the Company with the SEC.

1.10 “**GAAP**” means generally accepted accounting principles in the United States.

1.11 “**Holder**” means any holder of Registrable Securities who is a party to this Agreement.

1.12 “**Immediate Family Member**” means a child, stepchild, grandchild, parent, stepparent, grandparent, spouse, sibling, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law, including, adoptive relationships, of a natural person referred to herein.

1.13 “**Initiating Holders**” means, collectively, Holders who properly initiate a registration request under this Agreement.

1.14 “**IPO**” means the Company’s first underwritten public offering of its Common Stock under the Securities Act.

1.15 “**Key Employee**” means any executive-level employee (including, division director and vice president-level positions) as well as any employee who, either alone or in concert with others, develops, invents, programs, or designs any Company Intellectual Property (as defined in the Purchase Agreement).

1.16 “**Major Investor**” means any Investor that, individually or together with such Investor’s Affiliates, holds at least 875,000 shares of Registrable Securities (as adjusted for any stock split, stock dividend, combination, or other recapitalization or reclassification effected after the date hereof).

1.17 “**New Securities**” means, collectively, equity securities of the Company, whether or not currently authorized, as well as rights, options, or warrants to purchase such equity securities, or securities of any type whatsoever that are, or may become, convertible or exchangeable into or exercisable for such equity securities.

1.18 “**Person**” means any individual, corporation, partnership, trust, limited liability company, association or other entity.

1.19 “**Preferred Stock**” means, collectively, shares of the Company’s Series A Preferred Stock, Series B Preferred Stock and Series C Preferred Stock.

1.20 “**Preferred Stock Directors**” shall have the meaning given to such term in that certain Second Amended and Restated Voting Agreement of even date herewith by and among the Company, the Investors and the other parties named therein, as may be amended and/or restated from time to time.

1.21 “**Registrable Securities**” means (i) the Common Stock issuable or issued upon conversion of the Preferred Stock, (ii) any Common Stock, or any Common Stock issued or issuable (directly or indirectly) upon conversion and/or exercise of any other securities of the Company, held by the Investors; and (iii) any Common Stock issued as (or issuable upon the conversion or exercise of any warrant, right, or other security that is issued as) a dividend or other distribution with respect to, or in exchange for or in replacement of, the shares referenced in clauses (i) and (ii) above; excluding in all cases, however, any Registrable Securities sold by a Person in a transaction in which the applicable rights under this Agreement are not assigned pursuant to Subsection 6.1, and excluding for purposes of Section 2 any shares for which registration rights have terminated pursuant to Subsection 2.13 of this Agreement.

1.22 “**Registrable Securities then outstanding**” means the number of shares determined by adding the number of shares of outstanding Common Stock that are Registrable Securities and the number of shares of Common Stock issuable (directly or indirectly) pursuant to then exercisable and/or convertible securities that are Registrable Securities.

hereof. 1.23 “**Restricted Securities**” means the securities of the Company required to be notated with the legend set forth in Subsection 2.12(b)

1.24 “**SEC**” means the Securities and Exchange Commission.

1.25 “**SEC Rule 144**” means Rule 144 promulgated by the SEC under the Securities Act.

1.26 “**SEC Rule 145**” means Rule 145 promulgated by the SEC under the Securities Act.

1.27 “**Securities Act**” means the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

1.28 “**Selling Expenses**” means all underwriting discounts, selling commissions, and stock transfer taxes applicable to the sale of Registrable Securities, and fees and disbursements of counsel for any Holder, except for the fees and disbursements of the Selling Holder Counsel borne and paid by the Company as provided in Subsection 2.6.

1.29 “**Series A Preferred Stock**” means shares of the Company’s Series A Preferred Stock, par value \$0.0001 per share.

1.30 “**Series B Preferred Stock**” means shares of the Company’s Series B Preferred Stock, par value \$0.0001 per share.

1.31 “**Series C Preferred Stock**” means shares of the Company’s Series C Preferred Stock, par value \$0.0001 per share.

1.32 “**T. Rowe Price**” means T. Rowe Price Associates, Inc. and any successor or affiliated registered investment advisor to the T. Rowe Price Investors.

1.33 “**T. Rowe Price Investors**” means the Investors that are advisory or sub-advisory clients of T. Rowe Price with respect to holding shares of the Company.

1.34 “**Wellington**” means Wellington Biomedical Innovation Master Investors (Cayman) I L.P. and its permitted successors and assigns

1.35 “**Wellington Investors**” means Investors, or permitted transferees of Registrable Securities held by Wellington Investors, that are advisory or subadvisory clients of Wellington Management Company LLP.

2. Registration Rights. The Company covenants and agrees as follows:

2.1 Demand Registration.

(a) Form S-1 Demand. If at any time after the earlier of (i) five (5) years after the date of this Agreement or (ii) one hundred eighty (180) days after the effective date of the registration statement for the IPO, the Company receives a request from holders of a majority of

the Registrable Securities then outstanding (the “**Requisite Holders**”) that the Company file a Form S-1 registration statement with respect to at least forty percent (40%) of the Registrable Securities then outstanding (or a lesser percent if the anticipated aggregate offering price, net of Selling Expenses, would exceed \$10 million), then the Company shall (x) within ten (10) days after the date such request is given, give notice thereof (the “**Demand Notice**”) to all Holders other than the Initiating Holders; and (y) as soon as practicable, and in any event within sixty (60) days after the date such request is given by the Initiating Holders, file a Form S-1 registration statement under the Securities Act covering all Registrable Securities that the Initiating Holders requested to be registered and any additional Registrable Securities requested to be included in such registration by any other Holders, as specified by notice given by each such Holder to the Company within twenty (20) days of the date the Demand Notice is given, and in each case, subject to the limitations of Subsections 2.1(c) and 2.3.

(b) Form S-3 Demand. If at any time when it is eligible to use a Form S-3 registration statement, the Company receives a request from Holders of at least twenty percent (20%) of the Registrable Securities then outstanding that the Company file a Form S-3 registration statement with respect to outstanding Registrable Securities of such Holders having an anticipated aggregate offering price, net of Selling Expenses, of at least \$5 million, then the Company shall (i) within ten (10) days after the date such request is given, give a Demand Notice to all Holders other than the Initiating Holders; and (ii) as soon as practicable, and in any event within forty-five (45) days after the date such request is given by the Initiating Holders, file a Form S-3 registration statement under the Securities Act covering all Registrable Securities requested to be included in such registration by any other Holders, as specified by notice given by each such Holder to the Company within twenty (20) days of the date the Demand Notice is given, and in each case, subject to the limitations of Subsections 2.1(c) and 2.3.

(c) Notwithstanding the foregoing obligations, if the Company furnishes to Holders requesting a registration pursuant to this Subsection 2.1 a certificate signed by the Company’s chief executive officer stating that in the good faith judgment of the Company’s Board of Directors it would be materially detrimental to the Company and its stockholders for such registration statement to either become effective or remain effective for as long as such registration statement otherwise would be required to remain effective, because such action would (i) materially interfere with a significant acquisition, corporate reorganization, or other similar transaction involving the Company; (ii) require premature disclosure of material information that the Company has a bona fide business purpose for preserving as confidential; or (iii) render the Company unable to comply with requirements under the Securities Act or Exchange Act, then the Company shall have the right to defer taking action with respect to such filing, and any time periods with respect to filing or effectiveness thereof shall be tolled correspondingly, for a period of not more than ninety (90) days after the request of the Initiating Holders is given; provided, however, that the Company may not invoke this right more than once in any twelve (12) month period; and provided further that the Company shall not register any securities for its own account or that of any other stockholder during such ninety (90) day period other than (i) pursuant to a registration relating to the sale of securities to employees of the Company or a subsidiary pursuant to a stock option, stock purchase, or similar plan, (ii) a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Registrable Securities or (iii) a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered.

(d) The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Subsection 2.1(a)(i) during the period that is sixty (60) days before the Company's good faith estimate of the date of filing of, and ending on a date that is one hundred eighty (180) days after the effective date of, a Company-initiated registration, provided that the Company is actively employing in good faith commercially reasonable efforts to cause such registration statement to become effective; (ii) after the Company has effected a registration pursuant to Subsection 2.1(a); or (iii) if the Initiating Holders propose to dispose of shares of Registrable Securities that may be immediately registered on Form S-3 pursuant to a request made pursuant to Subsection 2.1(b). The Company shall not be obligated to effect, or to take any action to effect, any registration pursuant to Subsection 2.1(b) (i) during the period that is thirty (30) days before the Company's good faith estimate of the date of filing of, and ending on a date that is ninety (90) days after the effective date of, a Company-initiated registration, provided that the Company is actively employing in good faith commercially reasonable efforts to cause such registration statement to become effective; or (ii) if the Company has effected a registration pursuant to Subsection 2.1(b) within the twelve (12) month period immediately preceding the date of such request. A registration shall not be counted as "effected" for purposes of this Subsection 2.1(d) until such time as the applicable registration statement has been declared effective by the SEC, unless the Initiating Holders withdraw their request for such registration, elect not to pay the registration expenses therefor, and forfeit their right to one demand registration statement pursuant to Subsection 2.6, in which case such withdrawn registration statement shall be counted as "effected" for purposes of this Subsection 2.1(d).

2.2 Company Registration. If the Company proposes to register (including, for this purpose, a registration effected by the Company for stockholders other than the Holders) any of its securities under the Securities Act in connection with the public offering of such securities solely for cash (other than in an Excluded Registration), the Company shall, at such time, promptly give each Holder notice of such registration. Upon the request of each Holder given within twenty (20) days after such notice is given by the Company, the Company shall, subject to the provisions of Subsection 2.3, cause to be registered all of the Registrable Securities that each such Holder has requested to be included in such registration. The Company shall have the right to terminate or withdraw any registration initiated by it under this Subsection 2.2 before the effective date of such registration, whether or not any Holder has elected to include Registrable Securities in such registration. The expenses (other than Selling Expenses) of such withdrawn registration shall be borne by the Company in accordance with Subsection 2.6.

### 2.3 Underwriting Requirements.

(a) If, pursuant to Subsection 2.1, the Initiating Holders intend to distribute the Registrable Securities covered by their request by means of an underwriting, they shall so advise the Company as a part of their request made pursuant to Subsection 2.1, and the Company shall include such information in the Demand Notice. The underwriter(s) will be selected by the Company and shall be reasonably acceptable to a majority in interest of the Initiating Holders. In such event, the right of any Holder to include such Holder's Registrable Securities in such registration shall be conditioned upon such Holder's participation in such

underwriting and the inclusion of such Holder's Registrable Securities in the underwriting to the extent provided herein. All Holders proposing to distribute their securities through such underwriting shall (together with the Company as provided in Subsection 2.4(e)) enter into an underwriting agreement in customary form with the underwriter(s) selected for such underwriting. Notwithstanding any other provision of this Subsection 2.3, if the managing underwriter(s) advise(s) the Initiating Holders in writing that marketing factors require a limitation on the number of shares to be underwritten, then the Initiating Holders shall so advise all Holders of Registrable Securities that otherwise would be underwritten pursuant hereto, and the number of Registrable Securities that may be included in the underwriting shall be allocated among such Holders of Registrable Securities, including the Initiating Holders, in proportion (as nearly as practicable) to the number of Registrable Securities owned by each Holder or in such other proportion as shall mutually be agreed to by all such selling Holders; provided, however, that the number of Registrable Securities held by the Holders to be included in such underwriting shall not be reduced unless all other securities are first entirely excluded from the underwriting. To facilitate the allocation of shares in accordance with the above provisions, the Company or the underwriters may round the number of shares allocated to any Holder to the nearest one hundred (100) shares.

(b) In connection with any offering involving an underwriting of shares of the Company's capital stock pursuant to Subsection 2.2, the Company shall not be required to include any of the Holders' Registrable Securities in such underwriting unless the Holders accept the terms of the underwriting as agreed upon between the Company and its underwriters, and then only in such quantity as the underwriters in their sole discretion determine will not jeopardize the success of the offering by the Company. If the total number of securities, including Registrable Securities, requested by stockholders to be included in such offering exceeds the number of securities to be sold (other than by the Company) that the underwriters in their reasonable discretion determine is compatible with the success of the offering, then the Company shall be required to include in the offering only that number of such securities, including Registrable Securities, which the underwriters and the Company in their sole discretion determine will not jeopardize the success of the offering. If the underwriters determine that less than all of the Registrable Securities requested to be registered can be included in such offering, then the Registrable Securities that are included in such offering shall be allocated among the selling Holders in proportion (as nearly as practicable to) the number of Registrable Securities owned by each selling Holder or in such other proportions as shall mutually be agreed to by all such selling Holders. To facilitate the allocation of shares in accordance with the above provisions, the Company or the underwriters may round the number of shares allocated to any Holder to the nearest one hundred (100) shares. Notwithstanding the foregoing, in no event shall (i) the number of Registrable Securities included in the offering be reduced unless all other securities (other than securities to be sold by the Company) are first entirely excluded from the offering, or (ii) the number of Registrable Securities included in the offering be reduced below twenty percent (20%) of the total number of securities included in such offering, unless such offering is the IPO, in which case the selling Holders may be excluded further if the underwriters make the determination described above and no other stockholder's securities are included in such offering. For purposes of the provision in this Subsection 2.3(b) concerning apportionment, for any selling Holder that is a partnership, limited liability company, or corporation, the partners, members, retired partners, retired members, stockholders, and Affiliates of such Holder, or the estates and Immediate Family Members of any such partners, retired partners, members, and retired members and any trusts for the benefit of any of the foregoing Persons, shall be deemed to be a single "selling Holder," and any pro rata reduction with respect to such "selling Holder" shall be based upon the aggregate number of Registrable Securities owned by all Persons included in such "selling Holder," as defined in this sentence.



(c) For purposes of Subsection 2.1, a registration shall not be counted as “effected” if, as a result of an exercise of the underwriter’s cutback provisions in Subsection 2.3(a), fewer than fifty percent (50%) of the total number of Registrable Securities that Holders have requested to be included in such registration statement are actually included.

2.4 Obligations of the Company. Whenever required under this Section 2 to effect the registration of any Registrable Securities, the Company shall, as expeditiously as reasonably possible:

(a) prepare and file with the SEC a registration statement with respect to such Registrable Securities and use its commercially reasonable efforts to cause such registration statement to become effective and, upon the request of the Holders of a majority of the Registrable Securities registered thereunder, keep such registration statement effective for a period of up to one hundred twenty (120) days or, if earlier, until the distribution contemplated in the registration statement has been completed; provided, however, that (i) such one hundred twenty (120) day period shall be extended for a period of time equal to the period the Holder refrains, at the request of an underwriter of Common Stock (or other securities) of the Company, from selling any securities included in such registration, and (ii) in the case of any registration of Registrable Securities on Form S-3 that are intended to be offered on a continuous or delayed basis, subject to compliance with applicable SEC rules, such one hundred twenty (120) day period shall be extended for up to sixty (60) days, if necessary, to keep the registration statement effective until all such Registrable Securities are sold;

(b) prepare and file with the SEC such amendments and supplements to such registration statement, and the prospectus used in connection with such registration statement, as may be necessary to comply with the Securities Act in order to enable the disposition of all securities covered by such registration statement;

(c) furnish to the selling Holders such numbers of copies of a prospectus, including a preliminary prospectus, as required by the Securities Act, and such other documents as the Holders may reasonably request in order to facilitate their disposition of their Registrable Securities;

(d) use its commercially reasonable efforts to register and qualify the securities covered by such registration statement under such other securities or blue-sky laws of such jurisdictions as shall be reasonably requested by the selling Holders; provided that the Company shall not be required to qualify to do business or to file a general consent to service of process in any such states or jurisdictions, unless the Company is already subject to service in such jurisdiction and except as may be required by the Securities Act;

(e) in the event of any underwritten public offering, enter into and perform its obligations under an underwriting agreement, in usual and customary form, with the underwriter(s) of such offering;

(f) use its commercially reasonable efforts to cause all such Registrable Securities covered by such registration statement to be listed on a national securities exchange or trading system and each securities exchange and trading system (if any) on which similar securities issued by the Company are then listed;

(g) provide a transfer agent and registrar for all Registrable Securities registered pursuant to this Agreement and provide a CUSIP number for all such Registrable Securities, in each case not later than the effective date of such registration;

(h) promptly make available for inspection by the selling Holders, any managing underwriter(s) participating in any disposition pursuant to such registration statement, and any attorney or accountant or other agent retained by any such underwriter or selected by the selling Holders, all financial and other records, pertinent corporate documents, and properties of the Company, and cause the Company's officers, directors, employees, and independent accountants to supply all information reasonably requested by any such seller, underwriter, attorney, accountant, or agent, in each case, as necessary or advisable to verify the accuracy of the information in such registration statement and to conduct appropriate due diligence in connection therewith;

(i) notify each selling Holder, promptly after the Company receives notice thereof, of the time when such registration statement has been declared effective or a supplement to any prospectus forming a part of such registration statement has been filed; and

(j) after such registration statement becomes effective, notify each selling Holder of any request by the SEC that the Company amend or supplement such registration statement or prospectus.

In addition, the Company shall ensure that, at all times after any registration statement covering a public offering of securities of the Company under the Securities Act shall have become effective, its insider trading policy shall provide that the Company's directors may implement a trading program under Rule 10b5-1 of the Exchange Act.

2.5 Furnish Information. It shall be a condition precedent to the obligations of the Company to take any action pursuant to this Section 2 with respect to the Registrable Securities of any selling Holder that such Holder shall furnish to the Company such information regarding itself, the Registrable Securities held by it, and the intended method of disposition of such securities as is reasonably required to effect the registration of such Holder's Registrable Securities.

2.6 Expenses of Registration. All expenses (other than Selling Expenses) incurred in connection with registrations, filings, or qualifications pursuant to Section 2, including all registration, filing, and qualification fees; printers' and accounting fees; fees and disbursements of counsel for the Company; and the reasonable fees and disbursements, not to exceed \$25,000, of one counsel for the selling Holders ("**Selling Holder Counsel**"), shall be borne and paid by the Company; provided, however, that the Company shall not be required to pay for any expenses of any registration proceeding begun pursuant to Subsection 2.1 if the registration request is subsequently withdrawn at the request of the Holders of a majority of the Registrable Securities to

be registered (in which case all selling Holders shall bear such expenses pro rata based upon the number of Registrable Securities that were to be included in the withdrawn registration), unless the Holders of a majority of the Registrable Securities agree to forfeit their right to one registration pursuant to Subsections 2.1(a) or 2.1(b), as the case may be; provided further that if, at the time of such withdrawal, the Holders shall have learned of a material adverse change in the condition, business, or prospects of the Company from that known to the Holders at the time of their request and have withdrawn the request with reasonable promptness after learning of such information, then the Holders shall not be required to pay any of such expenses and shall not forfeit their right to one registration pursuant to Subsections 2.1(a) or 2.1(b). All Selling Expenses relating to Registrable Securities registered pursuant to this Section 2 shall be borne and paid by the Holders pro rata on the basis of the number of Registrable Securities registered on their behalf.

2.7 Delay of Registration. No Holder shall have any right to obtain or seek an injunction restraining or otherwise delaying any registration pursuant to this Agreement as the result of any controversy that might arise with respect to the interpretation or implementation of this Section 2.

2.8 Indemnification. If any Registrable Securities are included in a registration statement under this Section 2:

(a) To the extent permitted by law, the Company will indemnify and hold harmless each selling Holder, and the partners, members, officers, directors, and stockholders of each such Holder; legal counsel, accountants and investment advisers for each such Holder; any underwriter (as defined in the Securities Act) for each such Holder; and each Person, if any, who controls such Holder or underwriter within the meaning of the Securities Act or the Exchange Act, against any Damages, and the Company will pay to each such Holder, underwriter, controlling Person, or other aforementioned Person any legal or other expenses reasonably incurred thereby in connection with investigating or defending any claim or proceeding from which Damages may result, as such expenses are incurred; provided, however, that the indemnity agreement contained in this Subsection 2.8(a) shall not apply to amounts paid in settlement of any such claim or proceeding if such settlement is effected without the consent of the Company, which consent shall not be unreasonably withheld, nor shall the Company be liable for any Damages to the extent that they arise out of or are based upon actions or omissions made in reliance upon and in conformity with written information furnished by or on behalf of any such Holder, underwriter, controlling Person, or other aforementioned Person expressly for use in connection with such registration.

(b) To the extent permitted by law, each selling Holder, severally and not jointly, will indemnify and hold harmless the Company, and each of its directors, each of its officers who has signed the registration statement, each Person (if any), who controls the Company within the meaning of the Securities Act, legal counsel and accountants for the Company, any underwriter (as defined in the Securities Act), any other Holder selling securities in such registration statement, and any controlling Person of any such underwriter or other Holder, against any Damages, in each case only to the extent that such Damages arise out of or are based upon actions or omissions made in reliance upon and in conformity with written information furnished by or on behalf of such selling Holder expressly for use in connection with such registration; and each such selling Holder will pay to the Company and each other aforementioned Person any legal or other expenses reasonably incurred thereby in connection with investigating or defending any

claim or proceeding from which Damages may result, as such expenses are incurred; provided, however, that the indemnity agreement contained in this Subsection 2.8(b) shall not apply to amounts paid in settlement of any such claim or proceeding if such settlement is effected without the consent of the Holder, which consent shall not be unreasonably withheld; and provided further that in no event shall the aggregate amounts payable by any Holder by way of indemnity or contribution under Subsections 2.8(b) and 2.8(d) exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of fraud or willful misconduct by such Holder.

(c) Promptly after receipt by an indemnified party under this Subsection 2.8 of notice of the commencement of any action (including any governmental action) for which a party may be entitled to indemnification hereunder, such indemnified party will, if a claim in respect thereof is to be made against any indemnifying party under this Subsection 2.8, give the indemnifying party notice of the commencement thereof. The indemnifying party shall have the right to participate in such action and, to the extent the indemnifying party so desires, participate jointly with any other indemnifying party to which notice has been given, and to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party (together with all other indemnified parties that may be represented without conflict by one counsel) shall have the right to retain one separate counsel, with the fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such action. The failure to give notice to the indemnifying party within a reasonable time of the commencement of any such action shall relieve such indemnifying party of any liability to the indemnified party under this Subsection 2.8, to the extent that such failure materially prejudices the indemnifying party's ability to defend such action. The failure to give notice to the indemnifying party will not relieve it of any liability that it may have to any indemnified party otherwise than under this Subsection 2.8.

(d) To provide for just and equitable contribution to joint liability under the Securities Act in any case in which either: (i) any party otherwise entitled to indemnification hereunder makes a claim for indemnification pursuant to this Subsection 2.8 but it is judicially determined (by the entry of a final judgment or decree by a court of competent jurisdiction and the expiration of time to appeal or the denial of the last right of appeal) that such indemnification may not be enforced in such case, notwithstanding the fact that this Subsection 2.8 provides for indemnification in such case, or (ii) contribution under the Securities Act may be required on the part of any party hereto for which indemnification is provided under this Subsection 2.8, then, and in each such case, such parties will contribute to the aggregate losses, claims, damages, liabilities, or expenses to which they may be subject (after contribution from others) in such proportion as is appropriate to reflect the relative fault of each of the indemnifying party and the indemnified party in connection with the statements, omissions, or other actions that resulted in such loss, claim, damage, liability, or expense, as well as to reflect any other relevant equitable considerations. The relative fault of the indemnifying party and of the indemnified party shall be determined by reference to, among other things, whether the untrue or allegedly untrue statement of a material fact, or the omission or alleged omission of a material fact, relates to information supplied by the indemnifying party or by the indemnified party and the parties' relative intent, knowledge, access to information, and opportunity to correct or prevent such statement or omission; provided.

however, that, in any such case (x) no Holder will be required to contribute any amount in excess of the public offering price of all such Registrable Securities offered and sold by such Holder pursuant to such registration statement, and (y) no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) will be entitled to contribution from any Person who was not guilty of such fraudulent misrepresentation; and provided further that in no event shall a Holder's liability pursuant to this Subsection 2.8(d), when combined with the amounts paid or payable by such Holder pursuant to Subsection 2.8(b), exceed the proceeds from the offering received by such Holder (net of any Selling Expenses paid by such Holder), except in the case of willful misconduct or fraud by such Holder.

(e) Notwithstanding the foregoing, to the extent that the provisions on indemnification and contribution contained in the underwriting agreement entered into in connection with the underwritten public offering are in conflict with the foregoing provisions, the provisions in the underwriting agreement shall control.

(f) Unless otherwise superseded by an underwriting agreement entered into in connection with the underwritten public offering, the obligations of the Company and Holders under this Subsection 2.8 shall survive the completion of any offering of Registrable Securities in a registration under this Section 2, and otherwise shall survive the termination of this Agreement.

2.9 Reports Under Exchange Act. With a view to making available to the Holders the benefits of SEC Rule 144 and any other rule or regulation of the SEC that may at any time permit a Holder to sell securities of the Company to the public without registration or pursuant to a registration on Form S-3, the Company shall:

(a) make and keep available adequate current public information, as those terms are understood and defined in SEC Rule 144, at all times after the effective date of the registration statement filed by the Company for the IPO;

(b) use commercially reasonable efforts to file with the SEC in a timely manner all reports and other documents required of the Company under the Securities Act and the Exchange Act (at any time after the Company has become subject to such reporting requirements); and

(c) furnish to any Holder, so long as the Holder owns any Registrable Securities, forthwith upon request (i) to the extent accurate, a written statement by the Company that it has complied with the reporting requirements of SEC Rule 144 (at any time after ninety (90) days after the effective date of the registration statement filed by the Company for the IPO), the Securities Act, and the Exchange Act (at any time after the Company has become subject to such reporting requirements), or that it qualifies as a registrant whose securities may be resold pursuant to Form S-3 (at any time after the Company so qualifies) and (ii) such other information as may be reasonably requested in availing any Holder of any rule or regulation of the SEC that permits the selling of any such securities without registration (at any time after the Company has become subject to the reporting requirements under the Exchange Act) or pursuant to Form S-3 (at any time after the Company so qualifies to use such form).

2.10 Limitations on Subsequent Registration Rights. From and after the date of this Agreement, the Company shall not, without the prior written consent of the Requisite Holders enter into any agreement with any holder or prospective holder of any securities of the Company that would allow such holder or prospective holder (i) to include such securities in any registration unless, under the terms of such agreement, such holder or prospective holder may include such securities in any such registration only to the extent that the inclusion of such securities will not reduce the number of the Registrable Securities of the Holders that are included or (ii) allow such holder or prospective holder to initiate a demand for registration of any securities held by such holder or prospective holder; provided that this limitation shall not apply to any additional Investor who becomes a party to this Agreement in accordance with Subsection 6.9.

2.11 "Market Stand-off" Agreement. Each Holder hereby agrees that it will not, without the prior written consent of the managing underwriter, during the period commencing on the date of the final prospectus relating to the IPO and ending on the date specified by the Company and the managing underwriter (such period not to exceed one hundred eighty (180) days in the case of the IPO, or such other period as may be requested by the Company or an underwriter to accommodate regulatory restrictions on (1) the publication or other distribution of research reports, and (2) analyst recommendations and opinions, including, but not limited to, the restrictions contained in FINRA Rule 2711(f)(4) or NYSE Rule 472(f)(4), or any successor provisions or amendments thereto), (i) lend; offer; pledge; sell; contract to sell; sell any option or contract to purchase; purchase any option or contract to sell; grant any option, right, or warrant to purchase; or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any securities convertible into or exercisable or exchangeable (directly or indirectly) for Common Stock held immediately before the effective date of the registration statement for the IPO or (ii) enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of such securities, whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Common Stock or other securities, in cash, or otherwise. The foregoing provisions of this Subsection 2.11 shall apply only to the IPO, shall not apply to the sale of any shares to an underwriter pursuant to an underwriting agreement, or the transfer of any shares to any trust for the direct or indirect benefit of the Holder or the immediate family of the Holder, provided that the trustee of the trust agrees to be bound in writing by the restrictions set forth herein, and provided further that any such transfer shall not involve a disposition for value, and shall be applicable to the Holders only if all officers, directors and stockholders individually owning more than one percent (1%) of the Company's outstanding Common Stock (after giving effect to conversion into Common Stock of all outstanding Preferred Stock) are subject to the same restrictions. The underwriters in connection with such registration are intended third-party beneficiaries of this Subsection 2.11 and shall have the right, power and authority to enforce the provisions hereof as though they were a party hereto. Each Holder further agrees to execute such agreements as may be reasonably requested by the underwriters in connection with such registration that are consistent with this Subsection 2.11 or that are necessary to give further effect thereto. In the event that the Company or the managing underwriter waives or terminates any of the restrictions contained in this Subsection 2.11 or in a lock-up agreement with any Holder, officer, director or greater than one-percent stockholder of the Company (in any such case, the "**Released Securities**"), the restrictions contained in this Subsection 2.11 and in any lock-up agreements executed by the Major Investors shall be waived or terminated, as applicable, to the same extent and with respect to the same percentage of securities of each Major Investor as the percentage of Released Securities represent with respect to the securities held by the applicable Holder, officer, director or greater than one-percent stockholder.

2.12 Restrictions on Transfer.

(a) The Preferred Stock and the Registrable Securities shall not be sold, pledged, or otherwise transferred, and the Company shall not recognize and shall issue stop-transfer instructions to its transfer agent with respect to any such sale, pledge, or transfer, except upon the conditions specified in this Agreement, which conditions are intended to ensure compliance with the provisions of the Securities Act. A transferring Holder will cause any proposed purchaser, pledgee, or transferee of the Preferred Stock and the Registrable Securities held by such Holder to agree to take and hold such securities subject to the provisions and upon the conditions specified in this Agreement. Notwithstanding the foregoing, the Company shall not require any transferee of shares pursuant to an effective registration statement or, following the IPO, SEC Rule 144 to be bound by the terms of this Agreement.

(b) Each certificate, instrument, or book entry representing (i) the Preferred Stock, (ii) the Registrable Securities, and (iii) any other securities issued in respect of the securities referenced in clauses (i) and (ii), upon any stock split, stock dividend, recapitalization, merger, consolidation, or similar event, shall (unless otherwise permitted by the provisions of Subsection 2.12(c)) be notated with a legend substantially in the following form:

THE SECURITIES REPRESENTED HEREBY HAVE BEEN ACQUIRED FOR INVESTMENT AND HAVE NOT BEEN REGISTERED UNDER THE SECURITIES ACT OF 1933. SUCH SHARES MAY NOT BE SOLD, PLEDGED, OR TRANSFERRED IN THE ABSENCE OF SUCH REGISTRATION OR A VALID EXEMPTION FROM THE REGISTRATION AND PROSPECTUS DELIVERY REQUIREMENTS OF SAID ACT.

THE SECURITIES REPRESENTED HEREBY MAY BE TRANSFERRED ONLY IN ACCORDANCE WITH THE TERMS OF AN AGREEMENT BETWEEN THE COMPANY AND THE STOCKHOLDER, A COPY OF WHICH IS ON FILE WITH THE SECRETARY OF THE COMPANY.

The Holders consent to the Company making a notation in its records and giving instructions to any transfer agent of the Restricted Securities in order to implement the restrictions on transfer set forth in this Subsection 2.12.

(c) The holder of such Restricted Securities, by acceptance of ownership thereof, agrees to comply in all respects with the provisions of this Section 2. Before any proposed sale, pledge, or transfer of any Restricted Securities, unless there is in effect a registration statement under the Securities Act covering the proposed transaction or, following the IPO, the transfer is made pursuant to SEC Rule 144, the Holder thereof shall give notice to the Company of such Holder's intention to effect such sale, pledge, or transfer. Each such notice shall describe the manner and circumstances of the proposed sale, pledge, or transfer in sufficient detail and, if reasonably requested by the Company, shall be accompanied at such Holder's expense by either (i) a written opinion of legal counsel who shall, and whose legal opinion shall, be reasonably

satisfactory to the Company, addressed to the Company, to the effect that the proposed transaction may be effected without registration under the Securities Act; (ii) a “no action” letter from the SEC to the effect that the proposed sale, pledge, or transfer of such Restricted Securities without registration will not result in a recommendation by the staff of the SEC that action be taken with respect thereto; or (iii) any other evidence reasonably satisfactory to counsel to the Company to the effect that the proposed sale, pledge, or transfer of the Restricted Securities may be effected without registration under the Securities Act, whereupon the Holder of such Restricted Securities shall be entitled to sell, pledge, or transfer such Restricted Securities in accordance with the terms of the notice given by the Holder to the Company. The Company will not require such a legal opinion or “no action” letter (x) in any transaction in compliance with SEC Rule 144; or (y) in any transaction in which such Holder distributes Restricted Securities to an Affiliate of such Holder for no consideration; provided that, other than in connection with a transaction in compliance with SEC Rule 144 following the IPO, each transferee agrees in writing to be subject to the terms of this Subsection 2.12. Each certificate, instrument, or book entry representing the Restricted Securities transferred as above provided shall be notated with, except if such transfer is made pursuant to SEC Rule 144 or pursuant to an effective registration statement, the appropriate restrictive legend set forth in Subsection 2.12(b), except that such certificate instrument, or book entry shall not be notated with such restrictive legend if, in the opinion of counsel for such Holder and the Company, such legend is not required in order to establish compliance with any provisions of the Securities Act.

2.13 Termination of Registration Rights. The right of any Holder to request registration or inclusion of Registrable Securities in any registration pursuant to Subsections 2.1 or 2.2 shall terminate upon the earliest to occur of:

- (a) the closing of a Deemed Liquidation Event, as such term is defined in the Company’s Certificate of Incorporation;
- (b) such time following the IPO as Rule 144 or another similar exemption under the Securities Act is available for the sale of all of such Holder’s shares without limitation during a three-month period without registration; and
- (c) the fourth (4th) anniversary of the IPO.

### 3. Information Rights.

3.1 Delivery of Financial Statements. The Company shall deliver to each Major Investor:

- (a) as soon as practicable, but in any event within one hundred fifty (150) days after the end of each fiscal year of the Company, commencing with the fiscal year ending December 31, 2019 (i) a balance sheet as of the end of such year, (ii) statements of income and of cash flows for such year and (iii) a statement of stockholders’ equity as of the end of such year, all such financial statements audited and certified by independent public accountants of nationally recognized standing selected by the Company;
- (b) as soon as practicable, but in any event within sixty (60) days after the end of each of the first three (3) quarters of each fiscal year of the Company, unaudited



statements of income and cash flows for such fiscal quarter, and an unaudited balance sheet as of the end of such fiscal quarter, all prepared in accordance with GAAP (except that such financial statements may (i) be subject to normal year-end audit adjustments; and (ii) not contain all notes thereto that may be required in accordance with GAAP);

(c) as soon as practicable, but in any event within forty-five (45) days after the end of each quarter of each fiscal year of the Company, a statement showing the number of shares of each class and series of capital stock and securities convertible into or exercisable for shares of capital stock outstanding at the end of the period, the Common Stock issuable upon conversion or exercise of any outstanding securities convertible or exercisable for Common Stock and the exchange ratio or exercise price applicable thereto, and the number of shares of issued stock options and stock options not yet issued but reserved for issuance, if any, all in sufficient detail as to permit the Major Investors to calculate their respective percentage equity ownership in the Company;

(d) as soon as practicable, but in any event before the end of each fiscal year, a budget and business plan for the next fiscal year (collectively, the “**Budget**”), approved by the Board of Directors and prepared on a monthly basis, including balance sheets, income statements, and statements of cash flow for such months and, promptly after prepared, any other budgets or revised budgets prepared by the Company;

(e) such other information relating to the financial condition, business, prospects, or corporate affairs of the Company as any Major Investor may from time to time reasonably request; provided, however, that the Company shall not be obligated under this Subsection 3.1 to provide information (i) that the Company reasonably determines in good faith to be a trade secret or confidential information (unless covered by an enforceable confidentiality agreement in a form acceptable to the Company) or (ii) the disclosure of which would adversely affect the attorney-client privilege between the Company and its counsel.

If, for any period, the Company has any subsidiary whose accounts are consolidated with those of the Company, then in respect of such period the financial statements delivered pursuant to the foregoing sections shall be the consolidated and consolidating financial statements of the Company and all such consolidated subsidiaries.

Notwithstanding anything else in this Subsection 3.1 to the contrary, the Company may cease providing the information set forth in this Subsection 3.1 during the period starting with the date sixty (60) days before the Company’s good-faith estimate of the date of filing of a registration statement if it reasonably concludes it must do so to comply with the SEC rules applicable to such registration statement and related offering; provided that the Company’s covenants under this Subsection 3.1 shall be reinstated at such time as the Company is no longer actively employing its commercially reasonable efforts to cause such registration statement to become effective.

3.2 Inspection. The Company shall permit each Major Investor (provided that the Board of Directors has not reasonably determined that such Major Investor is a competitor of the Company), at such Major Investor’s expense, to visit and inspect the Company’s properties; examine its books of account and records; and discuss the Company’s affairs, finances, and accounts with its officers, during normal business hours of the Company as may be reasonably

requested by the Major Investor; provided, however, that the Company shall not be obligated pursuant to this Subsection 3.2 to provide access to any information that it reasonably and in good faith considers to be a trade secret or confidential information (unless covered by an enforceable confidentiality agreement, in form acceptable to the Company) or the disclosure of which would adversely affect the attorney-client privilege between the Company and its counsel.

3.3 Termination of Information Rights. The covenants set forth in Subsection 3.1 and 3.2 shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO, (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, or (iii) upon a Deemed Liquidation Event, as such term is defined in the Company's Certificate of Incorporation, whichever event occurs first; provided, however, that in the event the covenants set forth in Subsection 3.1 terminate upon a Deemed Liquidation Event, if the consideration received by the Investors in such Deemed Liquidation Event includes securities that are not publicly traded (which, for the avoidance of doubt, shall not include contingent value rights), the Company will use commercially reasonable efforts to ensure that the Major Investors receive financial information from the acquiring company or other successor to the Company comparable to those set forth in Section 3.1 of this Agreement.

3.4 Confidentiality. Each Investor agrees that such Investor will keep confidential and will not disclose, divulge, or use for any purpose (other than to monitor its investment in the Company) any confidential information obtained from the Company pursuant to the terms of this Agreement prior to the Company's IPO (except with respect to information furnished pursuant to Section 2 of this Agreement) (including notice of the Company's intention to file a registration statement), unless such confidential information (a) is known or becomes known to the public in general (other than as a result of a breach of this Subsection 3.4 by such Investor), (b) is or has been independently developed or conceived by the Investor without use of the Company's confidential information, or (c) is or has been made known or disclosed to the Investor by a third party without a breach of any obligation of confidentiality such third party may have to the Company; provided, however, that an Investor may disclose confidential information (i) to its attorneys, accountants, consultants, and other professionals to the extent necessary to obtain their services in connection with monitoring its investment in the Company; (ii) to any prospective purchaser of any Registrable Securities from such Investor, if such prospective purchaser agrees to be bound by the provisions of this Subsection 3.4; (iii) to any Affiliate of such Investor in the ordinary course of business, provided that such Investor informs such Person that such information is confidential and directs such Person to maintain the confidentiality of such information; or (iv) as may otherwise be required by law, provided that the Investor promptly notifies the Company of such disclosure and takes reasonable steps to minimize the extent of any such required disclosure. Furthermore, nothing contained herein shall prevent any Investor or any person receiving confidential information from an Investor in accordance with this Subsection 3.4 (each, a "**Permitted Disclosee**") from entering into any business, entering into any agreement with a third party, or investing in or engaging in investment discussions with any other company (whether or not competitive with the Company), provided that such Investor or Permitted Disclosee does not, except as permitted in accordance with this Subsection 3.4, disclose any proprietary or confidential information of the Company in connection with such activities; and provided further, each Investor may identify the Company and the value of such Investor's security holdings in the Company in accordance with applicable investment reporting and disclosure regulations or internal policies and respond to routine examinations, demands, requests or reporting requirements of any regulator without prior notice to or consent from the Company.

4. Rights to Future Stock Issuances.

4.1 Right of First Offer. Subject to the terms and conditions of this Subsection 4.1 and applicable securities laws, if the Company proposes to offer or sell any New Securities, the Company shall first offer such New Securities to each Major Investor. A Major Investor shall be entitled to apportion the right of first offer hereby granted to it, in such proportions as it deems appropriate, among itself and its Affiliates; provided that each such Affiliate (x) agrees to enter into this Agreement and each of (i) the Voting Agreement of even date herewith by and among the Company, the Investors and the other parties named therein and (ii) the Right of First Refusal and Co-Sale Agreement of even date herewith by and among the Company, the Investors and the other parties named therein, as an **“Investor”** under each such agreement and (y) agrees to purchase at least such number of New Securities as are allocable hereunder to the Major Investor holding the fewest number of shares of Preferred Stock and any other Derivative Securities.

(a) The Company shall give notice (the **“Offer Notice”**) to each Major Investor, stating (i) its bona fide intention to offer such New Securities, (ii) the number of such New Securities to be offered, and (iii) the price and terms, if any, upon which it proposes to offer such New Securities.

(b) By notification to the Company within twenty (20) days after the Offer Notice is given, each Major Investor may elect to purchase or otherwise acquire, at the price and on the terms specified in the Offer Notice, up to that portion of such New Securities which equals the proportion that the shares of Common Stock issuable upon the conversion of the Preferred Stock then held by such Major Investor bears to the total number of shares of Common Stock issuable upon the conversion of all Preferred Stock of the Company then outstanding. At the expiration of such twenty (20) day period, the Company shall promptly notify each Major Investor that elects to purchase or acquire all the shares available to it (each, a **“Fully Exercising Investor”**) of any other Major Investor’s failure to do likewise. During the ten (10) day period commencing after the Company has given such notice, each Fully Exercising Investor may, by giving notice to the Company, elect to purchase or acquire, in addition to the number of shares specified above, up to that portion of the New Securities for which Major Investors were entitled to subscribe but that were not subscribed for by the Major Investors which is equal to the proportion that the Common Stock issued and held, or issuable (directly or indirectly) upon conversion and/or exercise, as applicable, of Preferred Stock and any other Derivative Securities then held, by such Fully Exercising Investor bears to the Common Stock issued and held, or issuable (directly or indirectly) upon conversion and/or exercise, as applicable, of the Preferred Stock and any other Derivative Securities then held, by all Fully Exercising Investors who wish to purchase such unsubscribed shares. The closing of any sale pursuant to this Subsection 4.1(b) shall occur within the later of ninety (90) days of the date that the Offer Notice is given and the date of initial sale of New Securities pursuant to Subsection 4.1(c).

(c) If all New Securities referred to in the Offer Notice are not elected to be purchased or acquired as provided in Subsection 4.1(b), the Company may, during the ninety (90) day period following the expiration of the periods provided in Subsection 4.1(b), offer and

sell the remaining unsubscribed portion of such New Securities to any Person or Persons at a price not less than, and upon terms no more favorable to the offeree than, those specified in the Offer Notice. If the Company does not enter into an agreement for the sale of the New Securities within such period, or if such agreement is not consummated within thirty (30) days of the execution thereof, the right provided hereunder shall be deemed to be revived and such New Securities shall not be offered unless first reoffered to the Major Investors in accordance with this Subsection 4.1.

(d) The right of first offer in this Subsection 4.1 shall not be applicable to (i) Exempted Securities (as defined in the Company's Certificate of Incorporation) and (ii) shares of Common Stock issued in the IPO.

4.2 Termination. The covenants set forth in Subsection 4.1 shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO, (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act or (iii) upon a Deemed Liquidation Event, as such term is defined in the Company's Certificate of Incorporation, whichever event occurs first.

#### 5. Additional Covenants.

5.1 Insurance. The Company shall use its commercially reasonable efforts to obtain, within ninety (90) days of the date hereof, from financially sound and reputable insurers, Directors and Officers liability insurance, in an amount and on terms and conditions satisfactory to the Board of Directors, and will use commercially reasonable efforts to cause such insurance policies to be maintained until such time as the Board of Directors determines that such insurance should be discontinued.

5.2 Employee Agreements. The Company will cause (i) each person now or hereafter employed by it or by any subsidiary (or engaged by the Company or any subsidiary as a consultant/independent contractor) with access to confidential information and/or trade secrets to enter into a nondisclosure and proprietary rights assignment agreement; and (ii) each Key Employee to enter into a noncompetition and nonsolicitation agreement, substantially in the form approved by the Board of Directors.

5.3 Employee Stock. Unless otherwise approved by the Board of Directors, all future employees and consultants of the Company who purchase, receive options to purchase, or receive awards of shares of the Company's capital stock after the date hereof shall be required to execute restricted stock or option agreements, as applicable, providing for (i) vesting of shares over a four (4) year period, with the first twenty-five percent (25%) of such shares vesting following twelve (12) months of continued employment or service, and the remaining shares vesting in equal monthly installments over the following thirty-six (36) months, and (ii) a market stand-off provision substantially similar to that in Subsection 2.11. In addition, unless otherwise approved by the Board of Directors, the Company shall retain a "right of first refusal" on employee transfers until the Company's IPO and shall have the right to repurchase unvested shares at cost upon termination of employment of a holder of restricted stock.

5.4 Board Matters. Unless otherwise determined by the vote of a majority of the directors then in office, the Board shall meet quarterly in accordance with an agreed-upon

schedule. The Company shall reimburse each nonemployee directors for all reasonable out-of-pocket travel expenses incurred (consistent with the Company's travel policy) in connection with attending meetings of the Board of Directors or committees of the Board. Each Preferred Director shall be entitled in such person's discretion to be a member of any Board committee. Atlas Venture Fund X, L.P. and Atlas Venture Opportunity Fund I, L.P., or their Affiliates (together, "**Atlas**") shall appoint a Chair of the Board.

5.5 **Successor Indemnification.** If the Company or any of its successors or assignees consolidates with or merges into any other Person and is not the continuing or surviving corporation or entity of such consolidation or merger, then to the extent necessary, proper provision shall be made so that the successors and assignees of the Company assume the obligations of the Company with respect to indemnification of members of the Board of Directors as in effect immediately before such transaction, whether such obligations are contained in the Company's Bylaws, its Certificate of Incorporation, or elsewhere, as the case may be.

5.6 **Indemnification Matters.** The Company hereby acknowledges that one (1) or more of the directors nominated to serve on the Board of Directors by the Investors (each a "**Fund Director**") may have certain rights to indemnification, advancement of expenses and/or insurance provided by one or more of the Investors and certain of their Affiliates (collectively, the "**Fund Indemnitors**"). The Company hereby agrees (a) that it is the indemnitor of first resort (*i.e.*, its obligations to any such Fund Director are primary and any obligation of the Fund Indemnitors to advance expenses or to provide indemnification for the same expenses or liabilities incurred by such Fund Director are secondary), (b) that it shall be required to advance the full amount of expenses incurred by such Fund Director and shall be liable for the full amount of all expenses, judgments, penalties, fines and amounts paid in settlement by or on behalf of any such Fund Director to the extent legally permitted and as required by the Company's Certificate of Incorporation or Bylaws of the Company (or any agreement between the Company and such Fund Director), without regard to any rights such Fund Director may have against the Fund Indemnitors, and, (c) that it irrevocably waives, relinquishes and releases the Fund Indemnitors from any and all claims against the Fund Indemnitors for contribution, subrogation or any other recovery of any kind in respect thereof. The Company further agrees that no advancement or payment by the Fund Indemnitors on behalf of any such Fund Director with respect to any claim for which such Fund Director has sought indemnification from the Company shall affect the foregoing and the Fund Indemnitors shall have a right of contribution and/or be subrogated to the extent of such advancement or payment to all of the rights of recovery of such Fund Director against the Company.

5.7 **Right to Conduct Activities.** The Company hereby agrees and acknowledges that Atlas, Fidelity, Deerfield Partners, L.P. ("**Deerfield**"), Casdin Partners Master Fund, L.P. and Casdin Venture Opportunities Fund, L.P. (together, "**Casdin**"), Foresite Capital Fund IV, LP ("**Foresite**"), Wellington, the Wellington Investors, Harvard Management Private Equity Corporation ("**Harvard**"), Farallon Capital Management, L.L.C. and its Affiliates (collectively "**Farallon**") and the T. Rowe Price Investors and their Affiliates are professional investment funds, and as such invest in numerous portfolio companies, some of which may be deemed competitive with the Company's business (as currently conducted or as currently propose to be conducted). The Company hereby agrees that, to the extent permitted under applicable law, no such Investor shall be liable to the Company for any claim arising out of, or based upon, (i) the

investment by such Investor in any entity competitive with the Company or (ii) actions taken by any partner, officer or other representative of such Investor to assist any such competitive company, whether or not such action was taken as a member of the board of directors of such competitive company or otherwise, and whether or not such action has a detrimental effect on the Company; provided, however, that the foregoing shall not relieve (x) any of the Investors from liability associated with the unauthorized disclosure of the Company's confidential information obtained pursuant to this Agreement, or (y) any director or officer of the Company from any liability associated with his or her fiduciary duties to the Company. Nothing in this Agreement shall preclude or create an obligation or duty restricting Atlas, Fidelity, Deerfield, Casdin, Foresite, Wellington, the Wellington Investors, Harvard, Farallon, the T. Rowe Price Investors or T. Rowe Price from evaluating or purchasing securities, including publicly traded securities, of a particular enterprise, whether or not such enterprise has products or services which compete with those of the Company.

5.8 Termination of Covenants. The covenants set forth in this Section 5, except for Subsections 5.5, 5.6, and 5.7, shall terminate and be of no further force or effect (i) immediately before the consummation of the IPO, (ii) when the Company first becomes subject to the periodic reporting requirements of Section 12(g) or 15(d) of the Exchange Act, or (iii) upon a Deemed Liquidation Event, as such term is defined in the Company's Certificate of Incorporation, whichever event occurs first.

6. Miscellaneous.

6.1 Successors and Assigns. The rights under this Agreement may be assigned (but only with all related obligations) by a Holder to a transferee of Registrable Securities that (i) is an Affiliate of a Holder; (ii) is a Holder's Immediate Family Member or a trust for the benefit of an individual Holder or one or more of such Holder's Immediate Family Members; or (iii) after such transfer, holds at least 600,000 shares of Registrable Securities (subject to appropriate adjustment for stock splits, stock dividends, combinations, and other recapitalizations) or, if less, all of the Registrable Securities held by such Holder; provided, however, that (x) the Company is, within a reasonable time after such transfer, furnished with written notice of the name and address of such transferee and the Registrable Securities with respect to which such rights are being transferred; and (y) such transferee agrees in a written instrument delivered to the Company to be bound by and subject to the terms and conditions of this Agreement, including the provisions of Subsection 2.11. For the purposes of determining the number of shares of Registrable Securities held by a transferee, the holdings of a transferee (1) that is an Affiliate or stockholder of a Holder; (2) who is a Holder's Immediate Family Member; or (3) that is a trust for the benefit of an individual Holder or such Holder's Immediate Family Member shall be aggregated together and with those of the transferring Holder; provided further that all transferees who would not qualify individually for assignment of rights shall have a single attorney-in-fact for the purpose of exercising any rights, receiving notices, or taking any action under this Agreement. The terms and conditions of this Agreement inure to the benefit of and are binding upon the respective successors and permitted assignees of the parties. Nothing in this Agreement, express or implied, is intended to confer upon any party other than the parties hereto or their respective successors and permitted assignees any rights, remedies, obligations or liabilities under or by reason of this Agreement, except as expressly provided herein.

6.2 Governing Law. This Agreement shall be governed by the internal law of the State of Delaware.

6.3 Counterparts. This Agreement may be executed in two (2) or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature complying with the U.S. federal ESIGN Act of 2000, *e.g.*, www.docusign.com) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes.

6.4 Titles and Subtitles. The titles and subtitles used in this Agreement are for convenience only and are not to be considered in construing or interpreting this Agreement.

6.5 Notices. All notices and other communications given or made pursuant to this Agreement shall be in writing and shall be deemed effectively given upon the earlier of actual receipt or (i) personal delivery to the party to be notified; (ii) when sent, if sent by electronic mail or facsimile during the recipient's normal business hours, and if not sent during normal business hours, then on the recipient's next business day; (iii) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid; or (iv) one (1) business day after the business day of deposit with a nationally recognized overnight courier, freight prepaid, specifying next-day delivery, with written verification of receipt. All communications shall be sent to the respective parties only at their addresses as set forth on Schedule A or Schedule B (as applicable) hereto, or to the principal office of the Company and to the attention of the Chief Executive Officer, in the case of the Company, or to such email address, facsimile number, or address as subsequently modified by written notice given in accordance with this Subsection 6.5. If notice is given to the Company, a copy shall also be sent to Wilmer Cutler Pickering Hale and Dorr LLP, 60 State Street, Boston, MA 02109, Attn: Stuart Falber.

6.6 Amendments and Waivers. Any term of this Agreement may be amended and the observance of any term of this Agreement may be waived (either generally or in a particular instance, and either retroactively or prospectively) only with the written consent of (i) the Company and (ii) the Requisite Holders; provided that the Company may in its sole discretion waive compliance with Subsection 2.12(c) (and the Company's failure to object promptly in writing after notification of a proposed assignment allegedly in violation of Subsection 2.12(c) shall be deemed to be a waiver); provided further that any provision hereof may be waived by any waiving party on such party's own behalf, without the consent of any other party. The last sentence of Subsection 5.4 and this sentence may not be amended without the prior written consent of Atlas; and provided further that Subsection 5.7 and this sentence may not be amended with respect to a particular Investor without the prior written consent of such Investor. Notwithstanding the foregoing, (a) this Agreement may not be amended or terminated and the observance of any term hereof may not be waived with respect to any Investor without the written consent of such Investor, unless such amendment, termination, or waiver applies to all Investors in the same fashion (it being agreed that a waiver of the provisions of Section 4 with respect to a particular transaction shall be deemed to apply to all Investors in the same fashion if such waiver does so by its terms, notwithstanding the fact that certain Investors may nonetheless, by agreement with the Company, purchase securities in such transaction; provided, that in the event of any such waiver of the provisions of Section 4 with respect to a particular issuance of New Securities, if any of the Major

Investors participate in such waiver and purchase securities in such particular offering then each Major Investor shall have the right to participate in such offering by purchasing up to that portion of the New Securities which equals the proportion that the shares of Common Stock issuable upon the conversion of the Preferred Stock then held by such Major Investor bears to the total number of shares of Common Stock issuable upon the conversion of all Preferred Stock of the Company then outstanding, on the same terms as such other stockholders) and (b) Subsections 3.1 and 3.2, Section 4 and any other section of this Agreement that is only applicable to the Major Investors (including this clause (b) of this Subsection 6.6) may not be amended, modified, terminated or waived without the written consent of the holders of at least a majority of the Registrable Securities then outstanding and held by the Major Investors. The Company shall give prompt notice of any amendment or termination hereof or waiver hereunder to any party hereto that did not consent in writing to such amendment, termination, or waiver. Any amendment, termination, or waiver effected in accordance with this Subsection 6.6 shall be binding on all parties hereto, regardless of whether any such party has consented thereto. No waivers of or exceptions to any term, condition, or provision of this Agreement, in any one or more instances, shall be deemed to be or construed as a further or continuing waiver of any such term, condition, or provision.

6.7 Severability. In case any one or more of the provisions contained in this Agreement is for any reason held to be invalid, illegal or unenforceable in any respect, such invalidity, illegality, or unenforceability shall not affect any other provision of this Agreement, and such invalid, illegal, or unenforceable provision shall be reformed and construed so that it will be valid, legal, and enforceable to the maximum extent permitted by law.

6.8 Aggregation of Stock. All shares of Registrable Securities held or acquired by Affiliates shall be aggregated together for the purpose of determining the availability of any rights under this Agreement and such Affiliated persons may apportion such rights as among themselves in any manner they deem appropriate.

6.9 Additional Investors. Notwithstanding anything to the contrary contained herein, if the Company issues additional shares of Preferred Stock after the date hereof, whether pursuant to the Purchase Agreement or otherwise, any purchaser of such shares of Preferred Stock may become a party to this Agreement by executing and delivering an additional counterpart signature page to this Agreement, and thereafter shall be deemed an "Investor" for all purposes hereunder. No action or consent by the Investors shall be required for such joinder to this Agreement by such additional Investor, so long as such additional Investor has agreed in writing to be bound by all of the obligations as an "Investor" hereunder.

6.10 Entire Agreement. This Agreement (including any Schedules and Exhibits hereto), together with each Management Rights Letter (as defined in the Purchase Agreement), constitutes the full and entire understanding and agreement among the parties with respect to the subject matter hereof, and any other written or oral agreement relating to the subject matter hereof existing between the parties is expressly canceled.

6.11 Dispute Resolution. The parties (a) hereby irrevocably and unconditionally submit to the jurisdiction of the state courts of Delaware and to the jurisdiction of the federal district courts of Delaware for the purpose of any suit, action or other proceeding arising out of or based upon this Agreement, (b) agree not to commence any suit, action or other proceeding arising



out of or based upon this Agreement except in the state courts of Delaware or the federal district courts of Delaware, and (c) hereby waive, and agree not to assert, by way of motion, as a defense, or otherwise, in any such suit, action or proceeding, any claim that it is not subject personally to the jurisdiction of the above-named courts, that its property is exempt or immune from attachment or execution, that the suit, action or proceeding is brought in an inconvenient forum, that the venue of the suit, action or proceeding is improper or that this Agreement or the subject matter hereof may not be enforced in or by such court.

WAIVER OF JURY TRIAL: EACH PARTY HEREBY WAIVES ITS RIGHTS TO A JURY TRIAL OF ANY CLAIM OR CAUSE OF ACTION BASED UPON OR ARISING OUT OF THIS AGREEMENT, THE OTHER TRANSACTION DOCUMENTS, THE SECURITIES OR THE SUBJECT MATTER HEREOF OR THEREOF. THE SCOPE OF THIS WAIVER IS INTENDED TO BE ALL-ENCOMPASSING OF ANY AND ALL DISPUTES THAT MAY BE FILED IN ANY COURT AND THAT RELATE TO THE SUBJECT MATTER OF THIS TRANSACTION, INCLUDING, WITHOUT LIMITATION, CONTRACT CLAIMS, TORT CLAIMS (INCLUDING NEGLIGENCE), BREACH OF DUTY CLAIMS, AND ALL OTHER COMMON LAW AND STATUTORY CLAIMS. THIS SECTION HAS BEEN FULLY DISCUSSED BY EACH OF THE PARTIES HERETO AND THESE PROVISIONS WILL NOT BE SUBJECT TO ANY EXCEPTIONS. EACH PARTY HERETO HEREBY FURTHER WARRANTS AND REPRESENTS THAT SUCH PARTY HAS REVIEWED THIS WAIVER WITH ITS LEGAL COUNSEL, AND THAT SUCH PARTY KNOWINGLY AND VOLUNTARILY WAIVES ITS JURY TRIAL RIGHTS FOLLOWING CONSULTATION WITH LEGAL COUNSEL.

Each party will bear its own costs in respect of any disputes arising under this Agreement. The prevailing party shall be entitled to reasonable attorney's fees, costs, and necessary disbursements in addition to any other relief to which such party may be entitled. Each of the parties to this Agreement consents to personal jurisdiction for any equitable action sought in a federal district court of Delaware or any court of the State of Delaware having subject matter jurisdiction.

6.12 Delays or Omissions. No delay or omission to exercise any right, power, or remedy accruing to any party under this Agreement, upon any breach or default of any other party under this Agreement, shall impair any such right, power, or remedy of such nonbreaching or nondefaulting party, nor shall it be construed to be a waiver of or acquiescence to any such breach or default, or to any similar breach or default thereafter occurring, nor shall any waiver of any single breach or default be deemed a waiver of any other breach or default theretofore or thereafter occurring. All remedies, whether under this Agreement or by law or otherwise afforded to any party, shall be cumulative and not alternative.

*[Remainder of Page Intentionally Left Blank]*

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

GENERATION BIO CO.

By: /s/ Geoffrey McDonough

Name: Geoffrey McDonough

Title: Chief Executive Officer

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**T. ROWE PRICE NEW HORIZONS FUND, INC.  
T. ROWE PRICE NEW HORIZONS TRUST  
T. ROWE PRICE U.S. EQUITIES TRUST  
MASSMUTUAL SELECT FUNDS -  
MASSMUTUAL SELECT T. ROWE PRICE  
SMALL AND MID CAP BLEND FUND**

Each account, severally not jointly

By: T. Rowe Price Associates, Inc., Investment Adviser or  
Subadviser, as applicable

By: /s/ Franciso Alonso

Name: Franciso Alonso

Title: Vice President

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**T. ROWE PRICE HEALTH SCIENCES FUND, INC.  
TD MUTUAL FUNDS – TD HEALTH SCIENCES FUND  
VALIC COMPANY I – HEALTH SCIENCES FUND**

**T. ROWE PRICE HEALTH SCIENCES PORTFOLIO**

Each account, severally not jointly

By: T. Rowe Price Associates, Inc., Investment Adviser or  
Subadviser, as applicable

By: /s/ John Hall

Name: John Hall

Title: Vice President

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**T. ROWE PRICE SMALL-CAP STOCK FUND, INC.  
T. ROWE PRICE INSTITUTIONAL SMALL-CAP  
STOCK FUND  
T. ROWE PRICE SPECTRUM CONSERVATIVE  
ALLOCATION FUND  
T. ROWE PRICE SPECTRUM MODERATE  
ALLOCATION FUND  
T. ROWE PRICE SPECTRUM MODERATE  
GROWTH ALLOCATION FUND  
T. ROWE PRICE MODERATE ALLOCATION  
PORTFOLIO VALIC COMPANY I - SMALL CAP  
FUND  
TD MUTUAL FUNDS - TD U.S. SMALL-CAP EQUITY  
FUND  
T. ROWE PRICE U.S. SMALL-CAP CORE EQUITY  
TRUST U.S. SMALL-CAP STOCK TRUST  
MINNESOTA LIFE INSURANCE COMPANY  
COSTCO 401(K) RETIREMENT PLAN  
MASSMUTUAL SELECT FUNDS -  
MASSMUTUAL SELECT T. ROWE PRICE SMALL  
AND MID CAP BLEND FUND**

Each account, severally not jointly

By: T. Rowe Price Associates, Inc., Investment Adviser or  
Subadviser, as applicable

By: /s/ Franciso Alonso

Name: Franciso Alonso

Title: Vice President

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**ATLAS VENTURE OPPORTUNITY FUND I, L.P.**

By: /s/ Ommer Chohan

Name: Ommer Chohan

Title: Chief Financial Officer

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**FIDELITY MT. VERNON STREET TRUST:  
FIDELITY SERIES GROWTH COMPANY FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY GROWTH COMPANY COMMINGLED  
POOL**

By: Fidelity Management Trust Company, as Trustee

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY MT. VERNON STREET TRUST:  
FIDELITY GROWTH COMPANY FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

**FIDELITY SECURITIES FUND: FIDELITY BLUE  
CHIP GROWTH FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY BLUE CHIP GROWTH COMMINGLED  
POOL**

By: Fidelity Management Trust Company, as Trustee

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIAM TARGET DATE BLUE CHIP GROWTH  
COMMINGLED POOL**

By: Fidelity Institutional Asset Management Trust Company  
as Trustee

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY SECURITIES FUND: FIDELITY BLUE  
CHIP GROWTH K6 FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**



**FIDELITY SECURITIES FUND: FIDELITY SERIES  
CLUE CHIP GROWTH FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY SECURITIES FUND: FIDELITY FLEX  
LARGE CAP GROWTH FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY PURITAN TRUST: FIDELITY PURITAN  
FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY CENTRAL INVESTMENT PORTFOLIOS  
LLC: FIDELITY HEALTH CARE CENTRAL FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

**VARIABLE INSURANCE PRODUCTS FUND IV:  
HEALTH CARE PORTFOLIO**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY SELECT PORTFOLIOS: HEALTH CARE  
PORTFOLIO**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY ADVISOR SERIES VII: FIDELITY  
ADVISOR HEALTH CARE FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY CONTRAFUND: FIDELITY  
CONTRAFUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

**FIDELITY CONTRAFUND: FIDELITY  
CONTRAFUND K6**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY CONTRAFUND COMMINGLED POOL**

By: Fidelity Management Trust Company, as Trustee

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY SELECT PORTFOLIOS:  
PHARMACEUTICALS PORTFOLIO**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**FIDELITY BLUE CHIP GROWTH INSTITUTIONAL  
TRUST**

By: Its Manager Fidelity Investments Canada ULC

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

**FIDELITY MT. VERNON STREET TRUST:  
FIDELITY GROWTH COMPANY K6 FUND**

By: /s/ Colm Hogan

Name: Colm Hogan

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**INVUS PUBLIC EQUITIES, LP**

By: /s/ Raymond Debbane

Name: Raymond Debbane

Title: President of the General Partner

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**PURCHASER:**

**DEERFIELD PARTNERS, L.P.**

By: Deerfield Mgmt, L.P., General Partner

By: J.E. Flynn Capital, LLC, General Partner

By: /s/ David J. Clark

Name: David J. Clark

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

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**PURCHASER:**

**CASDIN VENTURE OPPORTUNITIES FUND, L.P.**

By: Casdin Venture Opportunities Fund GP, LLC,  
its General Partner

By: /s/ Eli Casdin

Name: Eli Casdin

Title: Managing Member

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

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**PURCHASER:**

**CASDIN PARTNERS MASTER FUND, L.P.**

By: Casdin Partners GP, LLC, its General Partner

By: /s/ Eli Casdin

Name: Eli Casdin

Title: Managing Member

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**



IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**FORESITE CAPITAL FUND IV, L.P.**

By: Foresite Capital Management IV, LLC

Its: General Partner

By: /s/ Dennis D. Ryan

Name: Dennis D. Ryan

Title: Chief Financial Officer

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**LEERINK PARTNERS CO-INVESTMENT FUND,  
LLC**

By: /s/ Joseph R. Gentile

Name: Joseph R. Gentile

Title: Manager

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**ZONE HEALTHCARE HOLDINGS, LLC**

By: Farallon Capital Management, L.L.C., its Manager

By: /s/ Philip Dreyfuss

Name: Philip Dreyfuss

Title: Managing Member

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

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**PURCHASER:**

**WELLINGTON BIOMEDICAL INNOVATION  
MASTER INVESTORS (CAYMAN) I L.P.**

/s/ Valerie N. Tipping

By: Wellington Management Company LLP, as investment  
adviser

Name: Valerie N. Tipping

Title: Managing Director & Counsel

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**THE TRUSTEES OF COLUMBIA UNIVERSITY IN  
THE CITY OF NEW YORK**

By: /s/ Julius Mercado

Name: Julius Mercado

Title: Chief Operating Officer;  
Columbia Investment Management  
Company, L.L.C.

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**HARVARD MANAGEMENT PRIVATE EQUITY CORPORATION**

By: /s/ Elaine Chan

Name: Elaine Chan

Title: Authorized Signatory

By: /s/ Shashank Mathur

Name: Shashank Mathur

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

**GB CO-INVESTMENT LLC**

By: /s/ Owen Littman

Name: Owen Littman

Title: Authorized Signatory

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

/s/ Gustav Christensen

\_\_\_\_\_

Gustav Christensen

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**



IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

/s/ Arthur M. Krieg

Arthur M. Krieg

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

/s/ Anthony Quinn

Anthony Quinn

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

/s/ Charles Rowland

Charles Rowland

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

IN WITNESS WHEREOF, the parties have executed this Second Amended and Restated Investors' Rights Agreement as of the date first written above.

**PURCHASER:**

/s/ Catherine Stehman-Breen

\_\_\_\_\_  
Catherine Stehman-Breen

**SIGNATURE PAGE TO SECOND AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT**

**SCHEDULE A**

**Investors**

**T. Rowe Price Health Sciences Fund, Inc.**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**TD Mutual Funds - TD Health Sciences Fund**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**VALIC Company I - Health Sciences Fund**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**T. Rowe Price Health Sciences Portfolio**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**T. Rowe Price New Horizons Fund, Inc.**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**T. Rowe Price New Horizons Trust**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**T. Rowe Price U.S. Equities Trust**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**MassMutual Select Funds - MassMutual Select**

**T. Rowe Price Small and Mid Cap Blend Fund**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**T. Rowe Price Small-Cap Stock Fund, Inc.**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**T. Rowe Price Institutional Small-Cap Stock Fund**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**T. Rowe Price Spectrum Conservative Allocation Fund**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**T. Rowe Price Spectrum Moderate Allocation Fund**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**T. Rowe Price Spectrum Moderate Growth Allocation Fund**

T. Rowe Price Associates, Inc.  
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Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**T. Rowe Price Moderate Allocation Portfolio**

T. Rowe Price Associates, Inc.  
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Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**U.S. Small-Cap Stock Trust**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**VALIC Company I - Small Cap Fund**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**TD Mutual Funds - TD U.S. Small-Cap Equity Fund**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**T. Rowe Price U.S. Small-Cap Core Equity Trust**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**Minnesota Life Insurance Company**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
[\*\*]

**Costco 401(k) Retirement Plan**

T. Rowe Price Associates, Inc.  
100 East Pratt Street  
Baltimore, MD 21202  
Attn: Andrew Baek, Vice President  
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**Zone Healthcare Holdings, LLC**  
c/o Farallon Capital Management, L.L.C.  
One Maritime Plaza, Suite 2100  
San Francisco, CA 94111  
Attn: Philip Dreyfuss  
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**Wellington Biomedical Innovation Master  
Investors (Cayman) I L.P.**  
c/o Wellington Management Company LLP  
Legal and Compliance  
280 Congress Street  
Boston, MA 02210  
[\*\*]

With a copy (which shall not constitute notice) to:

Cooley LLP  
500 Boylston Street, 14th Floor  
Boston, MA 02116  
Attn: Joshua Rottner  
[\*\*]

**The Trustees of Columbia University  
in the City of New York**  
405 Lexington Ave. 63rd Floor  
New York, NY 10174

**Harvard Management Private Equity  
Corporation**  
600 Atlantic Avenue  
Boston, MA 02210  
Attn: Elaine Chan / Emily Holden  
[\*\*]

**GB Co-Investment LLC**  
599 Lexington Ave., 20th floor  
New York, NY 10022

**Atlas Venture Opportunity Fund I, L.P.**  
400 Technology Square  
Cambridge, MA 02139

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**Atlas Venture Fund X, L.P.**

400 Technology Square  
Cambridge, MA 02139

**Invus Public Equities, LP**

750 Lexington Avenue, Floor 30  
New York, NY 10022

With copies to:

The Invus Group LLC  
750 Lexington Avenue  
New York, NY 10022  
Attn: Philippe J. Amouyal

**Casdin Partners Master Fund, L.P.**

1350 Avenue of the Americas, Suite 2600  
New York, New York 10019

**Casdin Venture Opportunities Fund, L.P.**

1350 Avenue of the Americas, Suite 2600  
New York, New York 10019

**Deerfield Partners, L.P.**

780 Third Avenue, 37<sup>th</sup> Floor  
New York, NY 10017  
Attention: Lawrence Atinsky

**Fidelity Mt. Vernon Street Trust: Fidelity Series  
Growth Company Fund**

State Street Bank & Trust  
P.O. Box 5756  
Boston, Massachusetts 02206  
Attn: WAVELENGTH + CO Fidelity Mt. Vernon  
Street Trust: Fidelity Series Growth Company Fund  
[\*\*]

**Fidelity Growth Company Commingled Pool**

Mag & Co.  
c/o Brown Brothers Harriman & Co.  
Attn: Corporate Actions /Vault  
140 Broadway  
New York, NY 10005  
[\*\*]

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**Fidelity Mt. Vernon Street Trust: Fidelity  
Growth Company Fund**

BNY Mellon

Attn: Stacey Wolfe

525 William Penn Place Rm 0400

Pittsburgh, PA 15259

[\*\*]

**Fidelity Securities Fund: Fidelity Blue Chip  
Growth Fund**

M.Gardiner & Co

c/o JPMorgan Chase Bank, N.A

P.O. Box 35308

Newark, NJ 07101-8006

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**Fidelity Blue Chip Growth Commingled Pool**

Mag & Co.

c/o Brown Brothers Harriman & Co.

Attn: Corporate Actions /Vault

140 Broadway

New York, NY 10005

[\*\*]

**FIAM Target Date Blue Chip Growth  
Commingled Pool**

State Street Bank & Trust

P.O. Box 5756

Boston, Massachusetts 02206

Attn: FLAPPER CO fbo FIAM Target Date Blue

Chip Growth Commingled Pool

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**Fidelity Securities Fund: Fidelity Blue Chip  
Growth K6 Fund**

The Northern Trust Company  
Attn: Fidelity Client Team – GFS Custody, C-1N  
801 South Canal Street  
Chicago, IL 60607  
Fidelity Securities Fund: Fidelity Blue Chip Growth K6 Fund  
[\*\*]

**Fidelity Securities Fund: Fidelity Series Blue Chip  
Growth Fund**

State Street Bank & Trust  
PO Box 5756  
Boston, Massachusetts 02206  
Attn: WAVECHART + CO fbo Fidelity Securities  
Fund: Fidelity Series Blue Chip Growth Fund  
[\*\*]

**Fidelity Securities Fund: Fidelity Flex Large Cap  
Growth Fund**

The Northern Trust Company  
Attn: Fidelity Client Team – GFS Custody, C-1N  
801 South Canal Street  
Chicago, IL 60607  
Fidelity Securities Fund: Fidelity Flex Large Cap  
Growth Fund  
[\*\*]

**Fidelity Puritan Trust: Fidelity Puritan Fund**

M.Gardiner & Co  
c/o JPMorgan Chase Bank, N.A  
P.O. Box 35308  
Newark, NJ 07101-8006  
[\*\*]

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**Fidelity Central Investment Portfolios LLC:**

**Fidelity Health Care Central Fund**

M.Gardiner & Co

c/o JPMorgan Chase Bank, N.A

P.O. Box 35308

Newark, NJ 07101-8006

[\*\*]

**Variable Insurance Products Fund IV: Health  
Care Portfolio**

M.Gardiner & Co

c/o JPMorgan Chase Bank, N.A

P.O. Box 35308

Newark, NJ 07101-8006

[\*\*]

**Fidelity Select Portfolios: Health Care Portfolio**

Mag & Co.

c/o Brown Brothers Harriman & Co.

Attn: Corporate Actions /Vault

140 Broadway

New York, NY 10005

[\*\*]

**Fidelity Advisor Series VII: Fidelity Advisor  
Health Care Fund**

M.Gardiner & Co

c/o JPMorgan Chase Bank, N.A

P.O. Box 35308

Newark, NJ 07101-8006

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**Fidelity Contrafund: Fidelity Contrafund**

Mag & Co.

c/o Brown Brothers Harriman & Co.

Attn: Corporate Actions /Vault

140 Broadway

New York, NY 10005

[\*\*]

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**Fidelity Contrafund: Fidelity Contrafund K6**

The Northern Trust Company  
Attn: Fidelity Client Team – GFS Custody, C-1N  
801 South Canal Street  
Chicago, IL 60607  
Fidelity Contrafund: Fidelity Contrafund K6  
[\*\*]

**Fidelity Contrafund Commingled Pool**

Mag & Co.  
c/o Brown Brothers Harriman & Co.  
Attn: Corporate Actions /Vault  
140 Broadway  
New York, NY 10005  
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**Fidelity Select Portfolios: Pharmaceuticals Portfolio**

Mag & Co.  
c/o Brown Brothers Harriman & Co.  
Attn: Corporate Actions /Vault  
140 Broadway  
New York, NY 10005  
[\*\*]

**Fidelity Blue Chip Growth Institutional Trust**

State Street Bank & Trust  
PO Box 5756  
Boston, Massachusetts 02206  
Attn: Nominee fbo fund name  
[\*\*]

**Fidelity Mt. Vernon Street Trust: Fidelity Growth Company K6 Fund**

BNY Mellon  
One Bny Mellon Center  
500 Grant Street AIM 151-2700  
Pittsburgh, PA 15258

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**Foresite Capital Fund IV, LP**

600 Montgomery Street, Suite 4500  
San Francisco, CA 94111

**SVB Leerink Holdings LLC**

One Federal Street, 37th Floor  
Boston, MA 02110  
Attention: General Counsel

**Leerink Partners Co-Investment Fund, LLC**

One Federal Street, 37th Floor  
Boston, MA 02110  
Attention: General Counsel

**Gustav Christensen**

[\*\*]

**William Holodnak**

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**Geoffrey McDonough**

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**Don Nicholson**

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**Arthur M. Krieg**

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**Anthony Quinn**

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**Charles Rowland**

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**Catherine Stehman-Breen**

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**2017 STOCK INCENTIVE PLAN**  
**OF**  
**TORUS THERAPEUTICS, INC.**



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**2017 STOCK INCENTIVE PLAN  
OF  
TORUS THERAPEUTICS, INC.**

1. Purpose

The purpose of this 2017 Stock Incentive Plan (the “Plan”) of Torus Therapeutics, Inc., a Delaware corporation (the “Company”), is to advance the interests of the Company’s stockholders by enhancing the Company’s ability to attract, retain and motivate persons who are expected to make important contributions to the Company and by providing such persons with equity ownership opportunities and performance-based incentives that are intended to better align the interests of such persons with those of the Company’s stockholders. Except where the context otherwise requires, the term “Company” shall include any of the Company’s present and future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Internal Revenue Code of 1986, as amended, and any regulations thereunder (the “Code”) and any other business venture (including, without limitation, joint venture or limited liability company) in which the Company has a controlling interest, as determined by the Board of Directors of the Company (the “Board”); *provided, however*, that such other business ventures shall be limited to entities that, where required by Section 409A of the Code, are eligible issuers of service recipient stock (as defined in Treas. Reg. Section 1.409A-1(b)(5)(iii)(E), or applicable successor regulation).

2. Eligibility

All of the Company’s employees, officers and directors, as well as consultants and advisors to the Company (as such terms consultants and advisors are defined and interpreted for purposes of Rule 701 under the Securities Act of 1933, as amended (the “Securities Act”) (or any successor rule)) are eligible to be granted Awards under the Plan. Each person who is granted an Award under the Plan is deemed a “Participant.” “Award” means Options (as defined in Section 5), SARs (as defined in Section 6), Restricted Stock (as defined in Section 7), Restricted Stock Units (as defined in Section 7) and Other Stock-Based Awards (as defined in Section 8).

3. Administration and Delegation

(a) Administration by the Board. The Plan will be administered by the Board. The Board shall have authority to grant Awards and to adopt, amend and repeal such administrative rules, guidelines and practices relating to the Plan as it shall deem advisable. The Board may construe and interpret the terms of the Plan and any Award agreements entered into under the Plan. The Board may correct any defect, supply any omission or reconcile any inconsistency in the Plan or any Award in the manner and to the extent it shall deem expedient to carry the Plan into effect and it shall be the sole and final judge of such expediency. All actions and decisions by the Board with respect to the Plan and any Awards shall be made in the Board’s discretion and shall be final and binding on all Participants and any other persons having or claiming any interest in the Plan or in any Award.

(b) Appointment of Committees. To the extent permitted by applicable law, the Board may delegate any or all of its powers under the Plan to one or more committees or subcommittees of the Board (each, a “Committee”). All references in the Plan to the “Board” shall mean the Board or a Committee to the extent that the Board’s powers or authority under the Plan have been delegated to such Committee.

#### 4. Stock Available for Awards

(a) Number of Shares. Subject to adjustment under Section 9, Awards may be made under the Plan for up to 5,424,000 shares of common stock, \$0.0001 par value per share, of the Company (the "Common Stock"), any or all of which Awards may be in the form of Incentive Stock Options (as defined in Section 5(b)). If any Award expires or is terminated, surrendered or canceled without having been fully exercised, is forfeited in whole or in part (including as the result of shares of Common Stock subject to such Award being repurchased by the Company at the original issuance price pursuant to a contractual repurchase right), or results in any Common Stock not being issued, the unused Common Stock subject to such Award shall again be available for the grant of Awards under the Plan. Further, shares of Common Stock tendered to the Company by a Participant to exercise an Award or to satisfy tax withholding obligations arising with respect to an Award shall be added to the number of shares of Common Stock available for the grant of Awards under the Plan. However, in the case of Incentive Stock Options, the two immediately preceding sentences shall be subject to any limitations under the Code. Shares issued under the Plan may consist in whole or in part of authorized but unissued shares or treasury shares.

(b) Substitute Awards. In connection with a merger or consolidation of an entity with the Company or the acquisition by the Company of property or stock of an entity, the Board may grant Awards in substitution for any options or other stock or stock-based awards granted by such entity or an affiliate thereof. Substitute Awards may be granted on such terms as the Board deems appropriate in the circumstances, notwithstanding any limitations on Awards contained in the Plan. Substitute Awards shall not count against the overall share limit set forth in Section 4(a), except as may be required by reason of Section 422 and related provisions of the Code.

#### 5. Stock Options

(a) General. The Board may grant options to purchase Common Stock (each, an "Option") and determine the number of shares of Common Stock to be subject to each Option, the exercise price of each Option and the conditions and limitations applicable to the exercise of each Option, including conditions relating to applicable federal or state securities laws, as it considers necessary or advisable.

(b) Incentive Stock Options. An Option that the Board intends to be an "incentive stock option" as defined in Section 422 of the Code (an "Incentive Stock Option") shall only be granted to employees of Torus Therapeutics, Inc., any of Torus Therapeutics, Inc.'s present and future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Code, and any other entities the employees of which are eligible to receive Incentive Stock Options under the Code, and shall be subject to and shall be construed consistently with the requirements of Section 422 of the Code. An Option that is not intended to be an Incentive Stock Option shall be designated non-statutory stock option (a "Nonstatutory Stock Option"). The Company shall have no liability to a Participant, or any other person, if an Option (or any part thereof) that is intended to be an Incentive Stock Option is not an Incentive Stock Option or if the Company converts an Incentive Stock Option to a Nonstatutory Stock Option.

(c) Exercise Price. The Board shall establish the exercise price of each Option and specify the exercise price in the applicable Option agreement. The exercise price shall be not less than 100% of the Grant Date Fair Market Value (as defined below) of the Common Stock on the date the Option is granted; provided that if the Board approves the grant of an Option with an exercise price to be determined on a future date, the exercise price shall not be less than 100% of the Grant Date Fair Market Value on such future date. The "Grant Date Fair Market Value" of a share of Common Stock for purposes of the Plan will be determined as follows:

(1) if the Common Stock is not publicly traded, the Board will determine the Fair Market Value for purposes of the Plan using any measure of value it determines to be appropriate (including, as it considers appropriate, relying on appraisals) in a manner consistent with the valuation principles under Code Section 409A, except as the Board may expressly determine otherwise;

(2) if the Common Stock is listed on a national securities exchange, the closing sale price (for the primary trading session) on the date of grant; or

(3) if the Common Stock is not listed on any such exchange, the average of the closing bid and asked prices as reported by an authorized OTCBB market data vendor as listed on the OTCBB website (otcbb.com) on the date of grant.

For any date that is not a trading day, the Grant Date Fair Market Value of a share of Common Stock for such date will be determined by using the closing sale price or average of the bid and asked prices, as appropriate, for the immediately preceding trading day and with the timing in the formulas above adjusted accordingly. The Board can substitute a particular time of day or other measure of "closing sale price" or "bid and asked prices" if appropriate because of exchange or market procedures or can, in its discretion, use weighted averages either on a daily basis or such longer period as complies with Code Section 409A.

The Board has discretion to determine the Grant Date Fair Market Value for purposes of the Plan, and all Awards are conditioned on the applicable Participant's agreement that the Board's determination is conclusive and binding even though others might make a different determination.

(d) Duration of Options. Each Option shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable option agreement; *provided, however*, that no Option will be granted with a term in excess of 10 years.

(e) Exercise of Options. Options may be exercised by delivery to the Company of a notice of exercise in a form of notice (which may be electronic) approved by the Company, together with payment in full (in the manner specified in Section 5(f)) of the exercise price for the number of shares for which the Option is exercised. Shares of Common Stock subject to the Option will be delivered by the Company as soon as practicable following exercise.

(f) Payment Upon Exercise. Common Stock purchased upon the exercise of an Option granted under the Plan shall be paid for as follows:

(1) in cash or by check, payable to the order of the Company;

(2) when the Common Stock is registered under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), except as may otherwise be provided in the applicable Option agreement or approved by the Board, in its discretion, by (i) delivery of an irrevocable and unconditional undertaking by a creditworthy broker to deliver promptly to the Company sufficient funds to pay the exercise price and any required tax withholding or (ii) delivery by the Participant to the Company of a copy of irrevocable and unconditional instructions to a creditworthy broker to deliver promptly to the Company cash or a check sufficient to pay the exercise price and any required tax withholding;

(3) when the Common Stock is registered under the Exchange Act and to the extent provided for in the applicable Option agreement or approved by the Board, in its discretion, by delivery (either by actual delivery or attestation) of shares of Common Stock owned by the Participant valued at their fair market value (valued in the manner determined by (or in a manner approved by) the Board), *provided* (i) such method of payment is then permitted under applicable law, (ii) such Common Stock, if acquired directly from the Company, was owned by the Participant for such minimum period of time, if any, as may be established by the Board in its discretion and (iii) such Common Stock is not subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements;

(4) to the extent provided for in the applicable Nonstatutory Stock Option agreement or approved by the Board in its discretion, by delivery of a notice of "net exercise" to the Company, as a result of which the Participant would receive (i) the number of shares underlying the portion of the Option being exercised, less (ii) such number of shares as is equal to (A) the aggregate exercise price for the portion of the Option being exercised divided by (B) the fair market value of the Common Stock (valued in the manner determined by (or in a manner approved by) the Board) on the date of exercise;

(5) to the extent permitted by applicable law and provided for in the applicable Option agreement or approved by the Board, in its discretion, by (i) delivery of a promissory note of the Participant to the Company on terms determined by the Board, or (ii) payment of such other lawful consideration as the Board may determine; or

(6) by any combination of the above permitted forms of payment.

#### 6. Stock Appreciation Rights

(a) General. The Board may grant Awards consisting of stock appreciation rights ("SARs") entitling the Participant, upon exercise, to receive an amount of Common Stock or cash or a combination thereof (such form to be determined by the Board) determined by reference to appreciation, from and after the date of grant, in the fair market value of a share of Common Stock (valued in the manner determined by (or in a manner approved by) the Board) over the measurement price established pursuant to Section 6(b). The date as of which such appreciation is determined shall be the exercise date.

(b) Measurement Price. The Board shall establish the measurement price of each SAR and specify it in the applicable SAR agreement. The measurement price shall not be less than

100% of the Grant Date Fair Market Value of a share of Common Stock on the date the SAR is granted; *provided*, that if the Board approves the grant of an SAR effective as of a future date, the measurement price shall not be less than 100% of the Grant Date Fair Market Value on such future date.

(c) Duration of SARs. Each SAR shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable SAR agreement; *provided, however*, that no SAR will be granted with a term in excess of 10 years.

(d) Exercise of SARs. SARs may be exercised by delivery to the Company of a notice of exercise in a form (which may be electronic) approved by the Company, together with any other documents required by the Board.

#### 7. Restricted Stock; Restricted Stock Units

(a) General. The Board may grant Awards entitling Participants to acquire shares of Common Stock ("Restricted Stock"), subject to the right of the Company to repurchase all or part of such shares at their issue price or other stated or formula price (or to require forfeiture of such shares if issued at no cost) from the Participant in the event that conditions specified by the Board in the applicable Award are not satisfied prior to the end of the applicable restriction period or periods established by the Board for such Award. The Board may also grant Awards entitling the Participant to receive shares of Common Stock or cash to be delivered at the time such Award vests ("Restricted Stock Units") (Restricted Stock and Restricted Stock Units are each referred to herein as a "Restricted Stock Award").

(b) Terms and Conditions for All Restricted Stock Awards. The Board shall determine the terms and conditions of a Restricted Stock Award, including the conditions for vesting and repurchase (or forfeiture) and the issue price, if any.

(c) Additional Provisions Relating to Restricted Stock.

(1) Dividends. Unless otherwise provided in the applicable Award agreement, any dividends (whether paid in cash, stock or property) declared and paid by the Company with respect to shares of Restricted Stock ("Accrued Dividends") shall be paid to the Participant only if and when such shares become free from the restrictions on transferability and forfeitability that apply to such shares. Each payment of Accrued Dividends will be made no later than the end of the calendar year in which the dividends are paid to stockholders of that class of stock or, if later, the 15th day of the third month following the lapsing of the restrictions on transferability and the forfeitability provisions applicable to the underlying shares of Restricted Stock.

(2) Stock Certificates. The Company may require that any stock certificates issued in respect of shares of Restricted Stock, as well as dividends or distributions paid on such Restricted Stock, shall be deposited in escrow by the Participant, together with a stock power endorsed in blank, with the Company (or its designee). At the expiration of the applicable restriction periods, the Company (or such designee) shall deliver the certificates no longer subject to such restrictions to the Participant or if the Participant has died, to Participant's Designated Beneficiary. "Designated Beneficiary" means (i) the beneficiary designated, in a manner determined by the Board, by a Participant to receive amounts due or exercise rights of the Participant in the event of the Participant's death or (ii) in the absence of an effective designation by a Participant, "Designated Beneficiary" means the Participant's estate.

(d) Additional Provisions Relating to Restricted Stock Units.

(1) Settlement. Upon the vesting of and/or lapsing of any other restrictions (i.e., settlement) with respect to each Restricted Stock Unit, the Participant shall be entitled to receive from the Company the number of shares of Common Stock specified in the Award agreement or (if so provided in the applicable Award agreement or otherwise determined by the Board) an amount of cash equal to the fair market value (valued in the manner determined by (or in a manner approved by) the Board) of such number of shares of Common Stock or a combination thereof. The Board may, in its discretion, provide that settlement of Restricted Stock Units shall be deferred, on a mandatory basis or at the election of the Participant in a manner that complies with Section 409A of the Code.

(2) Voting Rights. A Participant shall have no voting rights with respect to any Restricted Stock Units.

(3) Dividend Equivalents. The Award agreement for Restricted Stock Units may provide Participants with the right to receive an amount equal to any dividends or other distributions declared and paid on an equal number of outstanding shares of Common Stock ("Dividend Equivalents"). Dividend Equivalents may be paid currently or credited to an account for the Participants, may be settled in cash and/or shares of Common Stock and may be subject to the same restrictions on transfer and forfeitability as the Restricted Stock Units with respect to which paid, in each case to the extent provided in the applicable Award agreement.

8. Other Stock-Based Awards

(a) General. The Board may grant other Awards of shares of Common Stock, and other Awards that are valued in whole or in part by reference to, or are otherwise based on, shares of Common Stock or other property ("Other Stock-Based Awards"). Such Other Stock-Based Awards shall also be available as a form of payment in the settlement of other Awards granted under the Plan or as payment in lieu of compensation to which a Participant is otherwise entitled. Other Stock-Based Awards may be paid in shares of Common Stock or cash, as the Board shall determine.

(b) Terms and Conditions. Subject to the provisions of the Plan, the Board shall determine the terms and conditions of each Other Stock-Based Award, including any purchase price applicable thereto.

9. Adjustments for Changes in Common Stock and Certain Other Events

(a) Changes in Capitalization. In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Common Stock other than an ordinary cash dividend, (i) the number and class of securities available under the Plan, (ii) the number and class of securities and exercise price per share of each outstanding Option, (iii) the share and per-share provisions and the measurement price of each outstanding



SAR, (iv) the number of shares subject to and the repurchase price per share subject to each outstanding Award of Restricted Stock and (v) the share and per-share-related provisions and the purchase price, if any, of each outstanding Award of Restricted Stock Unit and each outstanding Other Stock-Based Award, shall be equitably adjusted by the Company (or substituted Awards may be made, if applicable) in the manner determined by the Board. Without limiting the generality of the foregoing, in the event the Company effects a split of the Common Stock by means of a stock dividend and the exercise price of and the number of shares subject to an outstanding Option are adjusted as of the date of the distribution of the dividend (rather than as of the record date for such dividend), then an optionee who exercises an Option between the record date and the distribution date for such stock dividend shall be entitled to receive, on the distribution date, the stock dividend with respect to the shares of Common Stock acquired upon such Option exercise, notwithstanding the fact that such shares were not outstanding as of the close of business on the record date for such stock dividend.

(b) Reorganization Events.

(1) Definition. A “Reorganization Event” shall mean: (a) any merger or consolidation of the Company with or into another entity as a result of which all of the Common Stock of the Company is converted into or exchanged for the right to receive cash, securities or other property or is cancelled, (b) any transfer or disposition of all of the Common Stock of the Company for cash, securities or other property pursuant to a share exchange or other transaction or (c) any liquidation or dissolution of the Company.

(2) Consequences of a Reorganization Event on Awards Other than Restricted Stock.

(i) In connection with a Reorganization Event, the Board may take any one or more of the following actions as to all or any (or any portion of) outstanding Awards other than Restricted Stock on such terms as the Board determines (except to the extent specifically provided otherwise in an applicable Award agreement or another agreement between the Company and the Participant): (i) provide that such Awards shall be assumed, or substantially equivalent Awards shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof), (ii) upon written notice to a Participant, provide that all of the Participant’s unexercised and/or unvested Awards will terminate immediately prior to the consummation of such Reorganization Event unless exercised by the Participant (to the extent then exercisable) within a specified period following the date of such notice, (iii) provide that outstanding Awards shall become exercisable, realizable, or deliverable, or restrictions applicable to an Award shall lapse, in whole or in part prior to or upon such Reorganization Event, (iv) in the event of a Reorganization Event under the terms of which holders of Common Stock will receive upon consummation thereof a cash payment for each share surrendered in the Reorganization Event (the “Acquisition Price”), make or provide for a cash payment to Participants with respect to each Award held by a Participant equal to (A) the number of shares of Common Stock subject to the vested portion of the Award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such Reorganization Event) multiplied by (B) the excess, if any, of (I) the Acquisition Price over (II) the exercise, measurement or purchase price of such Award and any applicable tax withholdings, in exchange for the termination of such Award, (v) provide that, in connection with a liquidation or dissolution of the Company, Awards shall convert into the right to receive liquidation proceeds (if applicable,

net of the exercise, measurement or purchase price thereof and any applicable tax withholdings) and (vi) any combination of the foregoing. In taking any of the actions permitted under this Section 9(b)(2), the Board shall not be obligated by the Plan to treat all Awards, all Awards held by a Participant, or all Awards of the same type, identically.

(ii) Notwithstanding the terms of Section 9(b)(2)(i), in the case of outstanding Restricted Stock Units that are subject to Section 409A of the Code: (i) if the applicable Restricted Stock Unit agreement provides that the Restricted Stock Units shall be settled upon a “change in control event” within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i), and the Reorganization Event constitutes such a “change in control event”, then no assumption or substitution shall be permitted pursuant to Section 9(b)(2)(i) and the Restricted Stock Units shall instead be settled in accordance with the terms of the applicable Restricted Stock Unit agreement; and (ii) the Board may only undertake the actions set forth in clauses (iii), (iv) or (v) of Section 9(b)(2)(i) if the Reorganization Event constitutes a “change in control event” as defined under Treasury Regulation Section 1.409A-3(i)(5)(i) and such action is permitted or required by Section 409A of the Code; if the Reorganization Event is not a “change in control event” as so defined or such action is not permitted or required by Section 409A of the Code, and the acquiring or succeeding corporation does not assume or substitute the Restricted Stock Units pursuant to clause (i) of Section 9(b)(2)(i), then the unvested Restricted Stock Units shall terminate immediately prior to the consummation of the Reorganization Event without any payment in exchange therefor.

(iii) For purposes of Section 9(b)(2)(i), an Award (other than Restricted Stock) shall be considered assumed if, following consummation of the Reorganization Event, such Award confers the right to purchase or receive pursuant to the terms of such Award, for each share of Common Stock subject to the Award immediately prior to the consummation of the Reorganization Event, the consideration (whether cash, securities or other property) received as a result of the Reorganization Event by holders of Common Stock for each share of Common Stock held immediately prior to the consummation of the Reorganization Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding shares of Common Stock); *provided, however*, that if the consideration received as a result of the Reorganization Event is not solely common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise or settlement of the Award to consist solely of such number of shares of common stock of the acquiring or succeeding corporation (or an affiliate thereof) that the Board determined to be equivalent in value (as of the date of such determination or another date specified by the Board) to the per share consideration received by holders of outstanding shares of Common Stock as a result of the Reorganization Event.

(3) Consequences of a Reorganization Event on Restricted Stock. Upon the occurrence of a Reorganization Event other than a liquidation or dissolution of the Company, the repurchase and other rights of the Company with respect to outstanding Restricted Stock shall inure to the benefit of the Company’s successor and shall, unless the Board determines otherwise, apply to the cash, securities or other property which the Common Stock was converted into or exchanged for pursuant to such Reorganization Event in the same manner and to the same extent as they applied to such Restricted Stock; *provided, however*, that the Board may provide for

termination or deemed satisfaction of such repurchase or other rights under the instrument evidencing any Restricted Stock or any other agreement between a Participant and the Company, either initially or by amendment, or provide for forfeiture of such Restricted Stock if issued at no cost. Upon the occurrence of a Reorganization Event involving the liquidation or dissolution of the Company, except to the extent specifically provided to the contrary in the instrument evidencing any Restricted Stock or any other agreement between a Participant and the Company, all restrictions and conditions on all Restricted Stock then outstanding shall automatically be deemed terminated or satisfied.

10. General Provisions Applicable to Awards.

(a) Transferability of Awards. Awards (or any interest in an Award, including, prior to exercise, any interest in shares of Common Stock issuable upon exercise of an Option or SAR) shall not be sold, assigned, transferred (including by establishing any short position, put equivalent position (as defined in Rule 16a-1 issued under the Exchange Act) or call equivalent position (as defined in Rule 16a-1 issued under the Exchange Act)), pledged, hypothecated or otherwise encumbered by the person to whom they are granted, either voluntarily or by operation of law, and, during the life of the Participant, shall be exercisable only by the Participant; except that Awards, other than Awards subject to Section 409A of the Code, may be transferred to family members (as defined in Rule 701(c)(3) under the Securities Act) through gifts or (other than Incentive Stock Options) domestic relations orders or to an executor or guardian upon the death or disability of the Participant. The Company shall not be required to recognize any such permitted transfer until such time as such permitted transferee shall deliver to the Company a written instrument, as a condition to such transfer, in form and substance satisfactory to the Company confirming that such transferee shall be bound by all of the terms and conditions of the Award. References to a Participant, to the extent relevant in the context, shall include references to authorized transferees. For the avoidance of doubt, nothing contained in this Section 10(a) shall be deemed to restrict a transfer to the Company.

(b) Documentation. Each Award shall be evidenced in such form (written, electronic or otherwise) as the Board shall determine. Each Award may contain terms and conditions in addition to those set forth in the Plan.

(c) Board Discretion. Except as otherwise provided by the Plan, each Award may be made alone or in addition or in relation to any other Award. The terms of each Award need not be identical, and the Board need not treat Participants uniformly.

(d) Termination of Status. The Board shall determine the effect on an Award of the disability, death, termination or other cessation of employment, authorized leave of absence or other change in the employment or other status of a Participant and the extent to which, and the period during which, the Participant, or the Participant's legal representative, conservator, guardian or Designated Beneficiary, may exercise rights under the Award.

(e) Withholding. The Participant must satisfy all applicable federal, state, and local or other income and employment tax withholding obligations before the Company will deliver stock certificates or otherwise recognize ownership of Common Stock under an Award. The Company may elect to satisfy the withholding obligations through additional withholding on salary or wages.

If the Company elects not to or cannot withhold from other compensation, the Participant must pay the Company the full amount, if any, required for withholding or have a broker tender to the Company cash equal to the withholding obligations. Payment of withholding obligations is due before the Company will issue any shares on exercise, vesting or release from forfeiture of an Award or at the same time as payment of the exercise or purchase price unless the Company determines otherwise. If provided for in an Award or approved by the Board in its discretion, a Participant may satisfy such tax obligations in whole or in part by delivery (either by actual delivery or attestation) of shares of Common Stock, including shares retained from the Award creating the tax obligation, valued at their fair market value (valued in the manner determined by (or in a manner approved by) the Company); *provided, however*, except as otherwise provided by the Board, that the total tax withholding where stock is being used to satisfy such tax obligations cannot exceed the Company's minimum statutory withholding obligations (based on minimum statutory withholding rates for federal and state tax purposes, including payroll taxes, that are applicable to such supplemental taxable income), *except that*, to the extent that the Company is able to retain shares of Common Stock having a fair market value (valued in the manner determined by (or in a manner approved by) the Company) that exceeds the statutory minimum applicable withholding tax without financial accounting implications or the Company is withholding in a jurisdiction that does not have a statutory minimum withholding tax, the Company may retain such number of shares of Common Stock (up to the number of shares having a fair market value (valued in the manner determined by (or in a manner approved by) the Company) equal to the maximum individual statutory rate of tax) as the Company shall determine in its discretion to satisfy the tax liability associated with any Award. Shares used to satisfy tax withholding requirements cannot be subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements.

(f) Amendment of Award.

(1) The Board may amend, modify or terminate any outstanding Award, including but not limited to, substituting therefor another Award of the same or a different type, changing the date of exercise or realization, and converting an Incentive Stock Option to a Nonstatutory Stock Option. The Participant's consent to such action shall be required unless (i) the Board determines that the action, taking into account any related action, does not materially and adversely affect the Participant's rights under the Plan or (ii) the change is permitted under Section 9.

(2) The Board may, without stockholder approval, amend any outstanding Award granted under the Plan to provide an exercise price per share that is lower than the then-current exercise price per share of such outstanding Award. The Board may also, without stockholder approval, cancel any outstanding award (whether or not granted under the Plan) and grant in substitution therefor new Awards under the Plan covering the same or a different number of shares of Common Stock and having an exercise price per share lower than the then-current exercise price per share of the cancelled award.

(g) Conditions on Delivery of Stock. The Company will not be obligated to deliver any shares of Common Stock pursuant to the Plan or to remove restrictions from shares previously issued or delivered under the Plan until (i) all conditions of the Award have been met or removed to the satisfaction of the Company, (ii) in the opinion of the Company's counsel, all other legal

matters in connection with the issuance and delivery of such shares have been satisfied, including any applicable securities laws and regulations and any applicable stock exchange or stock market rules and regulations, and (iii) the Participant has executed and delivered to the Company such representations or agreements as the Company may consider appropriate to satisfy the requirements of any applicable laws, rules or regulations.

(h) Acceleration. The Board may at any time provide that any Award shall become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

11. Miscellaneous.

(a) No Right To Employment or Other Status. No person shall have any claim or right to be granted an Award by virtue of the adoption of the Plan, and the grant of an Award shall not be construed as giving a Participant the right to continued employment or any other relationship with the Company. The Company expressly reserves the right at any time to dismiss or otherwise terminate its relationship with a Participant free from any liability or claim under the Plan, except as expressly provided in the applicable Award.

(b) No Rights As Stockholder. Subject to the provisions of the applicable Award, no Participant or Designated Beneficiary shall have any rights as a stockholder with respect to any shares of Common Stock to be distributed with respect to an Award until becoming the record holder of such shares.

(c) Effective Date and Term of Plan. The Plan shall become effective on the date on which it is adopted by the Board. No Awards shall be granted under the Plan after the expiration of 10 years from the earlier of (i) the date on which the Plan was adopted by the Board or (ii) the date the Plan was approved by the Company's stockholders, but Awards previously granted may extend beyond that date.

(d) Amendment of Plan. The Board may amend, suspend or terminate the Plan or any portion thereof at any time; *provided* that if at any time the approval of the Company's stockholders is required as to any modification or amendment under Section 422 of the Code or any successor provision with respect to Incentive Stock Options, the Board may not effect such modification or amendment without such approval. Unless otherwise specified in the amendment, any amendment to the Plan adopted in accordance with this Section 11(d) shall apply to, and be binding on the holders of, all Awards outstanding under the Plan at the time the amendment is adopted, provided the Board determines that such amendment, taking into account any related action, does not materially and adversely affect the rights of Participants under the Plan.

(e) Authorization of Sub-Plans (including Grants to non-U.S. Employees). The Board may from time to time establish one or more sub-plans under the Plan for purposes of satisfying applicable securities, tax or other laws of various jurisdictions. The Board shall establish such sub-plans by adopting supplements to the Plan containing (i) such limitations on the Board's discretion under the Plan as the Board deems necessary or desirable or (ii) such additional terms and conditions not otherwise inconsistent with the Plan as the Board shall deem necessary or desirable. All supplements adopted by the Board shall be deemed to be part of the Plan, but each supplement shall apply only to Participants within the affected jurisdiction and the Company shall not be required to provide copies of any supplement to Participants in any jurisdiction which is not the subject of such supplement.

(f) Compliance with Section 409A of the Code. If and to the extent (i) any portion of any payment, compensation or other benefit provided to a Participant pursuant to the Plan in connection with Participant's employment termination constitutes "nonqualified deferred compensation" within the meaning of Section 409A of the Code and (ii) the Participant is a specified employee as defined in Section 409A(a)(2)(B)(i) of the Code, in each case as determined by the Company in accordance with its procedures, by which determinations the Participant (through accepting the Award) agrees that the Participant is bound, such portion of the payment, compensation or other benefit shall not be paid before the day that is six months plus one day after the date of "separation from service" (as determined under Section 409A of the Code) (the "New Payment Date"), except as Section 409A of the Code may then permit. The aggregate of any payments that otherwise would have been paid to the Participant during the period between the date of separation from service and the New Payment Date shall be paid to the Participant in a lump sum on such New Payment Date, and any remaining payments will be paid on their original schedule.

The Company makes no representations or warranty and shall have no liability to the Participant or any other person if any provisions of or payments, compensation or other benefits under the Plan are determined to constitute nonqualified deferred compensation subject to Section 409A of the Code but do not to satisfy the conditions of that section.

(g) Limitations on Liability. Notwithstanding any other provisions of the Plan, no individual acting as a director, officer, other employee, or agent of the Company will be liable to any Participant, former Participant, spouse, beneficiary, or any other person for any claim, loss, liability, or expense incurred in connection with the Plan, nor will such individual be personally liable with respect to the Plan because of any contract or other instrument such individual executes in such individual's capacity as a director, officer, other employee, or agent of the Company. The Company will indemnify and hold harmless each director, officer, other employee, or agent of the Company to whom any duty or power relating to the administration or interpretation of the Plan has been or will be delegated, against any cost or expense (including attorneys' fees) or liability (including any sum paid in settlement of a claim with the Board's approval) arising out of any act or omission to act concerning the Plan unless arising out of such person's own fraud or bad faith.

(h) Governing Law. The provisions of the Plan and all Awards made hereunder shall be governed by and interpreted in accordance with the laws of the State of Delaware, excluding choice-of-law principles of the law of such state that would require the application of the laws of a jurisdiction other than the State of Delaware.

\* \* \* \*

**TORUS THERAPEUTICS, INC.,  
2017 STOCK INCENTIVE PLAN**

**CALIFORNIA SUPPLEMENT**

Pursuant to Section 11(e) of the Plan, the Board has adopted this supplement for purposes of satisfying the requirements of Section 25102(o) of the California Law:

Any Awards granted under the Plan to a Participant who is a resident of the State of California on the date of grant (a "California Participant") shall be subject to the following additional limitations, terms and conditions:

1. Additional Limitations on Options.

(a) Maximum Duration of Options. No Options granted to California Participants shall have a term in excess of 10 years measured from the Option grant date.

(b) Minimum Exercise Period Following Termination. Unless a California Participant's employment is terminated for cause (as defined by applicable law, the terms of the Plan or option grant or a contract of employment), in the event of termination of employment of such Participant, such Participant shall have the right to exercise an Option, to the extent that such Participant is entitled to exercise such Option on the date employment terminated, until the earlier of: (i) at least six months from the date of termination, if termination was caused by such Participant's death or disability, (ii) at least 30 days from the date of termination, if termination was caused other than by such Participant's death or disability and (iii) the Option expiration date.

2. Additional Limitations for Other Stock-Based Awards. The terms of all Awards granted to a California Participant under Section 8 of the Plan shall comply, to the extent applicable, with Section 260.140.46 of the California Code of Regulations.

3. Additional Limitations on Timing of Awards. No Award granted to a California Participant shall become exercisable, vested or realizable, as applicable to such Award, unless the Plan has been approved by the holders of a majority of the Company's outstanding voting securities by the later of (i) within 12 months before or after the date the Plan was adopted by the Board, or (ii) prior to or within 12 months of the granting of any Award to a California Participant.

4. Additional Restriction Regarding Recapitalizations, Stock Splits, Etc. For purposes of Section 9 of the Plan, in the event of a stock split, reverse stock split, stock dividend, recapitalization, combination, reclassification or other distribution of the Company's securities underlying the Award without the receipt of consideration by the Company, the number of securities purchasable, and in the case of Options, the exercise price of such Options, shall be proportionately adjusted.

5. Additional Limitations on Transferability of Awards. Notwithstanding the provisions of Section 10(a) of the Plan, an Award granted to a California Participant may not be transferred to an executor or guardian upon the disability of the Participant.

\* \* \* \*

**Torus Therapeutics, Inc.**

Amendment No. 1  
to  
2017 Stock Incentive Plan

The 2017 Stock Incentive Plan (the "Plan") of Torus Therapeutics, is hereby amended as follows (capitalized terms used herein and not defined herein shall have the respective meaning ascribed to such terms in the Plan):

Section 4(a) shall be amended and restated in its entirety to read as follows:

“4.1 Stock Available For Awards

Subject to adjustment under Section 9, Awards may be made under the Plan for up to 8,000,000 shares of common stock, \$0.0001 par value per share, of the Company (the "Common Stock"), any or all of which Awards may be in the form of Incentive Stock Options (as defined in Section 5(b)).

Except as aforesaid, the Plan shall remain in full force and effect.

Adopted by the Board of Directors of the Company on  
October 17, 2017 and effective as the filing of the  
Certificate of Amendment of the Certificate of  
Incorporation of the Company on October 18, 2017.



**Generation Bio Co.**

Amendment No. 2  
to  
2017 Stock Incentive Plan

The 2017 Stock Incentive Plan (the "Plan") of Generation Bio Co. is hereby amended as follows (capitalized terms used herein and not defined herein shall have the respective meaning ascribed to such terms in the Plan):

Section 4(a) shall be amended and restated in its entirety to read as follows:

**“4.1 Stock Available For Awards**

Subject to adjustment under Section 9, Awards may be made under the Plan for up to 9,600,000 shares of common stock, \$0.0001 par value per share, of the Company (the "Common Stock"), any or all of which Awards may be in the form of Incentive Stock Options (as defined in Section 5(b)).

Except as aforesaid, the Plan shall remain in full force and effect.

Adopted by the Board of Directors of the Company on  
November 17, 2017 and effective as the filing of the  
Amended and Restated Certificate of Incorporation of the  
Company on November 17, 2017.

**Generation Bio Co.**

Amendment No. 3  
to  
2017 Stock Incentive Plan

The 2017 Stock Incentive Plan (the "Plan") of Generation Bio Co. is hereby amended as follows (capitalized terms used herein and not defined herein shall have the respective meaning ascribed to such terms in the Plan):

Section 4(a) shall be amended and restated in its entirety to read as follows:

"4.1 Stock Available For Awards

Subject to adjustment under Section 9, Awards may be made under the Plan for up to 13,100,000 shares of common stock, \$0.0001 par value per share, of the Company (the "Common Stock"), any or all of which Awards may be in the form of Incentive Stock Options (as defined in Section 5(b)).

Except as aforesaid, the Plan shall remain in full force and effect.

Adopted by the Board of Directors of the Company on  
February 16, 2018 and effective as the filing of the  
Amended and Restated Certificate of Incorporation of the  
Company on February 21, 2018.

**Generation Bio Co.**

Amendment No. 4  
to  
2017 Stock Incentive Plan

The 2017 Stock Incentive Plan (the "Plan") of Generation Bio Co. is hereby amended as follows (capitalized terms used herein and not defined herein shall have the respective meaning ascribed to such terms in the Plan):

The first sentence of Section 4(a) shall be amended and restated in its entirety to read as follows:

"Subject to adjustment under Section 9, Awards may be made under the Plan for up to 14,850,000 shares of common stock, \$0.0001 par value per share, of the Company (the "Common Stock"), any or all of which Awards may be in the form of Incentive Stock Options (as defined in Section 5(b))."

Except as aforesaid, the Plan shall remain in full force and effect.

Adopted by the Board of Directors of the Company on  
December 6, 2018 and effective as the filing of the  
Certificate of Amendment of the Amended and Restated  
Certificate of Incorporation of the Company on  
December 17, 2018.

**Generation Bio Co.**

Amendment No. 5  
to  
2017 Stock Incentive Plan

The 2017 Stock Incentive Plan (the "Plan") of Generation Bio Co. is hereby amended as follows (capitalized terms used herein and not defined herein shall have the respective meaning ascribed to such terms in the Plan):

The first sentence of Section 4(a) shall be amended and restated in its entirety to read as follows:

"Subject to adjustment under Section 9, Awards may be made under the Plan for up to 18,150,000 shares of common stock, \$0.0001 par value per share, of the Company (the "Common Stock"), any or all of which Awards may be in the form of Incentive Stock Options (as defined in Section 5(b))."

Except as aforesaid, the Plan shall remain in full force and effect.

Adopted by the Board of Directors of the Company on  
January 8, 2020 and effective as of the filing of the  
Certificate of Amendment of the Amended and Restated  
Certificate of Incorporation of the Company on January 9, 2020

## GENERATION BIO CO.

**STOCK OPTION AGREEMENT  
GRANTED UNDER 2017 STOCK INCENTIVE PLAN**

This Stock Option Agreement (this "Agreement") is made between Generation Bio Co., a Delaware corporation (the "Company"), and the Participant pursuant to the 2017 Stock Incentive Plan (the "Plan").

**NOTICE OF GRANT**

**I. Participant Information**

|                      |         |
|----------------------|---------|
| Participant:         | [_____] |
| Participant Address: | [_____] |

**II. Grant Information**

|                            |  |
|----------------------------|--|
| Grant Date:                | [_____]  |
| Number of Shares:          | [_____]  |
| Exercise Price Per Share:  | [_____]  |
| Vesting Commencement Date: | [_____]  |
| Type of Option:            | [Incentive Stock Option] [Nonstatutory Stock Option] |

**III. Vesting Table**

| Vesting Date  | Shares that Vest <sup>(1)</sup> |
|---|---------------------------------|
| [_____] anniversary of the Vesting Commencement Date  | [# of shares]                   |
| End of each successive [_____] month period following the [_____] anniversary of the Vesting Commencement Date until the [_____] anniversary of the Vesting Commencement Date | [# of Shares]                   |

<sup>(1)</sup> The number of shares is subject to adjustment for any changes in the Company's capitalization as set forth in Section 9 of the Plan.

**IV. Final Exercise Date**

|                               |   |
|-------------------------------|---|
| 5:00 pm Eastern time on Date: | [Date is ten years minus one day from Grant Date] |
|-------------------------------|---|

This Agreement includes this Notice of Grant and the following Exhibits, which are expressly incorporated by reference in their entirety herein:

- Exhibit A – General Terms and Conditions
- Exhibit B – Notice of Stock Option Exercise
- Exhibit C – Torus Therapeutics, Inc. 2017 Stock Incentive Plan

IN WITNESS WHEREOF, the parties hereto have executed this Agreement.

**GENERATION BIO CO.**

**PARTICIPANT**

**SPOUSAL CONSENT<sup>1</sup>**

Name:

Name:

Name:

Title:

<sup>1</sup> If the Participant resides in a community property state, it is desirable to have the Participant's spouse also accept the option. The following are community property states: Arizona, California, Idaho, Louisiana, Nevada, New Mexico, Texas, and Washington. Although Wisconsin is not formally a community property state, it has laws governing the division of marital property similar to community property states and it may be desirable to have a Wisconsin Participant's spouse accept the option.

EXHIBIT A

GENERAL TERMS AND CONDITIONS

For valuable consideration, receipt of which is acknowledged, the parties hereto agree as follows:

1. Grant of Option. This Agreement evidences the grant by the Company, on the grant date (the “**Grant Date**”) set forth in the Notice of Grant that forms part of this Agreement (the “**Notice of Grant**”), to the Participant of an option to purchase, in whole or in part, on the terms provided herein and in the Company’s 2017 Stock Incentive Plan (the “**Plan**”), the number of shares set forth in the Notice of Grant (the “**Shares**”) of common stock, \$0.0001 par value per share, of the Company (“**Common Stock**”) at the exercise price per Share set forth in the Notice of Grant (the “**Exercise Price**”). Unless earlier terminated, this option shall expire at the time and on the date set forth in the Notice of Grant (the “**Final Exercise Date**”).

It is intended that the option evidenced by this Agreement shall be an incentive stock option as defined in Section 422 of the Internal Revenue Code of 1986, as amended, and any regulations promulgated thereunder (the “**Code**”) solely to the extent set forth in the Notice of Grant. To the extent not designated as an incentive stock option, or to the extent that the option does not qualify as an incentive stock option, the option shall be a nonstatutory stock option. Except as otherwise indicated by the context, the term “Participant”, as used in this option, shall be deemed to include any person who acquires the right to exercise this option validly under its terms.

2. Vesting Schedule.

This option will become exercisable (“**vest**”) in accordance with the Vesting Table set forth in the Notice of Grant.

The right of exercise shall be cumulative so that to the extent the option is not exercised in any period to the maximum extent permissible it shall continue to be exercisable, in whole or in part, with respect to all Shares for which it is vested until the earlier of the Final Exercise Date or the termination of this option under Section 3 hereof or the Plan.

3. Exercise of Option.

(a) Form of Exercise. Each election to exercise this option shall be accompanied by a completed Notice of Stock Option Exercise in the form attached hereto as Exhibit B, signed by the Participant, and received by the Company at its principal office, accompanied by this Agreement, and payment in full in the manner provided in the Plan. The Participant may purchase less than the number of Shares covered hereby, provided that no partial exercise of this option may be for any fractional share or for fewer than ten whole shares (unless the number of Shares that remain subject to this option at the time of exercise is less than ten whole shares, in which case the Participant may purchase the total number of whole shares that remain subject to this option).

(b) Continuous Relationship with the Company Required. Except as otherwise provided in this Section 3, this option may not be exercised unless the Participant, at the time he or she exercises this option, is, and has been at all times since the Grant Date, an employee, officer or director of, or consultant or advisor to, the Company or any parent or subsidiary of the Company as defined in Section 424(e) or (f) of the Code or any other entity the employees, officers, directors, consultants, or advisors of which are eligible to receive option grants under the Plan (an “**Eligible Participant**”).

(c) Termination of Relationship with the Company. If the Participant ceases to be an Eligible Participant for any reason, then, except as provided in paragraphs (d) and (e) below, the right to exercise this option shall terminate three months after such cessation (but in no event after the Final Exercise Date), provided that this option shall be exercisable only to the extent that the Participant was entitled to exercise this option on the date of such cessation. Notwithstanding the foregoing, if the Participant, prior to the Final Exercise Date, violates the non-competition or confidentiality provisions of any employment contract, confidentiality and nondisclosure agreement or other agreement between the Participant and the Company, the right to exercise this option shall terminate immediately upon such violation.

(d) Exercise Period Upon Death or Disability. If the Participant dies or becomes disabled (within the meaning of Section 22(e)(3) of the Code) prior to the Final Exercise Date while he or she is an Eligible Participant and the Company has not terminated such service relationship for “**cause**” as specified in paragraph (e) below, this option shall be exercisable, within the period of one year following the date of death or disability of the Participant, by the Participant (or in the case of death by an authorized transferee), provided that this option shall be exercisable only to the extent that this option was exercisable by the Participant on the date of his or her death or disability, and further provided that this option shall not be exercisable after the Final Exercise Date.

(e) Termination for Cause. If, prior to the Final Exercise Date, the Participant’s service relationship with the Company is terminated by the Company for Cause (as defined below), the right to exercise this option shall terminate immediately upon the effective date of such termination. If, prior to the Final Exercise Date, the Participant is given notice by the Company of the termination of his or her service relationship by the Company for Cause, and the effective date of such termination is subsequent to the date of the delivery of such notice, the right to exercise this option shall be suspended from the time of the delivery of such notice until the earlier of (i) such time as it is determined or otherwise agreed that the Participant’s service relationship shall not be terminated for Cause as provided in such notice or (ii) the effective date of such termination (in which case the right to exercise this option shall, pursuant to the preceding sentence, terminate immediately upon the effective date of such termination). If the Participant is party to an employment, consulting or severance agreement with the Company or subject to a severance plan maintained by the Company, in either case, that contains a definition of “cause” for termination of service, “Cause” shall have the meaning ascribed to such term in such agreement or plan. Otherwise, “Cause” shall mean willful misconduct by the Participant or willful failure by the Participant to perform his or her responsibilities to the Company (including, without limitation, breach by the Participant of any provision of any employment, consulting, advisory, nondisclosure, non-competition or other similar agreement between the Participant and



the Company), as determined by the Company, which determination shall be conclusive. The Participant's service relationship shall be considered to have been terminated for "Cause" if the Company determines, within 30 days after the Participant's termination of service, that termination for Cause was warranted.

4. Company Right of First Refusal.

(a) Notice of Proposed Transfer. If the Participant proposes to sell, assign, transfer, pledge, hypothecate or otherwise dispose of, by operation of law or otherwise (collectively, "**transfer**") any Shares acquired upon exercise of this option, then the Participant shall first give written notice of the proposed transfer (the "**Transfer Notice**") to the Company. The Transfer Notice shall name the proposed transferee and state the number of such Shares the Participant proposes to transfer (the "**Offered Shares**"), the price per share and all other material terms and conditions of the transfer.

(b) Company Right to Purchase. For 30 days following its receipt of such Transfer Notice, the Company shall have the option to purchase all or part of the Offered Shares at the price and upon the terms set forth in the Transfer Notice. In the event the Company elects to purchase all or part of the Offered Shares, it shall give written notice of such election to the Participant within such 30-day period. Within 10 days after his or her receipt of such notice, the Participant shall tender to the Company at its principal offices the certificate or certificates representing the Offered Shares to be purchased by the Company, duly endorsed in blank by the Participant or with duly endorsed stock powers attached thereto, all in a form suitable for transfer of the Offered Shares to the Company. Promptly following receipt of such certificate or certificates, the Company shall deliver or mail to the Participant a check in payment of the purchase price for such Offered Shares; provided that if the terms of payment set forth in the Transfer Notice were other than cash against delivery, the Company may pay for the Offered Shares on the same terms and conditions as were set forth in the Transfer Notice; and provided further that any delay in making such payment shall not invalidate the Company's exercise of its option to purchase the Offered Shares.

(c) Shares Not Purchased By Company. If the Company does not elect to acquire all of the Offered Shares, the Participant may, within the 30-day period following the expiration of the option granted to the Company under subsection (b) above, transfer the Offered Shares which the Company has not elected to acquire to the proposed transferee, provided that such transfer shall not be on terms and conditions more favorable to the transferee than those contained in the Transfer Notice. Notwithstanding any of the above, all Offered Shares transferred pursuant to this Section 4 shall remain subject to the right of first refusal set forth in this Section 4 and such transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Section 4.

(d) Consequences of Non-Delivery. After the time at which the Offered Shares are required to be delivered to the Company for transfer to the Company pursuant to subsection (b) above, the Company shall not pay any dividend to the Participant on account of such Offered Shares or permit the Participant to exercise any of the privileges or rights of a stockholder with respect to such Offered Shares, but shall, insofar as permitted by law, treat the Company as the owner of such Offered Shares.

(e) Exempt Transactions. The following transactions shall be exempt from the provisions of this Section 4:

(1) any transfer of Shares to or for the benefit of any spouse, child or grandchild of the Participant, or to a trust for their benefit;

(2) any transfer pursuant to an effective registration statement filed by the Company under the Securities Act of 1933, as amended (the “**Securities Act**”); and

(3) the sale of all or substantially all of the outstanding shares of capital stock of the Company (including pursuant to a merger or consolidation);

provided, however, that in the case of a transfer pursuant to clause (1) above, such Shares shall remain subject to the right of first refusal set forth in this Section 4.

(f) Assignment of Company Right. The Company may assign its rights to purchase Offered Shares in any particular transaction under this Section 4 to one or more persons or entities.

(g) Termination. The provisions of this Section 4 shall terminate upon the earlier of the following events:

(1) the closing of the sale of shares of Common Stock in an underwritten public offering pursuant to an effective registration statement filed by the Company under the Securities Act; or

(2) the sale of all or substantially all of the outstanding shares of capital stock, assets or business of the Company, by merger, consolidation, sale of assets or otherwise (other than a merger or consolidation in which all or substantially all of the individuals and entities who were beneficial owners of the Company’s voting securities immediately prior to such transaction beneficially own, directly or indirectly, more than 50% (determined on an as-converted basis) of the outstanding securities entitled to vote generally in the election of directors of the resulting, surviving or acquiring corporation in such transaction).

(h) No Obligation to Recognize Invalid Transfer. The Company shall not be required (1) to transfer on its books any of the Shares which shall have been sold or transferred in violation of any of the provisions set forth in this Section 4, or (2) to treat as owner of such Shares or to pay dividends to any transferee to whom any such Shares shall have been so sold or transferred.

(i) Legends. The certificate representing Shares shall bear a legend substantially in the following form (in addition to, or in combination with, any legend required by applicable federal and state securities laws and agreements relating to the transfer of the Company securities):

“The shares represented by this certificate are subject to a right of first refusal in favor of the Company, as provided in a certain stock option agreement with the Company.”

5. Agreement in Connection with Initial Public Offering.

The Participant agrees, in connection with the initial underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (i) not to (a) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any other securities of the Company or (b) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of shares of Common Stock or other securities of the Company, whether any transaction described in clause (a) or (b) is to be settled by delivery of securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days after the date of the final prospectus relating to the offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address NASD Rule 2711(f)(4) or NYSE Rule 472(f)(4) or any similar successor provision), and (ii) to execute any agreement reflecting clause (i) above as may be requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of the “lock-up” period.

6. Tax Matters.

(a) Withholding. No Shares will be issued pursuant to the exercise of this option unless and until the Participant pays to the Company, or makes provision satisfactory to the Company for payment of, any federal, state or local withholding taxes required by law to be withheld in respect of this option.

(b) Disqualifying Disposition. If this option satisfies the requirements to be treated as an incentive stock option under the Code and the Participant disposes of Shares acquired upon exercise of this option within two years from the Grant Date or one year after such Shares were acquired pursuant to exercise of this option, the Participant shall notify the Company in writing of such disposition.

7. Transfer Restrictions.

(a) This option may not be sold, assigned, transferred, pledged or otherwise encumbered by the Participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution, and, during the lifetime of the Participant, this option shall be exercisable only by the Participant.

(b) The Participant agrees that he or she will not transfer any Shares issued pursuant to the exercise of this option unless the transferee, as a condition to such transfer, delivers to the Company a written instrument confirming that such transferee shall be bound by all of the terms

and conditions of Section 4 and Section 5; provided that such a written confirmation shall not be required with respect to (1) Section 4 after such provision has terminated in accordance with Section 4(g) or (2) Section 5 after the completion of the lock-up period in connection with the Company's initial underwritten public offering.

8. Provisions of the Plan.

This option is subject to the provisions of the Plan (including the provisions relating to amendments to the Plan), a copy of which is attached hereto as Exhibit C.

[Remainder of Page Intentionally Left Blank]

EXHIBIT B

NOTICE OF STOCK OPTION EXERCISE

[DATE]<sup>2</sup>

Generation Bio Co.  
301 Binney Street  
Cambridge, MA 02142

Attention: Treasurer

Dear Sir or Madam:

I am the holder of [ ]<sup>3</sup> Stock Option granted to me under the Generation Bio Co. (the "**Company**") 2017 Stock Incentive Plan on [ ]<sup>4</sup> for the purchase of [ ]<sup>5</sup> shares of Common Stock of the Company at a purchase price of \$[ ]<sup>6</sup> per share.

I hereby exercise my option to purchase [ ]<sup>7</sup> shares of Common Stock (the "**Shares**"), for which I have enclosed [ ]<sup>8</sup> in the amount of [ ]<sup>9</sup>. Please register my stock certificate as follows:

Name(s): \_\_\_\_\_<sup>10</sup>

Address: \_\_\_\_\_

<sup>2</sup> Enter date of exercise.

<sup>3</sup> Enter either "an Incentive" or "a Nonstatutory" or both.

<sup>4</sup> Enter the date of grant.

<sup>5</sup> Enter the total number of shares of Common Stock for which the option was granted.

<sup>6</sup> Enter the option exercise price per share of Common Stock.

<sup>7</sup> Enter the number of shares of Common Stock to be purchased upon exercise of all or part of the option.

<sup>8</sup> Enter "cash", "personal check" or if permitted by the option or Plan, "stock certificates No. XXXX and XXXX".

<sup>9</sup> Enter the dollar amount (price per share of Common Stock times the number of shares of Common Stock to be purchased), or the number of shares tendered. Fair market value of shares tendered, together with cash or check, must cover the purchase price of the shares issued upon exercise.

<sup>10</sup> Enter name(s) to appear on stock certificate in one of the following formats: (a) your name only (i.e., John Doe); (b) your name and other name (i.e., John Doe and Jane Doe, Joint Tenants with Right to Survivorship); or for Nonstatutory Stock Options only, (c) a child's name, with you as custodian (i.e. Jane Doe, Custodian for Tommy Doe). Note: There may be income and/or gift tax consequences for registering shares in a child's name.

I represent, warrant and covenant as follows:

1. I am purchasing the Shares for my own account for investment only, and not with a view to, or for sale in connection with, any distribution of the Shares in violation of the Securities Act of 1933 (the “**Securities Act**”), or any rule or regulation under the Securities Act.
2. I have had such opportunity as I have deemed adequate to obtain from representatives of the Company such information as is necessary to permit me to evaluate the merits and risks of my investment in the Company.
3. I have sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the purchase of the Shares and to make an informed investment decision with respect to such purchase.
4. I can afford a complete loss of the value of the Shares and am able to bear the economic risk of holding such Shares for an indefinite period.
5. I understand that (i) the Shares have not been registered under the Securities Act and are “restricted securities” within the meaning of Rule 144 under the Securities Act, (ii) the Shares cannot be sold, transferred or otherwise disposed of unless they are subsequently registered under the Securities Act or an exemption from registration is then available; (iii) in any event, the exemption from registration under Rule 144 will not be available for at least six months and even then will not be available unless a public market then exists for the Common Stock, adequate information concerning the Company is then available to the public, and other terms and conditions of Rule 144 are complied with; and (iv) there is now no registration statement on file with the Securities and Exchange Commission with respect to any stock of the Company and the Company has no obligation or current intention to register the Shares under the Securities Act.

By the execution and delivery of this Notice of Stock Option Exercise, I shall be, and hereby agree to be, bound by the (i) Amended and Restated Voting Agreement, dated February 21, 2018, by and among the Company and the other signatories thereto (the “Voting Agreement”), as a “Stockholder” (as defined in the Voting Agreement) for all purposes under the Voting Agreement and (ii) Amended and Restated Right of First Refusal and Co-Sale Agreement, dated February 21, 2018, by and among the Company and the other signatories thereto (the “ROFR and Co-Sale Agreement”), as a “Common Holder” (as defined in the ROFR and Co-Sale Agreement) for all purposes under the ROFR and Co-Sale Agreement. In addition to the foregoing, I shall execute and deliver to the Company (i) an Adoption Agreement in the form attached to the Voting Agreement, thereby agreeing to be bound by and subject to the terms of the Voting Agreement as a “Stockholder” (as defined in the Voting Agreement) and (ii) a counterpart signature page to the ROFR and Co-Sale Agreement, thereby agreeing to be bound by and subject to the terms of the ROFR and Co-Sale Agreement as a “Common Holder” (as defined in the ROFR and Co-Sale Agreement). I acknowledge and agree that I have received a copy of the Voting Agreement and the Right of First Refusal and Co-Sale Agreement.

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Very truly yours,

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[Name]

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**EXHIBIT C**  
**GENERATION BIO Co. 2017 STOCK INCENTIVE PLAN**



**GENERATION BIO CO.**  
**RESTRICTED STOCK AGREEMENT**  
**GRANTED UNDER 2017 STOCK INCENTIVE PLAN**

This Restricted Stock Agreement (the “**Agreement**”) is made this [\_\_\_\_\_] day of [\_\_\_\_\_] 20[\_\_\_], between Generation Bio Co., a Delaware corporation (the “**Company**”), and [\_\_\_\_\_] (the “**Recipient**”).

For valuable consideration, receipt of which is acknowledged, the parties hereto agree as follows:

1. Issuance of Shares.

The Company hereby issues to the Recipient in consideration of services rendered and to be rendered by the Recipient to the Company, subject to the terms and conditions set forth in this Agreement and in the Company’s 2017 Stock Incentive Plan, as amended (the “**Plan**”), [\_\_\_\_\_] shares (the “**Shares**”) of common stock, \$0.0001 par value per share, of the Company (the “**Common Stock**”). Promptly following the execution of this Agreement by the Recipient and, to the extent required, the payment of amounts due under Section [10]/[11(a)] of this Agreement, the Company shall issue to the Recipient one or more certificates in the name of the Recipient for that number of Shares issued to the Recipient. The Recipient agrees that the Shares shall be subject to forfeiture in accordance with Section 3 of this Agreement and the restrictions on transfer set forth in Sections 4 and 5 of this Agreement.

2. Certain Definitions.

(a) If the Recipient is party to an employment, consulting or severance agreement with the Company that contains a definition of “cause” for termination of employment, “**Cause**” shall have the meaning ascribed to such term in such agreement. Otherwise, “Cause” shall exist upon (i) a good faith finding by the Board of Directors of the Company (A) of repeated and willful failure of the Recipient after written notice to perform the Recipient’s reasonably assigned duties for the Company, or (B) that the Recipient has engaged in dishonesty, gross negligence or misconduct, which dishonesty, gross negligence or misconduct has had a material adverse effect on the business affairs of the Company; (ii) the conviction of the Recipient of, or the entry of a pleading of guilty or nolo contendere by the Recipient to, any crime involving moral turpitude or any felony; or (iii) a breach by the Recipient of any material provision of any invention and non-disclosure agreement or non-competition and non-solicitation agreement with the Company, which breach is not cured within ten days written notice thereof.

(b) “**Change in Control**” shall mean the sale of all or substantially all of the outstanding shares of capital stock, assets or business of the Company, by merger, consolidation, sale of assets or otherwise (other than a merger or consolidation in which all or substantially all of the individuals and entities who were beneficial owners of the Company’s voting securities immediately prior to such transaction beneficially own, directly or indirectly, more than 50% (determined on an as-converted basis) of the outstanding securities entitled to vote generally in the election of directors of the resulting, surviving or acquiring corporation in such transaction).”

(c) “Code” shall mean the Internal Revenue Code of 1986, as amended.

(d) “Service” shall mean employment by or the provision of services to the Company or a parent or subsidiary thereof as an advisor, officer, consultant or member of the Board of Directors.

(e) “Vesting Commencement Date” shall mean [\_\_\_\_\_].

### 3. Vesting and Forfeiture of Unvested Shares.

(a) All of the Shares shall initially be subject to forfeiture. The Recipient shall acquire a vested interest in [(i) twenty-five percent (25%) of the Shares upon Recipient’s completion of one year of Service measured from the Vesting Commencement Date and (ii) the balance of the Shares in a series of successive equal quarterly installments of six and one-quarter percent (6.25%) of the Shares upon Recipient’s completion of each additional quarter of Service over the three year period measured from the first anniversary of the Vesting Commencement Date.]

(b) [Subject to the terms of the [Offer Letter/Consulting Agreement/Consulting and Scientific Advisory Board Agreement] dated as of \_\_\_\_\_, 20[\_\_\_] between the Recipient and the Company (the “[Offer Letter/Consulting Agreement]”), in/[In] the event that the Recipient ceases to provide Service for any reason or no reason, with or without Cause, prior to the [fourth (4<sup>th</sup>) anniversary] of the Vesting Commencement Date, vesting shall cease and all of the Shares that have not vested pursuant to this Agreement shall be forfeited immediately and automatically to the Company without payment to the Recipient.

### 4. Restrictions on Transfer.

(a) The Recipient shall not sell, assign, transfer, pledge, hypothecate or otherwise dispose of, by operation of law or otherwise (collectively “transfer”) any Shares that have not vested, or any interest therein, except that the Recipient may transfer such Shares (i) to or for the benefit of any spouse, children, parents, uncles, aunts, siblings, grandchildren and any other relatives approved by the Board of Directors (collectively, “Approved Relatives”) or to a trust established solely for the benefit of the Recipient and/or Approved Relatives; provided that such Shares shall remain subject to this Agreement (including without limitation the restrictions on transfer set forth in this Section 4 and the right of first refusal set forth in Section 5) and such permitted transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Agreement or (ii) as part of the sale of all or substantially all of the shares of capital stock of the Company (including pursuant to a merger or consolidation); provided that, in accordance with the Plan, the securities or other property received by the Recipient in connection with such transaction shall remain subject to this Agreement.

(b) The Recipient shall not transfer any vested Shares, or any interest therein, except in accordance with Section 5 below.

## 5. Right of First Refusal.

(a) If the Recipient proposes to transfer any vested Shares, then the Recipient shall first give written notice of the proposed transfer (the “**Transfer Notice**”) to the Company. The Transfer Notice shall name the proposed transferee and state the number of such Shares the Recipient proposes to transfer (the “**Offered Shares**”), the price per share and all other material terms and conditions of the transfer.

(b) For thirty (30) days following its receipt of such Transfer Notice, the Company shall have the option to purchase all or part of the Offered Shares at the price and upon the terms set forth in the Transfer Notice. In the event the Company elects to purchase all or part of the Offered Shares, it shall give written notice of such election to the Recipient within such 30-day period. Within ten (10) days after the Recipient’s receipt of such notice, the Recipient shall tender to the Company at its principal offices the certificate or certificates representing the Offered Shares to be purchased by the Company, duly endorsed in blank by the Recipient or with duly endorsed stock powers attached thereto, all in a form suitable for transfer of the Offered Shares to the Company. Promptly following receipt of such certificate or certificates, the Company shall deliver or mail to the Recipient a check in payment of the purchase price for such Offered Shares; provided that if the terms of payment set forth in the Transfer Notice were other than cash against delivery, the Company may pay for the Offered Shares on the same terms and conditions as were set forth in the Transfer Notice; and provided further that any delay in making such payment shall not invalidate the Company’s exercise of its option to purchase the Offered Shares.

(c) If the Company does not elect to acquire all of the Offered Shares, the Recipient may, within the 30-day period following the expiration of the option granted to the Company under subsection (b) above, transfer the Offered Shares which the Company has not elected to acquire to the proposed transferee; provided that such transfer shall not be on terms and conditions more favorable to the transferee than those contained in the Transfer Notice. Notwithstanding any of the above, all Offered Shares transferred pursuant to this Section 5 shall remain subject to this Agreement (including without limitation the restrictions on transfer set forth in Section 4 and the right of first refusal set forth in this Section 5) and such transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Agreement.

(d) After the time at which the Offered Shares are required to be delivered to the Company for transfer to the Company pursuant to subsection (b) above, the Company shall not pay any dividend to the Recipient on account of such Offered Shares or permit the Recipient to exercise any of the privileges or rights of a stockholder with respect to such Offered Shares, but shall, insofar as permitted by law, treat the Company as the owner of such Offered Shares.

(e) The following transactions shall be exempt from the provisions of this Section 5:

(1) a transfer of Shares to or for the benefit of any Approved Relatives, or to a trust established solely for the benefit of the Recipient and/or Approved Relatives;

(2) any transfer pursuant to an effective registration statement filed by the Company under the Securities Act of 1933, as amended (the “**Securities Act**”); and

(3) the sale of all or substantially all of the outstanding shares of capital stock of the Company (including pursuant to a merger or consolidation);

provided, however, that in the case of a transfer pursuant to clause (1) above, such Shares shall remain subject to this Agreement (including without limitation the restrictions on transfer set forth in Section 4 and the right of first refusal set forth in this Section 5) and such transferee shall, as a condition to such transfer, deliver to the Company a written instrument confirming that such transferee shall be bound by all of the terms and conditions of this Agreement.

(f) The Company may assign its rights to purchase Offered Shares in any particular transaction under this Section 5 to one or more persons or entities.

(g) The provisions of this Section 5 shall terminate upon the earlier of the following events:

(1) the closing of the sale of shares of Common Stock in an underwritten public offering pursuant to an effective registration statement filed by the Company under the Securities Act; or

(2) a Change in Control.

(h) The Company shall not be required (1) to transfer on its books any of the Shares which shall have been sold or transferred in violation of any of the provisions set forth in this Agreement, or (2) to treat as owner of such Shares or to pay dividends to any transferee to whom any such Shares shall have been so sold or transferred.

#### 6. Agreement in Connection with Initial Public Offering.

The Recipient agrees, in connection with the initial underwritten public offering of the Common Stock pursuant to a registration statement under the Securities Act, (i) not to (a) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any securities convertible into or exercisable or exchangeable for shares of Common Stock or (b) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of shares of Common Stock, whether any transaction described in clause (a) or (b) is to be settled by delivery of shares of Common Stock or other securities, in cash or otherwise, during the period beginning on the date of the filing of such registration statement with the Securities and Exchange Commission and ending 180 days from the date of the final prospectus relating to the offering (plus up to an additional 34 days to the extent requested by the managing underwriters for such offering in order to address NASD Rule 2711(f)(4) or NYSE Rule 472(f)(4) or any similar successor provision), and (ii) to execute any agreement reflecting clause (i) above as may be requested by the Company or the managing underwriters at the time of such offering. The Company may impose stop-transfer instructions with respect to the shares of Common Stock or other securities subject to the foregoing restriction until the end of the “lock-up” period.

7. Escrow.

The Recipient shall, upon the execution of this Agreement, execute Joint Escrow Instructions in the form attached to this Agreement as Exhibit A. The Joint Escrow Instructions shall be delivered to the Secretary of the Company, as escrow agent thereunder. The Recipient shall deliver to such escrow agent a stock assignment duly endorsed in blank, in the form attached to this Agreement as Exhibit B, and hereby instructs the Company to deliver to such escrow agent, on behalf of the Recipient, the certificate(s) evidencing the Shares issued hereunder. Such materials shall be held by such escrow agent pursuant to the terms of such Joint Escrow Instructions.

8. Restrictive Legends.

All certificates representing Shares shall have affixed thereto legends in substantially the following form, in addition to any other legends that may be required under federal or state securities laws:

“The shares of stock represented by this certificate are subject to restrictions on transfer and forfeiture under a certain Restricted Stock Agreement between the corporation and the registered owner of these shares (or such owner’s predecessor in interest), and such Agreement is available for inspection without charge at the office of the Secretary of the corporation.”

“The shares represented by this certificate have not been registered under the Securities Act of 1933, as amended, and may not be sold, transferred or otherwise disposed of in the absence of an effective registration statement under such Act or an opinion of counsel satisfactory to the corporation to the effect that such registration is not required.”

9. Investment Representations.

The Recipient represents, warrants and covenants as follows:

(a) The Recipient is acquiring the Shares for the Recipient’s own account for investment only, and not with a view to, or for sale in connection with, any distribution of the Shares in violation of the Securities Act, or any rule or regulation under the Securities Act.

(b) The Recipient has had such opportunity as the Recipient has deemed adequate to obtain from representatives of the Company such information as is necessary to permit the Recipient to evaluate the merits and risks of Recipient’s investment in the Company.

(c) The Recipient has sufficient experience in business, financial and investment matters to be able to evaluate the risks involved in the receipt of the Shares and to make an informed investment decision with respect to such receipt.

(d) The Recipient can afford a complete loss of the value of the Shares and is able to bear the economic risk of holding such Shares for an indefinite period.

(e) The Recipient understands that (i) the Shares have not been registered under the Securities Act and are “restricted securities” within the meaning of Rule 144 under the Securities Act; (ii) the Shares cannot be sold, transferred or otherwise disposed of unless they are subsequently registered under the Securities Act or an exemption from registration is then available; (iii) in any event, the exemption from registration under Rule 144 will not be available for at least one year and even then will not be available unless a public market then exists for the Common Stock, adequate information concerning the Company is then available to the public, and other terms and conditions of Rule 144 are complied with; and (iv) there is now no registration statement on file with the Securities and Exchange Commission with respect to any stock of the Company and the Company has no obligation or current intention to register the Shares under the Securities Act.

[10. Voting Proxy.

The Recipient hereby constitutes and appoints as the proxy of the Recipient, and hereby grants a power of attorney to, the Chairman of the Board of the Company, with full power of substitution, with respect to any election of directors and any other matter submitted to a vote of the stockholders of the Company (whether taken at an annual or special meeting of stockholders or by written action), and hereby authorizes each of them to represent and to vote all voting securities held by the Recipient as of the applicable record date in such manner as such person shall determine in his sole discretion. The proxy and power of attorney granted by this Section 10 shall terminate and be of no further force or effect until such time as the Company has issued and sold securities having an aggregate purchase price of \$10,000,000 (the “**Completed Financing Date**.” Each of the proxy and power of attorney granted hereby is given in consideration of the issuance of the Shares hereunder and, as such, each is coupled with an interest and shall be irrevocable until the Completed Financing Date.]

[10]/[11]. Withholding Taxes; Section 83(b) Election.

(a) The Recipient acknowledges and agrees that the Company has the right to deduct from payments of any kind otherwise due to the Recipient any federal, state or local taxes of any kind required by law to be withheld with respect to the issuance of the Shares to the Recipient or vesting of the Shares. The Recipient further acknowledges and agrees that, as a condition to the issuance of Shares to the Recipient hereunder, the Company may require the Recipient to satisfy the Company’s tax withholding obligations by making a cash payment to the Company in the amount of the Company’s withholding obligation as determined in good faith by the Company.

(b) The Recipient has reviewed with the Recipient's own tax advisors the federal, state, local and foreign tax consequences of this investment and the transactions contemplated by this Agreement. The Recipient is relying solely on such advisors and not on any statements or representations of the Company or any of its agents. The Recipient understands that the Recipient (and not the Company) shall be responsible for the Recipient's own tax liability that may arise as a result of this investment or the transactions contemplated by this Agreement. The Recipient understands that it may be beneficial in many circumstances to elect to be taxed at the time the Shares are granted by the Company rather than when and as the Shares vest by filing an election under Section 83(b) of the Code with the I.R.S. within 30 days from the date of grant by the Company.

THE RECIPIENT ACKNOWLEDGES THAT IT IS SOLELY THE RECIPIENT'S RESPONSIBILITY AND NOT THE COMPANY'S TO FILE TIMELY THE ELECTION UNDER SECTION 83(b), EVEN IF THE RECIPIENT REQUESTS THE COMPANY OR ITS REPRESENTATIVES TO MAKE THIS FILING ON THE RECIPIENT'S BEHALF.

[11]/[12]. Miscellaneous.

(a) No Rights to Employment. The Recipient acknowledges and agrees that the vesting of the Shares pursuant to Section 3 hereof is earned only by the Recipient's continuous Service (not through the act of being hired or purchasing the Shares hereunder). The Recipient further acknowledges and agrees that the transactions contemplated hereunder and the vesting schedule set forth herein do not constitute an express or implied promise of continued engagement as an employee or consultant for the vesting period, for any period, or at all.

(b) Severability. The invalidity or unenforceability of any provision of this Agreement shall not affect the validity or enforceability of any other provision of this Agreement, and each other provision of this Agreement shall be severable and enforceable to the extent permitted by law.

(c) Waiver. Any provision for the benefit of the Company contained in this Agreement may be waived, either generally or in any particular instance, by the Board of Directors of the Company.

(d) Binding Effect. This Agreement shall be binding upon and inure to the benefit of the Company and the Recipient and their respective heirs, executors, administrators, legal representatives, successors and assigns, subject to the restrictions on transfer set forth in Sections 4 and 5 of this Agreement.

(e) Notice. All notices required or permitted hereunder shall be in writing and deemed effectively given upon personal delivery or five days after deposit in the United States Post Office, by registered or certified mail, postage prepaid, addressed to the other party hereto at the address shown beneath his or her or its respective signature to this Agreement, or at such other address or addresses as either party shall designate to the other in accordance with this Section [11]/[12(e)].

(f) Pronouns. Whenever the context may require, any pronouns used in this Agreement shall include the corresponding masculine, feminine or neuter forms, and the singular form of nouns and pronouns shall include the plural, and vice versa.

(g) Entire Agreement. This Agreement constitutes the entire agreement between the parties, and supersedes all prior agreements and understandings, relating to the subject matter of this Agreement.

(h) Amendment. This Agreement may be amended or modified only by a written instrument executed by both the Company and the Recipient.

(i) Governing Law. This Agreement shall be construed, interpreted and enforced in accordance with the internal laws of the State of Delaware without regard to any applicable conflict of law principles.

(j) Recipient's Acknowledgments. The Recipient acknowledges that he or she: (i) has read this Agreement; (ii) has been represented in the preparation, negotiation, and execution of this Agreement by legal counsel of the Recipient's own choice or has voluntarily declined to seek such counsel; (iii) understands the terms and consequences of this Agreement; (iv) is fully aware of the legal and binding effect of this Agreement; and (v) understands that the law firm of WilmerHale is acting as counsel to the Company in connection with the transactions contemplated by the Agreement, and is not acting as counsel for the Recipient.

[(k) Provisions of the Plan. This Agreement is subject to the provisions of the Plan, a copy of which is furnished to the Recipient with this Agreement.]

*[Remainder of Page Intentionally Left Blank]*



IN WITNESS WHEREOF, the parties hereto have executed the Restricted Stock Agreement as of the date and year first above written. The Recipient hereby agrees to the terms and conditions thereof. The Participant hereby acknowledges receipt of a copy of the Company's 2017 Stock Incentive Plan.

**COMPANY:**

**GENERATION BIO CO.**

By: \_\_\_\_\_

Name: \_\_\_\_\_

Title: \_\_\_\_\_

Address: 301 Binney Street,  
Cambridge, MA 02142

**RECIPIENT:**

By: \_\_\_\_\_

Name: \_\_\_\_\_

Address: \_\_\_\_\_

\_\_\_\_\_

**SIGNATURE PAGE TO RESTRICTED STOCK AGREEMENT GRANTED UNDER STOCK INCENTIVE PLAN**

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**EXHIBIT A**  
**JOINT ESCROW INSTRUCTIONS**

**GENERATION BIO CO.**  
**JOINT ESCROW INSTRUCTIONS**

[\_\_\_\_], 20[\_\_]

Generation Bio Co.  
301 Binney Street,  
Cambridge, MA 02142

Attention: Secretary

Dear Secretary:

As Escrow Agent for Generation Bio Co., a Delaware corporation (the “**Company**”), and its successors in interest under the Restricted Stock Agreement (the “**Agreement**”) of even date herewith, to which a copy of these Joint Escrow Instructions is attached, and the undersigned person (“**Holder**”), you are hereby authorized and directed to hold the documents delivered to you pursuant to the terms of the Agreement in accordance with the following instructions:

1. Appointment. Holder irrevocably authorizes the Company to deposit with you any certificates evidencing Shares (as defined in the Agreement) to be held by you hereunder and any additions and substitutions to said Shares. For purposes of these Joint Escrow Instructions, “**Shares**” shall be deemed to include any additional or substitute property. Holder does hereby irrevocably constitute and appoint you as his or her attorney-in-fact and agent for the term of this escrow to execute with respect to such Shares all documents necessary or appropriate to make such Shares negotiable and to complete any transaction herein contemplated. Subject to the provisions of this Section 1 and the terms of the Agreement, Holder shall exercise all rights and privileges of a stockholder of the Company while the Shares are held by you.

2. Forfeiture of Shares.

(a) Upon any forfeiture by the Holder of the Shares pursuant to the Agreement, the Company shall give to Holder and you a written notice specifying the number of Shares to be forfeited and the time for a closing hereunder (the “**Closing**”) at the principal office of the Company. Holder and the Company hereby irrevocably authorize and direct you to close the transaction contemplated by such notice in accordance with the terms of said notice.

(b) At the Closing, you are directed (i) to date the stock assignment form or forms necessary for the transfer of the Shares, (ii) to fill in on such form or forms the number of Shares being transferred, and (iii) to deliver the same, together with the certificate or certificates evidencing the Shares to be transferred, to the Company.

3. Withdrawal. The Holder shall have the right to withdraw from this escrow any Shares that have vested pursuant to the Agreement.

4. Duties of Escrow Agent.

(a) Your duties hereunder may be altered, amended, modified or revoked only by a writing signed by all of the parties hereto.

(b) You shall be obligated only for the performance of such duties as are specifically set forth herein and may rely and shall be protected in relying or refraining from acting on any instrument reasonably believed by you to be genuine and to have been signed or presented by the proper party or parties. You shall not be personally liable for any act you may do or omit to do hereunder as Escrow Agent or as attorney-in-fact of Holder while acting in good faith and in the exercise of your own good judgment, and any act done or omitted by you pursuant to the advice of your own attorneys shall be conclusive evidence of such good faith.

(c) You are hereby expressly authorized to disregard any and all warnings given by any of the parties hereto or by any other person or entity, excepting only orders or process of courts of law, and are hereby expressly authorized to comply with and obey orders, judgments or decrees of any court. If you are uncertain of any actions to be taken or instructions to be followed, you may refuse to act in the absence of an order, judgment or decrees of a court. In case you obey or comply with any such order, judgment or decree of any court, you shall not be liable to any of the parties hereto or to any other person or entity, by reason of such compliance, notwithstanding any such order, judgment or decree being subsequently reversed, modified, annulled, set aside, vacated or found to have been entered without jurisdiction.

(d) You shall not be liable in any respect on account of the identity, authority or rights of the parties executing or delivering or purporting to execute or deliver the Agreement or any documents or papers deposited or called for hereunder.

(e) You shall be entitled to employ such legal counsel and other experts as you may deem necessary properly to advise you in connection with your obligations hereunder and may rely upon the advice of such counsel.

(f) Your rights and responsibilities as Escrow Agent hereunder shall terminate if (i) you cease to be Secretary of the Company or (ii) you resign by written notice to each party. In the event of a termination under clause (i), your successor as Secretary shall become Escrow Agent hereunder; in the event of a termination under clause (ii), the Company shall appoint a successor Escrow Agent hereunder.

(g) If you reasonably require other or further instruments in connection with these Joint Escrow Instructions or obligations in respect hereto, the necessary parties hereto shall join in furnishing such instruments.

(h) It is understood and agreed that if you believe a dispute has arisen with respect to the delivery and/or ownership or right of possession of the securities held by you hereunder, you are authorized and directed to retain in your possession without liability to anyone all or any part of said securities until such dispute shall have been settled either by mutual written agreement of the parties concerned or by a final order, decree or judgment of a court of competent jurisdiction after the time for appeal has expired and no appeal has been perfected, but you shall be under no duty whatsoever to institute or defend any such proceedings.

(i) These Joint Escrow Instructions set forth your sole duties with respect to any and all matters pertinent hereto and no implied duties or obligations shall be read into these Joint Escrow Instructions against you.

(j) The Company shall indemnify you and hold you harmless against any and all damages, losses, liabilities, costs, and expenses, including attorneys' fees and disbursements, (including without limitation the fees of counsel retained pursuant to Section 4(e) above, for anything done or omitted to be done by you as Escrow Agent in connection with this Agreement or the performance of your duties hereunder, except such as shall result from your gross negligence or willful misconduct.

5. Notice. Any notice required or permitted hereunder shall be given in writing and shall be deemed effectively given upon personal delivery or upon deposit in the United States Post Office, by registered or certified mail with postage and fees prepaid, addressed to each of the other parties thereunto entitled at the following addresses, or at such other addresses as a party may designate by ten days' advance written notice to each of the other parties hereto.

COMPANY: Notices to the Company shall be sent to the address set forth in the salutation hereto, Attn: Chief Executive Officer

HOLDER: Notices to Holder shall be sent to the address set forth below Holder's signature below.

ESCROW AGENT: Notices to the Escrow Agent shall be sent to the address set forth in the salutation hereto.

6. Miscellaneous.

(a) By signing these Joint Escrow Instructions, you become a party hereto only for the purpose of said Joint Escrow Instructions, and you do not become a party to the Agreement.

(b) This instrument shall be binding upon and inure to the benefit of the parties hereto and their respective successors and permitted assigns.

*[Remainder of Page Intentionally Left Blank]*

IN WITNESS WHEREOF, the parties hereto have executed these Joint Escrow Instructions as of the day and year first above written.

Very truly yours,

**COMPANY:**

**GENERATION BIO CO.**

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: \_\_\_\_\_

**HOLDER:**

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Address: \_\_\_\_\_  
\_\_\_\_\_

**ESCROW AGENT:**

By: \_\_\_\_\_  
Name: \_\_\_\_\_  
Title: Secretary

**SIGNATURE PAGE TO JOINT ESCROW INSTRUCTIONS**

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**EXHIBIT B**

**STOCK ASSIGNMENT SEPARATE FROM CERTIFICATE**

**STOCK ASSIGNMENT SEPARATE FROM CERTIFICATE**

FOR VALUE RECEIVED, I hereby sell, assign and transfer unto Generation Bio Co. (the “**Corporation**”) \_\_\_\_\_ (\_\_\_\_\_) shares of Common Stock, \$0.0001 par value per share, of the Corporation standing in my name on the books of the Corporation represented by Certificate(s) Number \_\_\_\_\_ herewith, and do hereby irrevocably constitute and appoint Wilmer Cutler Pickering Hale and Dorr LLP attorney to transfer the said stock on the books of the Corporation with full power of substitution in the premises.

Dated: \_\_\_\_\_

**RECIPIENT:**

\_\_\_\_\_  
[Name]

\_\_\_\_\_  
Name of Spouse (if any):

**Instructions to Recipient:** Please do not fill in any blanks other than the signature line(s). The purpose of the Stock Assignment Separate from Certificate is to enable the Company to acquire the Shares upon forfeiture by Recipient or exercise of its Right of First Refusal without requiring additional signatures on the part of the Recipient or Recipient’s spouse, if any. The signature(s) to this assignment must correspond with the name as written upon the face of the certificate, in every particular, without alteration, enlargement, or any change whatever.



NOTICE ON 83(B) ELECTIONS

**IF YOU WISH TO MAKE A SECTION 83(B) ELECTION, THE FILING OF SUCH ELECTION IS YOUR RESPONSIBILITY.**

**THE FORM FOR MAKING THIS SECTION 83(B) ELECTION IS ATTACHED TO THIS AGREEMENT. YOU MUST FILE THIS FORM WITHIN 30 DAYS OF THE GRANT DATE.**

**YOU (AND NOT THE COMPANY, ANY OF ITS AGENTS OR ANY OTHER PERSON) SHALL BE SOLELY RESPONSIBLE FOR FILING SUCH FORM WITH THE IRS, EVEN IF YOU REQUEST THE COMPANY, ITS AGENTS OR ANY OTHER PERSON TO MAKE THIS FILING ON YOUR BEHALF AND EVEN IF THE COMPANY, ANY OF ITS AGENTS OR ANY OTHER PERSON HAS PREVIOUSLY MADE THIS FILING ON YOUR BEHALF.**

The 83(b) election should be filed by mailing a signed election form by certified mail, return receipt requested to the IRS Service Center where you file your tax returns. See [www.irs.gov](http://www.irs.gov).

**SECTION 83(B) ELECTION**

The undersigned hereby makes an election pursuant to Section 83(b) of the Internal Revenue Code of 1986, as amended, with respect to the property described below and supplies the following information in accordance with Treas. Reg. § 1.83-2:

1. The name, address, and taxpayer identification number of the undersigned are:  
[Name]  
[Address]  
[City, State Zip]  
Taxpayer Identification Number: \_\_\_\_\_
2. The property with respect to which this election is being made is [\_\_\_\_\_] shares of common stock, \$0.0001 par value per share, of Generation Bio Co., a Delaware corporation (the “**Company**”).
3. The date on which the property was transferred or the date on which the restrictions on such property were imposed, whichever is later, is [\_\_\_\_\_] 20[\_\_\_] and the taxable year for which this election is being made is the calendar year 2017.
4. The property is subject to vesting provisions and may be forfeited under the terms of a stock restriction agreement executed between the undersigned and the Company.
5. The fair market value of the property at the time of the transfer or the date on which the restrictions on such property were imposed, whichever is later, (determined without regard to any lapse restriction, as defined in Treas. Reg. § 1.83-3(i)) is \$[\_\_\_\_\_], equal to a fair market value of \$0.001 per share.
6. The amount paid for the property by the undersigned is \$0.00.
7. This statement is executed on \_\_\_\_\_, 20[\_\_\_].

In accordance with Treas. Reg. § 1.83-2(d) & (e)(7), a copy of this statement has been furnished to the Company.

\_\_\_\_\_  
Signature of Taxpayer

\_\_\_\_\_  
Signature of Spouse (if any)

**SECTION 83(B) ELECTION**  
**BACKGROUND INFORMATION**

Section 83(b) of the Internal Revenue Code permits persons who receive restricted property, such as restricted stock, in connection with the performance of services to include the value of such property in their gross income for the year the property is received. Such persons who purchase stock of the company subject to a stock restriction agreement providing for the vesting of such stock over a period of time are entitled to make this election. Any person who makes a timely Section 83(b) election will recognize compensation income on the date of grant (the date listed in item 3 of the election form) equal to the difference, if any, between the fair market value of the stock and the amount paid for the stock. A person who pays taxes in connection with an election and subsequently forfeits the stock, however, will not receive a refund or other tax benefit for the taxes previously paid.

Any person who does not make the election will be required to include the value of the stock in gross income in the year in which the stock vests. In particular, when the stock vests, the person will recognize compensation income in an amount equal to the difference between the fair market value of the stock on the vesting date and the amount paid for the stock. As a result, if the value of the stock increases, a person who does not make a timely Section 83(b) election will have compensation income at the time each installment of stock vests.

Each person should consult with his or her tax or legal advisor regarding the advisability and timing of filing the election. **The original, signed and dated Section 83(b) election must be filed within 30 days of the grant date but may be filed prior to the grant date.** The election should be filed by certified mail, return receipt requested, with the Internal Revenue Service at the service center where the electing person ordinarily files his or her annual tax return. A copy of the Section 83(b) election, as filed, must be returned to the company. A copy of the Section 83(b) election must also be included with the person's federal income tax return for the year of grant (each person should check with his or her tax preparer regarding this and any state, local, foreign or other filing requirements).

**Please also note that the certified mailing receipt for the Section 83(b) election should be retained. This receipt is essential if the Internal Revenue Service does not receive the Section 83(b) election and challenges the election.**

LEASE

by and between

BMR-ROGERS STREET LLC,  
a Delaware limited liability company

and

GENERATION BIO CO.,  
a Delaware corporation

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## LEASE

THIS LEASE (this "Lease") is entered into as of this 2<sup>nd</sup> day of August, 2018 (the "Execution Date"), by and between BMR-ROGERS STREET LLC, a Delaware limited liability company ("Landlord"), and GENERATION BIO CO., a Delaware corporation ("Tenant").

### RECITALS

A. WHEREAS, pursuant to that certain ground lease dated as of March 30, 1999, by and among MBA-Rogers Street, LLC ("Ground Lessor," as successor-in-interest to O&T Realty, LLC, and MBA-Cambridge, LLC (collectively, "Initial Ground Lessor")), as landlord, and Rogers Street, LLC, a Delaware limited liability company ("Initial Ground Lessee"), as tenant; as such ground lease has been amended by that certain letter agreement dated as of July 29, 1999, between Initial Ground Lessor and Initial Ground Lessee, and that certain Agreement Regarding Arbitration and Lease Amendments dated as of December 15, 1999, by and between Initial Ground Lessor and Initial Ground Lessee; and as such ground lease has been assigned pursuant to that certain Assignment and Assumption of Ground Lease dated as of April 4, 2007, by and between Initial Ground Lessee and Landlord (such ground lease, as so amended and assigned, and as the same may be further amended, amended and restated, supplemented or otherwise modified from time to time, the "Ground Lease"), Landlord leases certain real property described on Exhibit A-1 attached hereto (the "Property") and the improvements located thereon, including the buildings at 301 Binney Street (the "Building"), 320 Bent Street and 157 Sixth Street in Cambridge, Massachusetts; and

B. WHEREAS, Landlord wishes to lease to Tenant, and Tenant desires to lease from Landlord, certain premises (the "Premises") located on the first (1st) floor and the fourth (4th) floor of the Building, pursuant to the terms and conditions of this Lease, as detailed below.

### AGREEMENT

NOW, THEREFORE, Landlord and Tenant, in consideration of the mutual promises contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, and intending to be legally bound, agree as follows:

#### 1. Lease of Premises.

1.1 Effective on the Term Commencement Date (as defined below), Landlord hereby leases to Tenant, and Tenant hereby leases from Landlord, the Premises, as shown on Exhibit A attached hereto, including exclusive shafts, cable runs, mechanical spaces and rooftop areas, for exclusive use by Tenant (subject to Landlord's rights hereunder) in accordance with the Permitted Use (as defined below) and no other uses. The Property and all landscaping, parking facilities, private drives and other improvements and appurtenances related thereto, including the Building and other buildings located on the Property, are hereinafter collectively referred to as the "Project." All portions of the Building that are for the non-exclusive use of the tenants of the Building only, and not the tenants of the Project generally, such as service corridors, stairways, elevators, public restrooms and public lobbies (all to the extent located in the Building), are hereinafter referred to as "Building Common Area." All portions of the Project that are for the non-exclusive use of tenants of the Project generally, including driveways, sidewalks, parking areas and landscaped areas, are hereinafter referred to as "Project Common Area." The Building Common Area and Project Common Area are collectively referred to herein as "Common Area."

2. Basic Lease Provisions. For convenience of the parties, certain basic provisions of this Lease are set forth herein. The provisions set forth herein are subject to the remaining terms and conditions of this Lease and are to be interpreted in light of such remaining terms and conditions.

2.1 This Lease shall take effect upon the Execution Date and, except as specifically otherwise provided within this Lease, each of the provisions hereof shall be binding upon and inure to the benefit of Landlord and Tenant from the date of execution and delivery hereof by all parties hereto.

2.2 In the definitions below, each current Rentable Area (as defined below) is expressed in rentable square feet. Rentable Area and "Tenant's Pro Rata Share" are subject to adjustment as provided in this Lease.

| <u>Definition or Provision</u>                     | <u>Means the Following<br/>(As of the Execution Date)</u> |
|--|---|
| Approximate Rentable Area of Fourth Floor Premises | 52,252 square feet  |
| Approximate Rentable Area of First Floor Premises  | 19,310 square feet  |
| Approximate Rentable Area of Premises (total)      | 71,562 square feet  |
| Approximate Rentable Area of Building              | 417,290 square feet                                       |
| Tenant's Pro Rata Share of Building                | 17.15%  |

2.3 Monthly and annual installments of Base Rent for the Fourth Floor Premises ("Fourth Floor Base Rent") as of the Fourth Floor Rent Commencement Date (as defined below), subject to adjustment under this Lease:

| <u>Dates</u>   | <u>Square Feet of<br/>Rentable Area<br/>(Fourth Floor<br/>Premises)</u> | <u>Base Rent per<br/>Square Foot of<br/>Rentable Area</u> | <u>Monthly<br/>Base<br/>Rent</u> | <u>Annual Base<br/>Rent</u> |
|--|---|---|----------------------------------|-----------------------------|
| Fourth Floor Rent Commencement Date—The day immediately prior to the first (1st) annual anniversary of the Fourth Floor Rent Commencement Date | 52,252  | \$86.00 annually  | \$374,472.67                     | \$4,493,672.00              |



2.4 Monthly and annual installments of Base Rent for the First Floor Premises (“First Floor Base Rent”, together with the Fourth Floor Base Rent, “Base Rent”) as of the First Floor Rent Commencement Date (as defined below), subject to adjustment under this Lease:

| <u>Dates</u>   | <u>Square Feet of Rentable Area (First Floor Premises)</u> | <u>Base Rent per Square Foot of Rentable Area</u> | <u>Monthly Base Rent</u> | <u>Annual Base Rent</u> |
|--|--|---|--------------------------|-------------------------|
| First Floor Rent Commencement Date—The day immediately prior to the first (1st) annual anniversary of the First Floor Rent Commencement Date | 19,310   | \$83.00 annually                                  | \$133,560.83             | \$1,602,730             |

2.5 Estimated Fourth Floor Term Commencement Date: August 3, 2018.

2.6 Estimated First Floor Term Commencement Date: January 18, 2019.

2.7 Estimated Term Expiration Date: April 30, 2029.

2.8 Security Deposit: \$2,051,444, subject to increase in accordance with the terms hereof.

2.9 Permitted Use: General office, laboratory and vivarium use in conformity with all federal, state, municipal and local laws, codes, ordinances, rules and regulations of Governmental Authorities (as defined below), committees, associations, or other regulatory committees, agencies or governing bodies having jurisdiction over the Premises, the Building, the Property, the Project, Landlord or Tenant, including both statutory and common law and hazardous waste rules and regulations (“Applicable Laws”).

2.10 Address for Rent Payment:

BMR-Rogers Street LLC  
 Attention Entity 635  
 P.O. Box 511415  
 Los Angeles, California 90051-7970

2.11 Address for Notices to Landlord:

BMR-Rogers Street LLC  
17190 Bernardo Center Drive  
San Diego, California 92128  
Attn: Legal Department

2.12 Address for Notices to Tenant:

Prior to the Fourth Floor Term Commencement Date:

Generation Bio Co.  
215 First Street, Suite 150  
Cambridge, MA 02142  
Attention: Chief Financial Officer

After the Fourth Floor Term Commencement Date:

Generation Bio Co.  
301 Binney Street  
Cambridge, MA 02142  
Attention: Chief Financial Officer

2.13 Address for Invoices to Tenant:

Prior to the Fourth Floor Term Commencement Date:

Generation Bio Co.  
215 First Street, Suite 150  
Cambridge MA 02142  
Attention: Chief Financial Officer

After the Fourth Floor Term Commencement Date:

Generation Bio Co.  
301 Binney Street  
Cambridge, MA 02142  
Attention: Chief Financial Officer

2.14 The following Exhibits are attached hereto and incorporated herein by reference:

- |             |   |
|-------------|---|
| Exhibit A   | Premises  |
| Exhibit A-1 | Property  |
| Exhibit A-2 | Lab/Office Zone Plan  |
| Exhibit B   | Work Letter   |
| Exhibit B-1 | Tenant Work Insurance Schedule  |
| Exhibit C   | Acknowledgement of Fourth Floor Term Commencement Date and Term Expiration Date |

|           |   |
|-----------|---|
| Exhibit D | Acknowledgement of First Floor Commencement Date and Term Expiration Date |
| Exhibit E | Form of Letter of Credit  |
| Exhibit F | Rules and Regulations   |
| Exhibit G | PTDM  |
| Exhibit H | Tenant's Personal Property  |
| Exhibit I | Form of Estoppel Certificate  |
| Exhibit J | Form of Ground Lease Non-Disturbance and Attornment Agreement             |

3. Term. The actual term of this Lease (as the same may be extended pursuant to Article 42 hereof, and as the same may be earlier terminated in accordance with this Lease, the "Term") shall commence on the actual Fourth Floor Term Commencement Date (as defined in Article 4) and end on the date (the "Term Expiration Date") that is the last day of the one hundred twenty-eighth (128th) complete calendar month after the Fourth Floor Term Commencement Date, subject to extension as provided herein.

4. Possession and Commencement Date.

4.1 The "Fourth Floor Term Commencement Date" shall be the date that is two (2) days after the Execution Date, and delivery of possession of the Fourth Floor Premises. Tenant shall execute and deliver to Landlord written acknowledgment of the actual Fourth Floor Term Commencement Date and the Term Expiration Date within ten (10) days after Tenant takes occupancy of the Fourth Floor, in the form attached as Exhibit C hereto. Failure to execute and deliver such acknowledgment, however, shall not affect the Fourth Floor Term Commencement Date or Landlord's or Tenant's liability hereunder. Failure by Tenant to obtain validation by any medical review board or other similar governmental licensing of the Fourth Floor required for the Permitted Use by Tenant shall not serve to extend the Fourth Floor Term Commencement Date.

4.2 Tenant shall cause the work described in the Work Letter (the "Fourth Floor Tenant Improvements") to be constructed in the Fourth Floor Premises pursuant to the Work Letter attached hereto as Exhibit B (the "Work Letter") at a cost to Landlord not to exceed (a) Nine Million Four Hundred Five Thousand Three Hundred Sixty and 00/100 Dollars (\$9,405,360.00) (based upon One Hundred Eighty Dollars (\$180.00) per square foot of Rentable Area (as defined below)) (the "Fourth Floor TI Allowance") plus (b) Seven Hundred Eighty-Three Thousand Seven Hundred Eighty and 00/100 Dollars (\$783,780.00) (based upon Fifteen and 00/100 Dollars (\$15.00) per square foot of Rentable Area) (the "Fourth Floor Base Building Allowance"). The Fourth Floor TI Allowance may be applied to the costs of (m) construction, (n) actual out-of-pocket costs incurred in connection with project review by Landlord, (o) commissioning of mechanical, electrical/ and plumbing systems by a licensed, qualified commissioning agent hired by Tenant, and review of such party's commissioning report by a licensed, qualified commissioning agent hired by Landlord, (p) space planning, architect, engineering and other related services performed by third parties unaffiliated with Tenant, (q) building permits and other taxes, fees, charges and levies by Governmental Authorities (as defined below) for permits or for inspections of the Tenant Improvements, and (r) costs and expenses for labor, material, equipment and fixtures. The Fourth Floor Base Building Allowance may be applied to the same items as the foregoing sentence, except with respect to base building work, and may also be applied as

reimbursement for the cost of the Fourth Floor Tenant Improvements. In no event shall the Fourth Floor TI Allowance or the Fourth Floor Base Building Allowance be used for (u) the cost of work that is not authorized by the Approved Plans (as defined in the Work Letter) or otherwise approved in writing by Landlord, (v) payments to Tenant or any affiliates of Tenant, (w) the purchase of any furniture, personal property or other non-building system equipment, (x) costs arising from any default by Tenant of its obligations under this Lease (y) costs that are recoverable by Tenant from a third party (e.g., insurers, warrantors, or tortfeasors) or (z) costs related to the First Floor Tenant Improvements. In addition, Landlord shall provide an allowance to Tenant to be used solely for architectural and engineering costs related to the Fourth Floor Premises Tenant Improvements in an amount not to exceed Six Thousand Two Hundred Seventy and 24/100 Dollars (\$6,270.24) (based upon 12/100 Dollars (\$0.12) per square foot of Rentable Area) (the "Fourth Floor A/E Allowance"); together with the Fourth Floor TI Allowance and the Fourth Floor Base Building Allowance, the "Fourth Floor Allowance").

4.3 Tenant shall have until the date that is eighteen (18) months after the Fourth Floor Term Commencement Date (the "Fourth Floor TI Deadline"), to submit Fund Requests (as defined in the Work Letter) to Landlord for disbursement of the unused portion of the Fourth Floor Allowance, after which date Landlord's obligation to fund any such costs for which Tenant has not submitted a Fund Request to Landlord shall expire.

4.4 The "First Floor Term Commencement Date" shall be the date that Landlord delivers to Tenant vacant possession of the First Floor Premises. Tenant shall be responsible for physically demising the First Floor Premises, demising the mechanical, electrical and plumbing systems and installing submeters as part of the First Floor Tenant Improvements (as defined below). Tenant shall execute and deliver to Landlord written acknowledgment of the actual First Floor Term Commencement Date within ten (10) days after Tenant takes occupancy of the First Floor Premises, in the form attached as Exhibit D hereto. Failure to execute and deliver such acknowledgment, however, shall not affect the First Floor Term Commencement Date or Landlord's or Tenant's liability hereunder. Failure by Tenant to obtain validation by any medical review board or other similar governmental licensing of the First Floor Premises required for the Permitted Use by Tenant shall not serve to extend the First Floor Term Commencement Date. Notwithstanding anything to the contrary set forth herein, in the event the City of Cambridge fails to issue a building permit to Tenant for the First Floor Tenant Improvements due to the insufficient size of the retail space in the adjacent suite in the Building, Landlord agrees to reduce the First Floor Premises by an amount sufficient to satisfy the City of Cambridge's retail requirements for the Building (but in no event shall the First Floor Premises be decreased by more than 5,214 Rentable Square Feet), and the parties shall cooperate to execute a Lease amendment removing such area from the First Floor Premises.

4.5 Tenant shall cause the work described in the Work Letter (the "First Floor Tenant Improvements") to be constructed in the First Floor Premises pursuant to the Work Letter at a cost to Landlord not to exceed One Million Six Hundred Forty One Thousand Three Hundred Fifty Dollars (\$1,641,350.00) (based upon Eighty-Five Dollars (\$85.00) per square foot of Rentable Area (as defined below)) (the "First Floor TI Allowance"). The First Floor TI Allowance may be applied to the costs of (m) construction, including demising the Premises and submetering, (n) actual out-of-pocket costs incurred in connection with project review by Landlord, (o) commissioning of mechanical, electrical/ and plumbing systems by a licensed, qualified

commissioning agent hired by Tenant, and review of such party's commissioning report by a licensed, qualified commissioning agent hired by Landlord, (p) space planning, architect, engineering and other related services performed by third parties unaffiliated with Tenant, (q) building permits and other taxes, fees, charges and levies by Governmental Authorities (as defined below) for permits or for inspections of the Tenant Improvements, and (r) costs and expenses for labor, material, equipment and fixtures. In no event shall the First Floor TI Allowance or the First Floor Base Building Allowance be used for (v) the cost of work that is not authorized by the Approved Plans (as defined in the Work Letter) or otherwise approved in writing by Landlord, (w) payments to Tenant or any affiliates of Tenant, (x) the purchase of any furniture, personal property or other non-building system equipment, (y) costs arising from any default by Tenant of its obligations under this Lease or (z) costs that are recoverable by Tenant from a third party (e.g., insurers, warrantors, or tortfeasors). Notwithstanding anything to the contrary set forth herein, Tenant may elect to use up to forty percent (40%) of the First Floor TI Allowance to fund the Fourth Floor Tenant Improvements; provided however, that Tenant shall submit separate Fund Requests for costs related to the Fourth Floor Tenant Improvements so that such costs are separately accounted for. In addition, Landlord shall provide an allowance to Tenant to be used solely for architectural and engineering costs related to the Fourth Floor Tenant Improvements in an amount not to exceed Two Thousand Three Hundred Seventeen and 20/100 Dollars (\$2,317.20) (based upon 12/100 Dollars (\$0.12) per square foot of Rentable Area) (the "First Floor A/E Allowance"; together with the First Floor TI Allowance, the "First Floor Allowance").

4.6 Tenant shall have until the date that is twenty-four (24) months after the First Floor Term Commencement Date (the "First Floor TI Deadline"), to submit Fund Requests to Landlord for disbursement of the unused portion of the First Floor Allowance, after which date Landlord's obligation to fund any such costs for which Tenant has not submitted a Fund Request to Landlord shall expire.

4.7 In no event shall any unused Fourth Floor Allowance or First Floor Allowance entitle Tenant to a credit against Rent payable under this Lease. Tenant shall deliver to Landlord (a) a certificate of occupancy (or its substantial equivalent) for each of the Fourth Floor Premises and First Floor Premises suitable for the Permitted Use and (b) a Certificate of Substantial Completion in the form of the American Institute of Architects document G704, executed by the project architect and the general contractor, upon completion of each of the Fourth Floor Premises and First Floor Premises.

4.8 Prior to entering upon the Premises or any portion thereof, Tenant shall furnish to Landlord evidence satisfactory to Landlord that insurance coverages required of Tenant under the provisions of Article 23 are in effect, and such entry shall be subject to all the terms and conditions of this Lease other than the payment of Base Rent or Tenant's Adjusted Share of Operating Expenses (as defined below).

4.9 Landlord and Tenant shall mutually agree upon the selection of the architect, engineer, general contractor and major subcontractors, and Landlord and Tenant shall each participate in the review of the competitive bid process; provided that Landlord hereby pre- approves of The Richmond Group for the Tenant Improvements and/or Tenant's base building work, and provided further that Landlord's approval of any such party proposed by Tenant shall not be unreasonably withheld, conditioned, or delayed. Landlord may refuse to approve any

architects, consultants, contractors, subcontractors or material suppliers that Landlord reasonably believes could cause labor disharmony or may not have sufficient experience, in Landlord's reasonable opinion, to perform work in an occupied Class "A" laboratory research building and in tenant-occupied lab areas.

4.10 Notwithstanding anything to the contrary in this Lease, Landlord and Tenant agree that all Tenant Improvements shall be programmed generally in accordance with the lab and office zones identified on Exhibit A-2 attached hereto.

5. Condition of Premises. Landlord represents to Tenant that, on the Term Commencement Date, all base building systems within the Premises (or the applicable portion thereof), including the HVAC (as hereinafter defined), electrical, life safety and plumbing systems, shall be in good working order (provided that the sole remedy for any breach of the foregoing representation shall be that Landlord shall promptly repair or remedy the violation of the foregoing representation at its sole cost, provided that Landlord may include the costs thereof in Operating Expenses to the extent that Landlord is permitted to do so under Article 9 below, and Tenant shall not be entitled to any monetary damages for any breach of such representation). Except as set forth in the immediately foregoing sentence, Tenant acknowledges that neither Landlord nor any agent of Landlord has made any representation or warranty with respect to the condition of the Premises, the Building or the Project, or with respect to the suitability of the Premises, the Building or the Project for the conduct of Tenant's business. Subject to the foregoing, and without in any way derogating from Landlord's ongoing maintenance, repair and restoration obligations set forth elsewhere in this Lease, Tenant acknowledges that (a) it is fully familiar with the condition of the Premises and agrees to take the same in its condition "as is" as of the Execution Date and (b) Landlord shall have no obligation to alter, repair or otherwise prepare the Premises for Tenant's occupancy or to pay for or construct any improvements to the Premises, except with respect to payment of the TI Allowance and the Base Building Allowance. Tenant's taking of possession of the Premises shall, except as otherwise agreed to in writing by Landlord and Tenant, conclusively establish that the Premises, the Building and the Project were at such time in good, sanitary and satisfactory condition and repair.

#### 6. Rentable Area.

6.1 The term "Rentable Area" shall reflect such areas as reasonably calculated by Landlord's architect in a manner consistent with Landlord's determination of Rentable Area for the remainder of the Building and Project, as the same may be reasonably adjusted from time to time by Landlord in consultation with Landlord's architect only to reflect a physical change to the outer walls, roof or basement of the Building, a physical change to those areas of the Building not utilized as usable area, including that portion of the Building devoted to corridors, equipment rooms, restrooms, elevator lobby, atrium and mailroom, or a physical change to the demising walls of the Premises.

6.2 The Rentable Area of the Building is generally determined by making separate calculations of Rentable Area applicable to each floor within the Building and totaling the Rentable Area of all floors within the Building. The Rentable Area of a floor is computed by measuring to the outside finished surface of the permanent outer Building walls. The full area calculated as previously set forth is included as Rentable Area, without deduction for columns and projections or vertical penetrations, including stairs, elevator shafts, flues, pipe shafts, vertical ducts and the like, as well as such items' enclosing walls.

6.3 The term “Rentable Area,” when applied to the Premises, is that area equal to the usable area of the Premises, plus an equitable allocation of Rentable Area within the Building that is not then utilized or expected to be utilized as usable area, including that portion of the Building devoted to corridors, equipment rooms, restrooms, elevator lobby, atrium and mailroom.

## 7. Rent.

7.1 Tenant shall pay to Landlord as Fourth Floor Base Rent, commencing on the date that is eight (8) months after the Fourth Floor Term Commencement Date (the “Fourth Floor Rent Commencement Date”), the sums set forth in Section 2.3, subject to the rental adjustments provided in Article 8 hereof. Tenant shall pay to Landlord as First Floor Base Rent, commencing on the date that is the earlier of (a) the issuance of a certificate of occupancy (or its equivalent) for the First Floor Premises, or (b) six (6) months after the First Floor Term Commencement Date (the “First Floor Rent Commencement Date”), the sums set forth in Section 2.4, subject to the rental adjustments provided in Article 8 hereof. Base Rent shall be paid in equal monthly installments as set forth in Sections 2.3 and 2.4, subject to the rental adjustments provided in Article 8 hereof, each in advance on the first day of each and every calendar month during the Term.

7.2 In addition to Base Rent, Tenant shall pay to Landlord as additional rent (“Additional Rent”) commencing on the Fourth Floor Rent Commencement Date and the First Floor Rent Commencement Date with respect to the Fourth Floor Premises and the First Floor Premises, respectively, (a) Tenant’s Adjusted Share (as defined below) of Operating Expenses (as defined below), (b) the Property Management Fee (as defined below), and (c) any other amounts that Tenant assumes or agrees to pay under the provisions of this Lease that are owed to Landlord, including any and all other sums that may become due by reason of any default of Tenant or failure on Tenant’s part to comply with the agreements, terms, covenants and conditions of this Lease to be performed by Tenant, after notice and the lapse of any applicable cure periods.

7.3 Base Rent and Additional Rent shall together be denominated “Rent.” Rent shall be paid to Landlord, without abatement, deduction or offset, in lawful money of the United States of America to the address set forth in Section 2.8 or to such other person or at such other place as Landlord may from time designate in writing. In the event the Term commences or ends on a day other than the first day of a calendar month, then the Rent for such fraction of a month shall be prorated for such period on the basis of the number of days in the month and shall be paid at the then-current rate for such fractional month.

7.4 Tenant’s obligation to pay Rent shall not be discharged or otherwise affected by (a) any Applicable Laws now or hereafter applicable to the Premises, (b) any other restriction on Tenant’s use, (c) except as expressly provided herein, any casualty or taking or (d) any other occurrence; and Tenant waives all rights now or hereafter existing to terminate or cancel this Lease or quit or surrender the Premises or any part thereof, or to assert any defense in the nature of constructive eviction to any action seeking to recover rent. Tenant’s obligation to pay Rent with respect to any period or obligations arising, existing or pertaining to the period prior to the date of the expiration or earlier termination of the Term or this Lease shall survive any such expiration or

earlier termination; provided, however, that nothing in this sentence shall in any way affect Tenant's obligations with respect to any other period. Subject to the provisions of this Lease, Tenant shall have the right to injunctive relief or to seek judgements for direct money damages occasioned by Landlord's breach of its Lease covenants beyond notice and applicable cure periods (but may not set-off any such judgement against Rent or other amount owing hereunder). Nothing in this Section 7.4 shall limit the exercise of Tenant's express remedies on the terms and conditions set forth in Section 16.2 below.

8. Rent Adjustments. Each of the Fourth Floor Base Rent and the First Floor Base Rent shall be subject to an annual upward adjustment of three percent (3%) of the then-current applicable Base Rent. The first such adjustment shall become effective commencing on (a) the first (1st) annual anniversary of the Fourth Floor Rent Commencement Date with respect to the Fourth Floor Premises, and (b) the first (1st) anniversary of the First Floor Rent Commencement Date with respect to the First Floor Premises. Subsequent adjustments shall become effective on every successive annual anniversary for each of the Fourth Floor Premises and the First Floor Premises, respectively, for so long as this Lease continues in effect, including during any extension of the Term.

9. Operating Expenses.

9.1 As used herein, the term "Operating Expenses" shall include:

(a) Government impositions, including property tax costs consisting of real and personal property taxes (including amounts due under any improvement bond upon the Building or the Project (including the parcel or parcels of real property upon which the Building, the other buildings in the Project and areas serving the Building and the Project are located)) or assessments in lieu thereof imposed by any federal, state, regional, local or municipal governmental authority, agency or subdivision (each, a "Governmental Authority"); taxes on or measured by gross rentals received from the rental of space in the Project; taxes based on the square footage of the Premises, the Building or the Project, as well as any parking charges, utilities surcharges or any other costs levied, assessed or imposed by, or at the direction of, or arising from Applicable Laws or interpretations thereof, promulgated by any Governmental Authority in connection with the use or occupancy of the Project or the parking facilities serving the Project; taxes on this transaction or any document to which Tenant is a party creating or transferring an interest in the Premises; any fee for a business license to operate an office building; and any expenses, including the reasonable cost of attorneys or experts, reasonably incurred by Landlord in seeking reduction by the taxing authority of the applicable taxes, less tax refunds obtained as a result of an application for review thereof; and

(b) All other costs of any kind paid or incurred by Landlord in connection with the operation or maintenance of the Building and the Project, which shall include costs of repairs and replacements to improvements within the Project as appropriate to maintain the Project as required hereunder; costs of utilities furnished to the Common Area; sewer fees; cable television; trash collection; cleaning, including windows; heating, ventilation and air-conditioning ("HVAC"); maintenance of landscaping and grounds; snow removal; maintenance of drives and parking areas; maintenance of the roof; security services and devices; building supplies; maintenance or replacement of equipment utilized for operation and maintenance of the Project;



license, permit and inspection fees; sales, use and excise taxes on goods and services purchased by Landlord in connection with the operation, maintenance or repair of the Building or Project systems and equipment; telephone, postage, stationery supplies and other expenses incurred in connection with the operation, maintenance or repair of the Project; accounting, legal and other professional fees and expenses incurred in connection with the Project; costs of furniture, draperies, carpeting, landscaping supplies, snow removal and other customary and ordinary items of personal property provided by Landlord for use in the Common Area or the Project office; capital expenditures but only to the extent permitted by Section 9.1(c) below; costs of complying with Applicable Laws (except to the extent such costs are incurred to remedy non-compliance as of the Execution Date with Applicable Laws); costs to keep the Project in compliance with, or costs or fees otherwise required under or incurred pursuant to any Property Operations Documents (as defined below), including condominium fees; insurance premiums, including premiums for commercial general liability, property casualty, earthquake, terrorism and environmental coverages; portions of insured losses paid by Landlord as part of the deductible portion of a loss pursuant to the terms of insurance policies; service contracts; costs of services of independent contractors retained to do work of a nature referenced above; and costs of compensation (including employment taxes and fringe benefits) of all persons who perform regular and recurring duties connected with the day-to-day operation and maintenance of the Project, its equipment, the adjacent walks, landscaped areas, drives and parking areas, including janitors, floor waxers, window washers, watchmen, gardeners, sweepers, plow truck drivers, handymen, and engineering/maintenance/facilities personnel.

(c) Notwithstanding the foregoing, Operating Expenses shall not include any net income, franchise, capital stock, estate or inheritance taxes, or taxes that are the personal obligation of Tenant or of another tenant of the Project; any leasing commissions; expenses (including attorney fees and court costs) incurred in connection with (i) negotiations or disputes with tenants of the Property or other occupants or prospective tenants or other occupants, (ii) the enforcement of any leases or (iii) the defense of Landlord's title to, or interest in, the Building or any part thereof; costs (including permit, license, and inspection fees) incurred in connection with preparing rental space for a tenant, that relate to preparation of rental space for a tenant; expenses of initial development and construction, including grading, paving, landscaping and decorating (as distinguished from maintenance, repair and replacement of the foregoing); Landlord's costs of any services provided to tenants or other occupants for which Landlord is actually reimbursed by such tenants or other occupants (other than reimbursement through Operating Expenses) as an additional charge or rental over and above the basic rent (and escalations thereof) payable under the lease with such tenant or other occupant; costs in connection with services that are provided to another tenant or occupant of the Building, but are not offered to Tenant; capital expenditures, except for those incurred (i) in replacing obsolete equipment, (ii) for the primary purpose of reducing Operating Expenses, or (iii) required to comply with changes in Applicable Laws that take effect after the Execution Date of the Lease, in each case amortized over the useful life thereof (but in no event more than ten (10) years), as reasonably determined by Landlord; costs (i.e., interest and penalties) incurred due to Landlord's default of this Lease or any other lease, mortgage, or other agreement, in each case affecting the Building or Property; payments to subsidiaries or affiliates of Landlord, or to any other party, in each case as a result of a non-arm's length transaction, for management or other services for the Building, or for supplies or other materials for the Building, to the extent that such payments exceed arm's length competitive prices in the Cambridge, Massachusetts market for the services, supplies or materials provided;

Landlord's legal existence and general corporate overhead and general administrative expenses; legal expenses relating to other tenants; costs of repairs to the extent reimbursed by payment of insurance proceeds received by Landlord; advertising and promotional expenditures directly related to Landlord's efforts to lease space in the Building; the cost of repairs of other work occasioned by fire, windstorm or other insured casualty, to the extent Landlord actually receives proceeds of such insurance for such repairs or other work; debt service; interest upon loans to Landlord or secured by mortgage or deed of trust covering the Project or a portion thereof or any other debt of Landlord (provided that interest upon a government assessment or improvement bond payable in installments shall constitute an Operating Expense under Subsection 9.1(a)); rental payments under any ground lease; the cost of correcting defects in the construction of the Building, Building equipment, parking lot or other site improvements, but only to the extent such costs are covered by and actually reimbursed to Landlord under any applicable warranty or service contract held by Landlord; costs incurred directly and solely as a result of Landlord's gross negligence or willful misconduct; salaries of executive officers of Landlord; depreciation claimed by Landlord for tax purposes (provided that this exclusion of depreciation is not intended to delete from Operating Expenses actual costs of repairs and replacements and reasonable reserves in regard thereto that are provided for in Subsection 9.1(b)); taxes that are excluded from Operating Expenses by the last sentence of Subsection 9.1(a); costs or expenses incurred in connection with the financing or sale of the Project or any portion thereof; costs expressly excluded from Operating Expenses elsewhere in this Lease or that are charged to or paid by Tenant under other provisions of this Lease; professional fees and disbursements and other costs and expenses related to the ownership (as opposed to the use, occupancy, operation, maintenance or repair) of the Project; political and charitable contributions; and any item that, if included in Operating Expenses, would involve a double collection for such item by Landlord. To the extent that Tenant uses more than Tenant's Pro Rata Share of any item of Operating Expenses, Tenant shall pay Landlord for such excess in addition to Tenant's obligation to pay Tenant's Pro Rata Share of Operating Expenses (such excess, together with Tenant's Pro Rata Share, "Tenant's Adjusted Share").

9.2 Tenant shall pay to Landlord on the first day of each calendar month of the Term from and after the Fourth Floor Rent Commencement Date and the First Floor Rent Commencement Date (as applicable), as Additional Rent, (a) the Property Management Fee (as defined below) and (b) Landlord's estimate of Tenant's Adjusted Share of Operating Expenses with respect to the Building and the Project, as applicable, for such month.

(x) The "Property Management Fee" shall equal three percent (3%) of the Base Rent due from Tenant. Tenant shall pay the Property Management Fee in accordance with Section 9.2 with respect to the entire Term commencing on the Fourth Floor Rent Commencement Date and the First Floor Rent Commencement Date (as applicable), including any extensions thereof or any holdover periods, regardless of whether Tenant is obligated to pay Base Rent, Operating Expenses or any other Rent with respect to any such period or portion thereof.

(a) Within ninety (90) days after the conclusion of each calendar year (or such longer period as may be reasonably required by Landlord not to exceed one hundred eighty (180) days), Landlord shall furnish to Tenant a statement showing in reasonable detail the actual Operating Expenses, Tenant's Adjusted Share of Operating Expenses, and the cost of providing utilities to the Premises for the previous calendar year ("Landlord's Statement"). Any additional sum due from Tenant to Landlord shall be due and payable within thirty (30) days after receipt of

an invoice therefor. If the amounts paid by Tenant pursuant to this Section exceed Tenant's Adjusted Share of Operating Expenses for the previous calendar year, then Landlord shall credit the difference against the Rent next due and owing from Tenant; provided that, if the Lease term has expired, Landlord shall accompany Landlord's Statement with payment for the amount of such difference.

(b) Any amount due under this Section for any period that is less than a full month shall be prorated for such fractional month on the basis of the number of days in the month.

9.3 Landlord may, from time to time, modify Landlord's calculation and allocation procedures for Operating Expenses, so long as such modifications produce Dollar results substantially consistent with Landlord's then-current practice at the Project. Landlord or an affiliate(s) of Landlord currently own other property(ies) adjacent to the Project or its neighboring properties (collectively, "Neighboring Properties"). In connection with Landlord performing services for the Project pursuant to this Lease, similar services may be performed by the same vendor(s) for Neighboring Properties. In such a case, Landlord shall reasonably allocate to each Building and the Project the costs for such services based upon the ratio that the square footage of the Building or the Project (as applicable) bears to the total square footage of all of the Neighboring Properties or buildings within the Neighboring Properties for which the services are performed, unless the scope of the services performed for any building or property (including the Building and the Project) is disproportionately more or less than for others, in which case Landlord shall equitably allocate the costs based on the scope of the services being performed for each building or property (including the Building and the Project). Since the Project consists of multiple buildings, certain Operating Expenses may pertain to a particular building(s) and other Operating Expenses to the Project as a whole. Landlord reserves the right in its sole discretion to allocate any such costs applicable to any particular building within the Project to such building, and other such costs applicable to the Project to each building in the Project (including the Building), with the tenants in each building being responsible for paying their respective proportionate shares of their buildings to the extent required under their leases. Landlord shall allocate such costs to the buildings (including the Building) in a reasonable, non-discriminatory manner, and such allocation shall be binding on Tenant.

9.4 Landlord's annual statement shall be final and binding upon Tenant unless Tenant, within thirty (30) days after Tenant's receipt thereof, shall contest any item therein by giving written notice to Landlord, specifying each item contested and the reasons therefor; provided that Tenant shall in all events pay the amount specified in Landlord's annual statement, pending the results of the Independent Review and determination of the Accountant(s), as applicable and as each such term is defined below. If, during such thirty (30)-day period, Tenant reasonably and in good faith questions or contests the correctness of Landlord's statement of Tenant's Adjusted Share of Operating Expenses, Landlord shall provide Tenant with reasonable access to Landlord's books and records to the extent relevant to determination of Operating Expenses, and such information as Landlord reasonably determines to be responsive to Tenant's written inquiries. In the event that, after Tenant's review of such information, Landlord and Tenant cannot agree upon the amount of Tenant's Adjusted Share of Operating Expenses, then Tenant shall have the right to have an independent public accounting firm hired by Tenant on an hourly basis and not on a contingent-fee basis (at Tenant's sole cost and expense) and approved by Landlord (which approval Landlord shall not unreasonably withhold or delay) audit and review such of Landlord's

books and records for the year in question as directly relate to the determination of Operating Expenses for such year (the “Independent Review”), but not books and records of entities other than Landlord. Landlord shall make such books and records available at the location where Landlord maintains them in the ordinary course of its business. Landlord need not provide copies of any books or records. Tenant shall commence the Independent Review within thirty (30) days after the date Landlord has given Tenant access to Landlord’s books and records for the Independent Review. Tenant shall complete the Independent Review and notify Landlord in writing of Tenant’s specific objections to Landlord’s calculation of Operating Expenses (including Tenant’s accounting firm’s written statement of the basis, nature and amount of each proposed adjustment) no later than sixty (60) days after Landlord has first given Tenant access to Landlord’s books and records for the Independent Review. Landlord shall review the results of any such Independent Review. The parties shall endeavor to agree promptly and reasonably upon Operating Expenses taking into account the results of such Independent Review. If, as of the date that is sixty (60) days after Tenant has submitted the Independent Review to Landlord, the parties have not agreed on the appropriate adjustments to Operating Expenses, then the parties shall engage a mutually agreeable independent third party accountant with at least ten (10) years’ experience in commercial real estate accounting in the Cambridge, Massachusetts area (the “Accountant”). If the parties cannot agree on the Accountant, each shall within ten (10) days after such impasse appoint an Accountant (different from the accountant and accounting firm that conducted the Independent Review) and, within ten (10) days after the appointment of both such Accountants, those two Accountants shall select a third (which cannot be the accountant and accounting firm that conducted the Independent Review). If either party fails to timely appoint an Accountant, then the Accountant the other party appoints shall be the sole Accountant. Within ten (10) days after appointment of the Accountant(s), Landlord and Tenant shall each simultaneously give the Accountants (with a copy to the other party) its determination of Operating Expenses, with such supporting data or information as each submitting party determines appropriate. Within ten (10) days after such submissions, the Accountants shall by majority vote select either Landlord’s or Tenant’s determination of Operating Expenses. The Accountants may not select or designate any other determination of Operating Expenses. The determination of the Accountant(s) shall bind the parties. If the parties agree or the Accountant(s) determine that the Operating Expenses actually paid by Tenant for the calendar year in question exceeded Tenant’s obligations for such calendar year, then Landlord shall, at Tenant’s option, either (a) credit the excess to the next succeeding installments of estimated Additional Rent or (b) pay the excess to Tenant within thirty (30) days after delivery of such results. If the parties agree or the Accountant(s) determine that Tenant’s payments of Operating Expenses for such calendar year were less than Tenant’s obligation for the calendar year, then Tenant shall pay the deficiency to Landlord within thirty (30) days after delivery of such results. If the Independent Review reveals or the Accountant(s) determine that the Operating Expenses billed to Tenant by Landlord and paid by Tenant to Landlord for the applicable calendar year in question exceeded by more than ten percent (10%) what Tenant should have been billed during such calendar year, then Landlord shall pay the reasonable cost of the Independent Review. In all other cases Tenant shall pay the cost of the Independent Review and the Accountant(s).

9.5 Tenant shall not be responsible for Operating Expenses with respect to any time period prior to the date which is eight (8) months after the Term Commencement Date; Tenant’s responsibility for Tenant’s Adjusted Share of Operating Expenses shall continue to the latest of (a) the date of termination of the Lease, (b) the date Tenant has fully vacated the Premises and (c) if termination of the Lease is due to a default by Tenant, the date of rental commencement of a replacement tenant.

9.6 Operating Expenses for the calendar year in which Tenant's obligation to share therein commences and for the calendar year in which such obligation ceases shall be prorated based on the actual number of days of such calendar year which occur prior to the ceasing of such obligation. Expenses such as taxes, assessments and insurance premiums that are incurred for an extended time period shall be prorated based upon the time periods to which they apply so that the amounts attributed to the Premises relate in a reasonable manner to the time period wherein Tenant has an obligation to share in Operating Expenses.

9.7 Within thirty (30) days after the end of each calendar year quarter (*i.e.* by April 30, July 30, October 30 and January 30) in which Tenant believes it is entitled to reimbursement from Landlord pursuant to the terms of this Lease, Tenant shall submit to Landlord an invoice, or, in the event an invoice is not available, an itemized list, of all costs and expenses that (a) Tenant has incurred (either internally or by employing third parties) during the prior month and (b) for which Tenant reasonably believes it is entitled to reimbursements from Landlord pursuant to the terms of this Lease or the Work Letter. Tenant's failure to send any submittal required by this Section 9.7 shall not be a default of Tenant hereunder nor shall it constitute a waiver of Tenant's right to seek reimbursement from Landlord, however, Tenant shall promptly respond to any written requests from Landlord requesting any such submittal, and to the extent Tenant is seeking any such reimbursement it shall provide the information required under this Section 9.7 in connection with any request for reimbursement.

9.8 In the event that the Building or Project is less than fully occupied during a calendar year, Tenant acknowledges that Landlord may extrapolate Operating Expenses that vary depending on the occupancy of the Building or Project, as applicable, to equal Landlord's reasonable estimate of what such Operating Expenses would have been had the Building or Project, as applicable, been ninety-five percent (95%) occupied during such calendar year; provided, however, that Landlord shall not recover more than one hundred percent (100%) of Operating Expenses.

#### 10. Taxes on Tenant's Property.

10.1 Tenant shall pay prior to delinquency any and all taxes levied against (a) personal property and trade fixtures located at the Premises and (b) any gross or net receipts of or sales by Tenant.

10.2 If any such taxes on Tenant's personal property or trade fixtures are levied against Landlord or Landlord's property or, if the assessed valuation of the Building, the Property or the Project is increased by inclusion therein of a value attributable to Tenant's personal property or trade fixtures, and if Landlord, after written notice to Tenant, pays the taxes based upon any such increase in the assessed value of the Building, the Property or the Project, then Tenant shall, upon demand, repay to Landlord the taxes so paid by Landlord.

10.3 If any improvements in or alterations to the Premises, made or requested by Tenant, whether owned by Landlord or Tenant and whether or not affixed to the real property so as to become a part thereof, are assessed for real property tax purposes at a valuation higher than the valuation at which improvements conforming to Landlord's building standards (the "Building Standard") in other spaces in the Building are assessed, then the real property taxes and assessments levied against Landlord or the Building, the Property or the Project by reason of such excess assessed valuation shall be deemed to be taxes levied against personal property of Tenant and shall be governed by the provisions of Section 10.2. Any such excess assessed valuation due to improvements in or alterations to space in the Project leased by other tenants at the Project shall not be included in Operating Expenses. If the records of the applicable governmental assessor's office are available and sufficiently detailed to serve as a basis for determining whether such Tenant improvements or alterations are assessed at a higher valuation than the Building Standard, then such records shall be binding on both Landlord and Tenant.

#### 11. Security Deposit.

11.1 Tenant shall deposit with Landlord on or before the Execution Date the sum set forth in Section 2.8 (the "Security Deposit"), which sum shall be held by Landlord as security for the faithful performance by Tenant of all of the terms, covenants and conditions of this Lease to be kept and performed by Tenant during the Term and ending upon the expiration or termination of Tenant's obligations under this Lease. If Tenant Defaults (as defined below) with respect to any provision of this Lease, including any provision relating to the payment of Rent, then Landlord may (but shall not be required to) use, apply or retain all or any part of the Security Deposit for the payment of any Rent or any other sum in default, or to compensate Landlord for any other loss or damage that Landlord may suffer by reason of Tenant's default. If any portion of the Security Deposit is so used or applied, then Tenant shall, within ten (10) days following demand therefor, deposit cash with Landlord in an amount sufficient to restore the Security Deposit to its original amount, and Tenant's failure to do so shall be a material breach of this Lease. The provisions of this Article shall survive the expiration or earlier termination of this Lease.

11.2 In the event of bankruptcy or other debtor-creditor proceedings against Tenant, the Security Deposit shall be deemed to be applied first to the payment of Rent and other charges due Landlord for all periods prior to the filing of such proceedings.

11.3 Landlord may deliver to any purchaser of Landlord's interest in the Premises the funds deposited hereunder by Tenant, and thereupon Landlord shall be discharged from any further liability with respect to such deposit. This provision shall also apply to any subsequent transfers.

11.4 If Tenant shall fully and faithfully perform every provision of this Lease to be performed by it, then the Security Deposit, or any balance thereof, shall be returned to Tenant (or, at Landlord's option, to the last assignee of Tenant's interest hereunder) within thirty (30) days after the expiration or earlier termination of this Lease.

11.5 If the Security Deposit shall be in cash, Landlord shall hold the Security Deposit in an account at a banking organization selected by Landlord; provided, however, that Landlord shall not be required to maintain a separate account for the Security Deposit, but may intermingle it with other funds of Landlord. Landlord shall be entitled to all interest and/or dividends, if any, accruing on the Security Deposit. Landlord shall not be required to credit Tenant with any interest for any period during which Landlord does not receive interest on the Security Deposit.

11.6 The Security Deposit may be in the form of cash, a letter of credit or any other security instrument acceptable to Landlord in its sole discretion. Tenant may at any time, except when Tenant is in Default (as defined below), deliver a letter of credit (the "L/C Security") as the entire Security Deposit, as follows:

(a) If Tenant elects to deliver L/C Security, then Tenant shall provide Landlord, and maintain in full force and effect throughout the Term and until the date that is six (6) months after the then-current Term Expiration Date, a letter of credit in the form of Exhibit E issued by an issuer reasonably satisfactory to Landlord, in the amount of the Security Deposit, with an initial term of at least one year. Landlord may require the L/C Security to be re-issued by a different issuer at any time during the Term if Landlord reasonably believes that the issuing bank of the L/C Security is or may soon become insolvent; provided, however, Landlord shall return the existing L/C Security to the existing issuer immediately upon receipt of the substitute L/C Security. If any issuer of the L/C Security shall become insolvent or placed into FDIC receivership, then Tenant shall immediately deliver to Landlord (without the requirement of notice from Landlord) substitute L/C Security issued by an issuer reasonably satisfactory to Landlord, and otherwise conforming to the requirements set forth in this Article. As used herein with respect to the issuer of the L/C Security, "insolvent" shall mean the determination of insolvency as made by such issuer's primary bank regulator (*i.e.*, the state bank supervisor for state chartered banks; the OCC or OTS, respectively, for federally chartered banks or thrifts; or the Federal Reserve for its member banks). If, at the Term Expiration Date, any Rent remains uncalculated or unpaid, then (i) Landlord shall with reasonable diligence complete any necessary calculations, (ii) Tenant shall extend the expiry date of such L/C Security from time to time as Landlord reasonably requires and (iii) in such extended period, Landlord shall not unreasonably refuse to consent to an appropriate reduction of the L/C Security. Tenant shall reimburse Landlord's legal costs (as estimated by Landlord's counsel and not to exceed \$3,000) in handling Landlord's acceptance of L/C Security or its replacement or extension.

(b) If Tenant delivers to Landlord satisfactory L/C Security in place of the entire Security Deposit, Landlord shall remit to Tenant any cash Security Deposit Landlord previously held.

(c) Landlord may draw upon the L/C Security, and hold and apply the proceeds in the same manner and for the same purposes as the Security Deposit, if (i) an uncured Default (as defined below) exists, (ii) as of the date that is forty-five (45) days before any L/C Security expires (even if such scheduled expiry date is after the Term Expiration Date) Tenant has not delivered to Landlord an amendment or replacement for such L/C Security, reasonably satisfactory to Landlord, extending the expiry date to the earlier of (1) six (6) months after the then-current Term Expiration Date or (2) the date that is one year after the then-current expiry date of the L/C Security, (iii) the L/C Security provides for automatic renewals, Landlord asks the issuer to confirm the current L/C Security expiry date, and the issuer fails to do so within ten (10) business days, (iv) Tenant fails to pay (when and as Landlord reasonably requires) any bank charges for Landlord's transfer of the L/C Security or (v) the issuer of the L/C Security ceases, or announces that it will cease, to maintain an office in the city where Landlord may present drafts under the L/C Security (and fails to permit drawing upon the L/C Security by overnight courier or facsimile). This Section does not limit any other provisions of this Lease allowing Landlord to draw the L/C Security under specified circumstances.

(d) Tenant shall not seek to enjoin, prevent, or otherwise interfere with Landlord's draw under L/C Security, even if it violates this Lease. Tenant acknowledges that the only effect of a wrongful draw would be to substitute a cash Security Deposit for L/C Security, causing Tenant no legally recognizable damage. Landlord shall hold the proceeds of any draw in the same manner and for the same purposes as a cash Security Deposit. In the event of a wrongful draw, the parties shall cooperate to allow Tenant to post replacement L/C Security simultaneously with the return to Tenant of the wrongfully drawn sums, and Landlord shall upon request confirm in writing to the issuer of the L/C Security that Landlord's draw was erroneous.

(e) If Landlord transfers its interest in the Premises, then Tenant shall at Tenant's expense, within five (5) business days after receiving a request from Landlord, deliver (and, if the issuer requires, Landlord shall consent to) an amendment to the L/C Security naming Landlord's grantee as substitute beneficiary. If the required Security Deposit changes while L/C Security is in force, then Tenant shall deliver (and, if the issuer requires, Landlord shall consent to) a corresponding amendment to the L/C Security.

## 12. Use.

12.1 Tenant shall use the Premises for the Permitted Use, and shall not use the Premises, or permit or suffer the Premises to be used, for any other purpose without Landlord's prior written consent, which consent Landlord may withhold in its sole and absolute discretion.

12.2 Tenant shall not use or occupy the Premises in violation of Applicable Laws; zoning ordinances; or the certificate of occupancy (or its substantial equivalent) issued for the Building or the Project, and shall, upon five (5) days' written notice from Landlord, discontinue any use of the Premises that is declared or claimed by any Governmental Authority having jurisdiction to be a violation of any of the above, or that in Landlord's reasonable opinion violates any of the above. Tenant shall comply with any direction of any Governmental Authority having jurisdiction that shall, by reason of the nature of Tenant's use or occupancy of the Premises, impose any duty upon Tenant or Landlord with respect to the Premises or with respect to the use or occupation thereof, and shall indemnify, defend (at Landlord's option and with counsel reasonably acceptable to Landlord) and hold harmless (collectively, "Indemnify," "Indemnity" or "Indemnification," as the case may require) Landlord and its affiliates, employees, agents and contractors; and any lender, mortgagee, ground lessor or beneficiary (each, a "Lender" and, collectively with Landlord and its affiliates, employees, agents and contractors, the "Landlord Indemnitees") harmless from and against any and all demands, claims, liabilities, losses, costs, expenses, actions, causes of action, damages, suits or judgments, and all reasonable expenses (including reasonable attorneys' fees, charges and disbursements, regardless of whether the applicable demand, claim, action, cause of action or suit is voluntarily withdrawn or dismissed) incurred in investigating or resisting the same (collectively, "Claims") of any kind or nature that arise before, during or after the Term as a result of Tenant's breach of this Section.

12.3 Tenant shall not do or permit to be done anything that will invalidate or increase the cost of any fire, environmental, extended coverage or any other insurance policy covering the Building or the Project, and shall comply with all reasonable rules, orders, regulations and requirements of the insurers of the Building and the Project, and Tenant shall promptly, upon demand, reimburse Landlord for any additional premium charged for such policy by reason of Tenant's failure to comply with the provisions of this Article.



12.4 Tenant shall keep all doors opening onto public corridors closed, except when in use for ingress and egress.

12.5 No additional locks or bolts of any kind shall be placed upon any of the doors or windows by Tenant, nor shall any changes be made to existing locks or the mechanisms thereof without Landlord's prior written consent, which shall not be unreasonably withheld, conditioned, or delayed. Tenant shall, upon termination of this Lease, return to Landlord all keys to offices and restrooms either furnished to or otherwise procured by Tenant. In the event any key so furnished to Tenant is lost, Tenant shall pay to Landlord the cost of replacing the same or of changing the lock or locks opened by such lost key if Landlord shall deem it necessary to make such change.

12.6 No awnings or other projections shall be attached to any outside wall of the Building. No curtains, blinds, shades or screens shall be attached to or hung in, or used in connection with, any window or door of the Premises other than Landlord's standard window coverings. Neither the interior nor exterior of any windows shall be coated or otherwise sunscreensed without Landlord's prior written consent, nor shall any bottles, parcels or other articles be placed on the windowsills or items attached to windows that are visible from outside the Premises. No equipment, furniture or other items of personal property shall be placed on any exterior balcony without Landlord's prior written consent.

12.7 No sign, advertisement or notice ("Signage") shall be exhibited, painted or affixed by Tenant on any part of the Premises or the Building without Landlord's prior written consent. Signage shall conform to Landlord's design criteria. For any Signage, Tenant shall, at Tenant's own cost and expense, (a) acquire all permits for such Signage in compliance with Applicable Laws and (b) design, fabricate, install and maintain such Signage in a first-class condition. Tenant shall be responsible for reimbursing Landlord for costs incurred by Landlord in removing any of Tenant's Signage upon the expiration or earlier termination of the Lease. Interior signs in the Building lobby and the directory tablet shall be inscribed, painted or affixed for Tenant by Landlord at Landlord's sole cost and expense, and shall be of a size, color and type and be located in a place acceptable to Landlord. The directory tablet shall be provided exclusively for the display of the name and location of tenants only. Tenant shall not place anything on the exterior of the corridor walls or corridor doors other than Landlord's standard lettering. Tenant, at Tenant's sole cost and expense, shall have Signage rights for the primary entrance to the Premises substantially consistent with the Signage permitted for comparable Tenants in the Project, as Landlord reasonably determines. At Landlord's option, Landlord may install any Tenant Signage, and Tenant shall pay all costs associated with such installation within thirty (30) days after demand therefor.

12.8 Tenant may only place equipment within the Premises with floor loading consistent with the Building's structural design unless Tenant obtains Landlord's prior written approval. Tenant may place such equipment only in a location designed to carry the weight of such equipment.

12.9 Tenant shall cause any equipment or machinery to be installed in the Premises so as to reasonably prevent sounds or vibrations therefrom from extending into the Common Area or other offices in the Project.

12.10 Tenant shall not (a) do or permit anything to be done in or about the Premises that shall in any way obstruct or interfere with the rights of other tenants or occupants of the Project, or injure or annoy them, (b) use or allow the Premises to be used for immoral, unlawful or objectionable purposes, (c) cause, maintain or permit any nuisance or waste in, on or about the Project or (d) take any other action that would in Landlord's reasonable determination in any manner adversely affect other tenants' quiet use and enjoyment of their space or adversely impact their ability to conduct business in a professional and suitable work environment. Notwithstanding anything in this Lease to the contrary, Tenant may not install any security systems (including cameras) outside the Premises or that record sounds or images outside the Premises without Landlord's prior written consent, which Landlord may withhold in its sole and absolute discretion.

12.11 Notwithstanding any other provision herein to the contrary, from and after the Term Commencement Date, Tenant shall be responsible for all liabilities, costs and expenses arising from or in connection with the compliance of the Premises with the Americans with Disabilities Act, 42 U.S.C. § 12101, et seq., and any state and local accessibility laws, codes, ordinances and rules (collectively, and together with regulations promulgated pursuant thereto, the "ADA") (except to the extent that any such noncompliance of the Premises with the ADA (as in effect and interpreted as of the Term Commencement Date) existed as of the Term Commencement Date), and Tenant shall Indemnify the Landlord Indemnitees from and against Claims arising out of Tenant's failure to comply with its obligations relating to the ADA under this Section. Landlord shall be responsible for all liabilities, costs and expenses arising out of or in connection with the compliance of the Common Areas with the ADA (which costs may be included in Operating Expenses to the extent permitted in Article 9 except to the extent that any such noncompliance of the Common Areas with the ADA (as in effect and interpreted as of the Term Commencement Date) existed as of the Term Commencement Date). This Section (as well as any other provisions of this Lease dealing with Indemnification of the Landlord Indemnitees by Tenant) shall be deemed to be modified in each case by the insertion in the appropriate place of the following: "except as otherwise provided in Mass. G.L. Ter. Ed., C. 186, Section 15." For the avoidance of doubt, "Lenders" shall also include historic tax credit investors and new market tax credit investors. The provisions of this Section shall survive the expiration or earlier termination of this Lease.

12.12 Tenant shall maintain temperature and humidity in the Premises in accordance with ASHRAE standards at all times.

12.13 Tenant shall establish and maintain a chemical safety program administered by a licensed, qualified individual in accordance with the requirements of the Massachusetts Water Resources Authority ("MWRA") and any other applicable Governmental Authority. Tenant shall be solely responsible for all costs incurred in connection with such chemical safety program, and Tenant shall provide Landlord with such documentation as Landlord may reasonably require evidencing Tenant's compliance with the requirements of (a) the MWRA and any other applicable Governmental Authority with respect to such chemical safety program and (b) this Section. Tenant shall be required to obtain and maintain during the Term (m) any permit required by the MWRA

(“MWRA Permit”) and (n) a wastewater treatment operator license from the Commonwealth of Massachusetts with respect to Tenant’s Acid Neutralization Tank (as defined below) in the Building. Tenant shall not introduce anything into the Acid Neutralization Tank (x) in violation of the terms of the MWRA Permit, (y) in violation of Applicable Laws or (z) that would interfere with the proper functioning of the Acid Neutralization Tank. Landlord agrees to reasonably cooperate with Tenant in order for Tenant to obtain the MWRA Permit and the wastewater treatment operator license, without any obligation for Landlord to incur any costs in connection therewith. Tenant shall reimburse Landlord within ten (10) business days after demand for any costs incurred by Landlord pursuant to this Section.

13. Rules and Regulations, CC&Rs, Parking Facilities and Common Area.

13.1 Tenant shall have the non-exclusive right, in common with others, to use the Common Area in conjunction with Tenant’s use of the Premises for the Permitted Use, and such use of the Common Area and Tenant’s use of the Premises shall be subject to the rules and regulations adopted by Landlord and attached hereto as Exhibit F, together with such other reasonable and nondiscriminatory rules and regulations as are hereafter reasonably promulgated by Landlord in its sole and absolute discretion (the “Rules and Regulations”). Tenant shall ensure that its contractors, subcontractors, employees, subtenants and invitees faithfully observe and comply with the Rules and Regulations. Landlord shall not be responsible to Tenant for the violation or non-performance by any other tenant or any agent, employee or invitee thereof of any of the Rules and Regulations.

13.2 This Lease is subject to any recorded covenants, conditions or restrictions on the Project or Property (including the Parking and Transportation Demand Management Plan Ordinance- Final Amendment Decision, issued on May 24, 2002, by the City of Cambridge (as the same may be amended from time to time, the “PTDM”) , as the same may be amended, amended and restated, supplemented or otherwise modified from time to time (the “CC&Rs”) and Tenant shall, at its sole cost and expense, comply with and cause the Project to comply with the CC&Rs and the documents listed on Exhibit G attached hereto (together with the PTDM, the “Property Operations Documents”). Tenant acknowledges that Tenant, at its sole cost and expense, shall comply with the tenant requirements in the PTDM, including the requirements set forth in the “Alternative Work Programs,” “Public Transportation Incentives,” “Ridesharing Programs” and “Provisions of Bicycle and Pedestrian Amenities” sections thereof. Tenant, at its sole cost and expense, shall also comply with the reporting requirements set forth in the PTDM at Landlord’s request. Any costs incurred by Landlord in connection with the PTDM shall constitute an Operating Expense.

13.3 The Charles River Transportation Management Association (of which Landlord or an affiliate of Landlord is currently a member) provides certain programs to help improve transportation in the Cambridge area. Their website is [www.charlesrivertma.org](http://www.charlesrivertma.org).

13.4 Tenant shall have a non-exclusive, irrevocable license to use (a) forty-seven (47) parking spaces with respect to the Fourth Floor Premises, and (b) eighteen (18) parking spaces with respect to the First Floor Premises, in the facilities serving the Building and the Project in common on an unreserved basis with other tenants of the Building and the Project during the Term at a cost of Three Hundred Forty and 00/100 Dollars (\$340.00) per parking space per month

(subject to market rate adjustment by Landlord from time to time throughout the Term), which Tenant shall pay commencing on the respective Rent Commencement Date simultaneously with payments of Base Rent as Additional Rent. Tenant, at any time and from time to time during the Term, may elect to waive its right to use some or all of its parking spaces upon written notice to Landlord. If Tenant so elects, then it shall forfeit for the then-remainder of the Term (including any extension thereof) any and all rights to such waived parking spaces, and no amounts shall be due with respect to such waived parking spaces thereafter. Tenant shall have the right to rent additional parking spaces on a monthly basis, subject to availability.

13.5 Tenant agrees not to unreasonably overburden the parking facilities and agrees to cooperate with Landlord and other tenants in the use of the parking facilities, and Landlord hereby agrees that Tenant shall not be deemed to be overburdening the parking facilities if Tenant is using the number of spaces (or fewer) then allocated to Tenant and Tenant is otherwise complying with any rules and regulations concerning the parking facilities. Landlord reserves the right to determine that parking facilities are becoming overcrowded and to limit Tenant's use thereof. Upon such determination, Landlord may reasonably allocate parking spaces among Tenant and other tenants of the Building or the Project; provided, however, that Landlord shall not be permitted to reduce the number of parking spaces to which Tenant is then entitled to use under this Lease. Nothing in this Section, however, is intended to create an affirmative duty on Landlord's part to monitor parking.

13.6 Subject to the terms of this Lease including the Rules and Regulations and the rights of other tenants of the Building, Tenant shall have the non-exclusive right to access the freight loading dock and the freight elevator twenty-four (24) hours per day, seven (7) days per week, at no additional cost. Landlord shall not be responsible for any coordination of the use of the freight elevator or the loading dock by tenants at the Building. Landlord shall provide a dumpster and/or trash compactor at the loading dock for Tenant's use for the disposal of non-Hazardous Materials, and Tenant shall pay Tenant's Adjusted Share of the cost of said dumpster and/or trash compactor. Tenant shall be solely responsible for the disposal of any Hazardous Materials in accordance with Applicable Laws

#### 14. Project Control by Landlord.

14.1 Landlord reserves full control over the Building and the Project to the extent not inconsistent with Tenant's enjoyment of the Premises as provided by this Lease. This reservation includes Landlord's right to subdivide the Project; convert the Building and the other buildings within the Project to condominium units; change the size of the Project by selling all or a portion of the Project or adding real property and any improvements thereon to the Project; grant easements and licenses to third parties; maintain or establish ownership of the Building separate from fee title to the Property; make additions to or reconstruct portions of the Building and the Project; install, use, maintain, repair, replace and relocate for service to the Premises and other parts of the Building or the Project pipes, ducts, conduits, wires and appurtenant fixtures, wherever located in the Premises, the Building or elsewhere at the Project; and alter or relocate any other Common Area or facility, including private drives, lobbies, entrances and landscaping; provided, however, that such rights shall be exercised in a way that does not materially adversely affect Tenant's beneficial use and occupancy of the Premises, including the Permitted Use and Tenant's access to the Premises. Tenant acknowledges that Landlord specifically reserves the right to allow

the exclusive use of corridors and restroom facilities located on specific floors to one or more tenants occupying such floors; provided, however, that Tenant shall not be deprived of the use of the corridors reasonably required to serve the Premises or of restroom facilities serving the floor upon which the Premises are located.

14.2 Possession of areas of the Premises necessary for utilities, services, safety and operation of the Building is reserved to Landlord.

14.3 Tenant shall, at Landlord's request, promptly execute such further documents as may be reasonably appropriate to assist Landlord in the performance of its obligations hereunder; provided that Tenant need not execute any document that creates additional liability for Tenant or that deprives Tenant of the quiet enjoyment and use of the Premises as provided for in this Lease.

14.4 Landlord may, at any and all reasonable times during non-business hours (or during business hours, if (a) with respect to Subsections 14.4(u) through 14.4(y), Tenant so requests, and (b) with respect to Subsection 14.4(z), if Landlord so requests), and upon twenty-four (24) hours' prior notice (which may be oral or by email to the office manager or other Tenant-designated individual at the Premises; but provided that no time restrictions shall apply or advance notice be required if an emergency necessitates immediate entry), enter the Premises to (u) inspect the same and to determine whether Tenant is in compliance with its obligations hereunder, (v) supply any service Landlord is required to provide hereunder, (w) alter, improve or repair any portion of the Building other than the Premises for which access to the Premises is reasonably necessary, (x) post notices of nonresponsibility, (y) access the telephone equipment, electrical substation and fire risers and (z) show the Premises to prospective tenants during the final year of the Term and current and prospective purchasers and lenders at any time. In connection with any such alteration, improvement or repair as described in Subsection 14.4(w), Landlord may erect in the Premises or elsewhere in the Project scaffolding and other structures reasonably required for the alteration, improvement or repair work to be performed. In no event shall Tenant's Rent abate as a result of Landlord's activities pursuant to this Section; provided, however, that all such activities shall be conducted in such a manner so as to cause as little interference to Tenant as is reasonably possible. Landlord shall at all times retain a key with which to unlock all of the doors in the Premises. If an emergency necessitates immediate access to the Premises, Landlord may use whatever force is necessary to enter the Premises, and any such entry to the Premises shall not constitute a forcible or unlawful entry to the Premises, a detainer of the Premises, or an eviction of Tenant from the Premises or any portion thereof.

15. Quiet Enjoyment. Landlord covenants that Tenant, so long as no Default (as hereinafter defined) has occurred and is continuing under this Lease, may peacefully and quietly have, hold and enjoy the Premises, free from any claim by Landlord or persons claiming under Landlord, but subject to all of the terms and provisions hereof, provisions of Applicable Laws and rights of record to which this Lease is or may become subordinate. This covenant is in lieu of any other quiet enjoyment covenant, either express or implied.

## 16. Utilities and Services.

16.1 Tenant shall pay for all water (including the cost to service, repair and replace reverse osmosis, de-ionized and other treated water), gas, heat, light, power, telephone, internet service, cable television, other telecommunications and other utilities supplied to the Premises, together with any fees, surcharges and taxes thereon commencing on the applicable Term Commencement Date. If any such utility is not separately metered or sub-metered to Tenant, Tenant shall pay Tenant's Adjusted Share of all charges of such utility jointly metered with other premises as part of Tenant's Adjusted Share of Operating Expenses or, in the alternative, Landlord may, at its option, monitor the usage of such utilities by Tenant and charge Tenant with the cost of purchasing, installing and monitoring such metering equipment, which cost shall be paid by Tenant as Additional Rent. Landlord may base its bills for utilities on reasonable estimates; provided that Landlord adjusts such billings promptly thereafter or as part of the next Landlord's Statement to reflect the actual cost of providing utilities to the Premises. To the extent that Tenant uses more than Tenant's Pro Rata Share of any utilities, then Tenant shall pay Landlord for Tenant's Adjusted Share of such utilities to reflect such excess. In the event that the Building or Project is less than fully occupied during a calendar year, Tenant acknowledges that Landlord may extrapolate utility usage that varies depending on the occupancy of the Building or Project (as applicable) to equal Landlord's reasonable estimate of what such utility usage would have been had the Building or Project, as applicable, been ninety-five percent (95%) occupied during such calendar year; provided, however, that Landlord shall not recover more than one hundred percent (100%) of the cost of such utilities. Tenant shall not be liable for the cost of utilities supplied to the Premises attributable to the time period prior to the Term Commencement Date; provided, however, that, if Landlord shall permit Tenant possession of the Premises prior to the Term Commencement Date and Tenant uses the Premises for any purpose other than placement of personal property as set forth in Section 4.3, then Tenant shall be responsible for the cost of utilities supplied to the Premises from such earlier date of possession.

16.2 Landlord shall not be liable for, nor shall any eviction of Tenant result from, the failure to furnish any utility or service, whether or not such failure is caused by accidents; breakage; casualties (to the extent not caused by the party claiming Force Majeure); Severe Weather Conditions (as defined below); physical natural disasters (but excluding weather conditions that are not Severe Weather Conditions); strikes, lockouts or other labor disturbances or labor disputes (other than labor disturbances and labor disputes resulting solely from the acts or omissions of the party claiming Force Majeure); acts of terrorism; riots or civil disturbances; wars or insurrections; shortages of materials (which shortages are not unique to the party claiming Force Majeure); government regulations, moratoria or other governmental actions, inactions or delays; failures to grant consent or delays in granting consent by any Lender whose consent is required under any applicable Loan Document failures by third parties to deliver gas, oil or another suitable fuel supply, or inability of the party claiming Force Majeure, by exercise of reasonable diligence, to obtain gas, oil or another suitable fuel; or other causes beyond the reasonable control of the party claiming that Force Majeure has occurred (collectively, "Force Majeure"); or, to the extent permitted by Applicable Laws, Landlord's negligence. In the event of such failure, Tenant shall not be entitled to termination of this Lease or any abatement or reduction of Rent, nor shall Tenant be relieved from the operation of any covenant or agreement of this Lease. "Severe Weather Conditions" means weather conditions that are materially worse than those that reasonably would be anticipated for the Property at the applicable time based on historic meteorological records. Notwithstanding anything to the contrary in this Lease, if, for more than five (5) consecutive business days following written notice to Landlord and as a direct result of Landlord's gross negligence or willful misconduct (and except to the extent that such failure is caused in whole or in part by the action or inaction of a Tenant Party (as defined below)), the provision of HVAC or

other utilities to all or a material portion of the Premises that Landlord must provide pursuant to this Lease is interrupted (a “Material Services Failure”), then Tenant’s Base Rent and Tenant’s Adjusted Share of Operating Expenses (or, to the extent that less than all of the Premises are affected, a proportionate amount (based on the Rentable Area of the Premises that is rendered unusable) of Base Rent and Tenant’s Adjusted Share of Operating Expenses) shall thereafter be abated until the Premises are again usable by Tenant for the Permitted Use; provided, however, that, if Landlord is diligently pursuing the restoration of such HVAC and other utilities and Landlord provides substitute HVAC and other utilities reasonably suitable for Tenant’s continued use and occupancy of the Premises for the Permitted Use (e.g., supplying potable water or portable air conditioning equipment), then neither Base Rent nor Operating Expenses shall be abated. During any Material Services Failure, Tenant will cooperate with Landlord to arrange for the provision of any interrupted utility services on an interim basis via temporary measures until final corrective measures can be accomplished, and Tenant will permit Landlord the necessary access to the Premises to remedy such Material Service Failure. In the event of any interruption of HVAC or other utilities that Landlord must provide pursuant to this Lease, regardless of the cause, Landlord shall diligently pursue the restoration of such HVAC and other utilities. Notwithstanding anything in this Lease to the contrary, but subject to Article 24 (which shall govern in the event of a casualty), the provisions of this Section shall be Tenant’s sole recourse and remedy in the event of an interruption of HVAC or other utilities to the Premises, including related to Section 16.8.

16.3 Tenant shall pay for, prior to delinquency of payment therefor, any utilities and services that may be furnished to the Premises during or, if Tenant occupies the Premises after the expiration or earlier termination of the Term, after the Term, beyond those utilities provided by Landlord, including telephone, internet service, cable television and other telecommunications, together with any fees, surcharges and taxes thereon. Upon Landlord’s demand, utilities and services provided to the Premises that are separately metered shall be paid by Tenant directly to the supplier of such utilities or services.

16.4 Tenant shall not, without Landlord’s prior written consent, use any device in the Premises (including data processing machines) that will in any way (a) increase the amount of ventilation, air exchange, gas, steam, electricity or water required or consumed in the Premises based upon Tenant’s Pro Rata Share of the Building or Project (as applicable) beyond the existing capacity of the Building or the Project usually furnished or supplied for the Permitted Use or (b) exceed Tenant’s Pro Rata Share of the Building’s or Project’s (as applicable) capacity to provide such utilities or services. Notwithstanding anything to the contrary set forth herein, Tenant shall have the right to use Air Handling Unit 4-1, Air Handling Unit 4-2, and Air Handling Unit 4-5, each exclusively serving the Premises, and Tenant shall not exceed the capacity of the aforementioned air handling units.

16.5 If Tenant shall require utilities or services in excess of those usually furnished or supplied for tenants in similar spaces in the Building or the Project by reason of Tenant’s equipment or extended hours of business operations, then Tenant shall first procure Landlord’s consent for the use thereof, which consent Landlord may condition upon the availability of such excess utilities or services, and Tenant shall pay as Additional Rent an amount equal to the cost of providing such excess utilities and services.

16.6 Landlord shall provide water in the Common Area for lavatory and landscaping purposes only, which water shall be from the local municipal or similar source; provided, however, that if Landlord determines that Tenant requires, uses or consumes water provided to the Common Area for any purpose other than ordinary lavatory purposes, Landlord may install a water meter ("Tenant Water Meter") and thereby measure Tenant's water consumption for all purposes. Tenant shall pay Landlord for the costs of any Tenant Water Meter and the installation and maintenance thereof during the Term. If Landlord installs a Tenant Water Meter, Tenant shall pay for water consumed by Tenant, as shown on such meter, as and when bills are rendered. If Tenant fails to timely make such payments, Landlord may pay such charges and collect the same from Tenant. Any such costs or expenses incurred or payments made by Landlord for any of the reasons or purposes stated in this Section shall be deemed to be Additional Rent payable by Tenant and collectible by Landlord as such.

16.7 Landlord reserves the right to stop service of the elevator, plumbing, ventilation, air conditioning and utility systems, when Landlord reasonably deems necessary or desirable, due to accident, emergency or the need to make repairs, alterations or improvements, until such repairs, alterations or improvements shall have been completed, and, except as provided in Section 16.2, Landlord shall further have no responsibility or liability for failure to supply elevator facilities, plumbing, ventilation, air conditioning or utility service when prevented from doing so by Force Majeure or, to the extent permitted by Applicable Laws, Landlord's negligence. Without limiting the foregoing, it is expressly understood and agreed that any covenants on Landlord's part to furnish any service pursuant to any of the terms, covenants, conditions, provisions or agreements of this Lease, or to perform any act or thing for the benefit of Tenant, shall not be deemed breached if Landlord is unable to furnish or perform the same by virtue of Force Majeure or, to the extent permitted by Applicable Laws, Landlord's negligence.

16.8 For the Premises, Landlord shall (a) maintain and operate the HVAC systems (not including supplemental units installed by Tenant) used for the Permitted Use only ("Base HVAC") and (b) furnish HVAC as reasonably required (except as this Lease otherwise provides or as to any special requirements that arise from Tenant's particular use of the Premises) for reasonably comfortable occupancy of the Premises twenty-four (24) hours a day, every day during the Term, subject to casualty, eminent domain or as otherwise specified in this Article. Notwithstanding anything to the contrary in this Section, Landlord shall have no liability, and Tenant shall have no right or remedy, on account of any interruption or impairment in HVAC services except as provided in Section 16.2.

16.9 For any utilities serving the Premises for which Tenant is billed directly by such utility provider, Tenant agrees to furnish to Landlord (a) any invoices or statements for such utilities within thirty (30) days after Tenant's receipt thereof, (b) within thirty (30) days after Landlord's request, any other utility usage information reasonably requested by Landlord, and (c) within thirty (30) days after each calendar year during the Term, authorization to allow Landlord to access Tenant's usage information necessary for Landlord to complete an ENERGY STAR® Statement of Performance (or similar comprehensive utility usage report (e.g., related to Labs 21), if requested by Landlord) and any other information in Tenant's possession reasonably requested by Landlord for the immediately preceding year; and Tenant shall comply with any other energy usage or consumption requirements required by Applicable Laws. Tenant shall retain records of utility usage at the Premises, including invoices and statements from the utility provider, for at



least sixty (60) months, or such other period of time as may be requested by Landlord. Tenant acknowledges that any utility information for the Premises, the Building and the Project may be shared with third parties, including Landlord's consultants and Governmental Authorities. In the event that Tenant fails to comply with this Section, Tenant hereby authorizes Landlord to collect utility usage information directly from the applicable utility providers, and Tenant shall pay Landlord a fee of Five Hundred Dollars (\$500) per month to collect such utility usage information. In addition to the foregoing, Tenant shall comply with all Applicable Laws related to the disclosure and tracking of energy consumption at the Premises. The provisions of this Section shall survive the expiration or earlier termination of this Lease.

16.10 Tenant, at its sole cost and expense, and subject to the terms and provisions of Article 17 of this Lease, may install a separate acid neutralization tank (the "Acid Neutralization Tank") in the portion of the Premises located on the first floor of the Building, as shown on Exhibit A. In connection with the installation of the Acid Neutralization Tank, Tenant may connect to the Building's common laboratory waste sanitary sewer connection and to the municipal sewer line in the street adjacent to the Building. Tenant, at its sole cost and expense, shall be responsible for obtaining, and complying with at all times, the MWRA Permit and any other permits and approvals from Governmental Authorities necessary to install, use or operate the Acid Neutralization Tank, and Tenant may not operate the Acid Neutralization Tank without first having provided to Landlord, for Landlord's approval, copies of all such permits and approvals. Tenant shall be responsible for all costs, charges and expenses in connection with or arising out of the operation, use, maintenance, repair or refurbishment of the Acid Neutralization Tank, including all clean-up costs relating to the Acid Neutralization Tank. Tenant shall Indemnify the Landlord Indemnitees from and against any and all Claims, including (a) diminution in value of the Project or any portion thereof, (b) damages for the loss or restriction on use of rentable or usable space or of any amenity of the Project, (c) damages arising from any adverse impact on marketing of space in the Project or any portion thereof and (d) sums paid in settlement of Claims that arise during or after the Term as a result of Tenant's improper use of the Acid Neutralization Tank. This Indemnification by Tenant includes costs incurred in connection with any investigation of site conditions or any clean-up, remediation, removal or restoration required by any Governmental Authority arising from Tenant's use of the Acid Neutralization Tank.

16.11 Subject to each and every term and provision of this Lease (including reasonable closures for repairs or maintenance pursuant to the terms of this Lease), and subject to reasonable closures as the result of casualty, condemnation, emergencies or other circumstances beyond Landlord's control, Tenant shall have the right to access the Premises twenty-four (24) hours per day, seven (7) days per week.

## 17. Alterations.

17.1 Tenant shall make no alterations, additions or improvements in or to the Premises or engage in any construction, demolition, reconstruction, renovation or other work (whether major or minor) of any kind in, at or serving the Premises ("Alterations") without Landlord's prior written approval, which approval may be subject to the consent of one or more Lenders, if required under any applicable Loan Document, but which approval Landlord shall not otherwise unreasonably withhold; provided, however, that, in the event any proposed Alteration affects (a) any structural portions of the Building, including exterior walls, the roof, the foundation or slab, foundation or

slab systems (including barriers and subslab systems) or the core of the Building, (b) the exterior of the Building or (c) any Building systems, including elevator, plumbing, HVAC, electrical, security, life safety and power, then Landlord may withhold its approval in its sole and absolute discretion. Tenant shall, in making any Alterations, use only those architects, contractors, suppliers and mechanics of which Landlord has given prior written approval, which approval shall be in Landlord's reasonable discretion. In seeking Landlord's approval, Tenant shall provide Landlord, at least thirty (30) days in advance of the proposed construction, with plans, specifications, bid proposals, certified stamped engineering drawings and calculations by Tenant's engineer of record or architect of record (including connections to the Building's structural system, modifications to the Building's envelope, non-structural penetrations in slabs or walls, and modifications or tie-ins to life safety systems), work contracts, requests for laydown areas and such other information concerning the nature and cost of the Alterations as Landlord may reasonably request, provided that Tenant shall not commence any such Alterations that require Landlord's consent unless and until Tenant has received the written approval of Landlord and any and all Lenders whose consent is required under any applicable Loan Document. In no event shall Tenant use or Landlord be required to approve any architects, consultants, contractors, subcontractors or material suppliers that Landlord reasonably believes could cause labor disharmony or may not have sufficient experience, in Landlord's reasonable opinion, to perform work in an occupied Class "A" laboratory research building and in tenant-occupied lab areas. Notwithstanding the foregoing, Tenant may make strictly cosmetic changes to the Premises that do not require any permits or more than three (3) total contractors and subcontractors ("Cosmetic Alterations") without Landlord's consent; provided that (y) the cost of any Cosmetic Alterations does not exceed Eighty-Five Thousand Dollars (\$85,000.00) in any one instance or Three Hundred Fifty Thousand Dollars (\$350,000.00) annually, (z) such Cosmetic Alterations do not (i) require any structural or other substantial modifications to the Premises, (ii) require any changes to or adversely affect the Building systems, (iii) affect the exterior of the Building, or (iv) trigger any requirement under Applicable Laws that would require Landlord to make any alteration or improvement to the Premises, the Building or the Project. Tenant shall give Landlord at least fifteen (15) days' prior written notice of any Cosmetic Alterations. Notwithstanding anything in this Article 17 to the contrary, the installation of the Acid Neutralization Tank shall not be deemed a Cosmetic Alteration, irrespective of cost.

17.2 Tenant shall not construct or permit to be constructed partitions or other obstructions that might interfere with free access to mechanical installation or service facilities of the Building or with other tenants' components located within the Building, or interfere with the moving of Landlord's equipment to or from the enclosures containing such installations or facilities.

17.3 Tenant shall accomplish any work performed on the Premises or the Building in such a manner as to permit any life safety systems to remain fully operable at all times.

17.4 Any work performed on the Premises, the Building or the Project by Tenant or Tenant's contractors shall be done at such times and in such manner as Landlord may from time to time designate. Tenant covenants and agrees that all work done by Tenant or Tenant's contractors shall be performed in full compliance with Applicable Laws. Within thirty (30) days after completion of any Alterations (other than Cosmetic Alterations, unless requested in advance by Landlord), Tenant shall provide Landlord with complete "as built" drawing print sets and

electronic CADD files on disc (or files in such other current format in common use as Landlord reasonably approves or requires) showing any changes in the Premises, as well as a commissioning report prepared by a licensed, qualified commissioning agent hired by Tenant and reasonably approved by Landlord for all new or affected mechanical, electrical and plumbing systems. Any such "as built" plans shall show the applicable Alterations as an overlay on the Building as-built plans; provided that Landlord provides the Building "as built" plans to Tenant.

17.5 Before commencing any Alteration, Tenant shall (a) give Landlord at least thirty (30) days' prior written notice of the proposed commencement of such work and the names and addresses of the persons supply labor or materials therefor so that Landlord may enter the Premises to post and keep posted thereon and therein notices or to take any further action that Landlord may reasonably deem proper for the protection of Landlord's interest in the Project and (b) shall, if required by Landlord, secure, at Tenant's own cost and expense, a completion and lien indemnity bond satisfactory to Landlord for such work.

17.6 Tenant shall repair any damage to the Premises arising from Tenant's removal of any property from the Premises. During any such restoration period, Tenant shall pay Rent to Landlord as provided herein as if such space were otherwise occupied by Tenant. The provisions of this Section shall survive the expiration or earlier termination of this Lease.

17.7 The Premises plus any Alterations; Signage; Tenant Improvements; attached equipment, decorations, fixtures and trade fixtures; movable laboratory casework and related appliances; and other additions and improvements attached to or built into the Premises made by either of the parties (including all floor and wall coverings; paneling; sinks and related plumbing fixtures; laboratory benches; exterior venting fume hoods; walk-in freezers and refrigerators; ductwork; conduits; electrical panels and circuits; attached machinery and equipment; and built-in furniture and cabinets, in each case, together with all additions and accessories thereto), shall (unless, prior to such construction or installation, or in connection with Landlord's consent thereto, Landlord elects otherwise in writing) at all times remain the property of Landlord, shall remain in the Premises and shall (unless, prior to construction or installation thereof, Landlord elects otherwise in writing) be surrendered to Landlord upon the expiration or earlier termination of this Lease. For the avoidance of doubt, the items listed on Exhibit H attached hereto (which Exhibit H may be updated by Tenant from and after the Term Commencement Date, subject to Landlord's reasonable written consent) constitute Tenant's property and shall be removed by Tenant upon the expiration or earlier termination of the Lease.

17.8 Notwithstanding any other provision of this Article to the contrary, in no event shall Tenant remove any improvement from the Premises in which any Lender has a security interest or as to which Landlord contributed payment, including the Tenant Improvements, without Landlord's prior written consent, which consent Landlord may withhold in its sole and absolute discretion.

17.9 If Tenant shall fail to remove any of its property from the Premises prior to the expiration or earlier termination of this Lease, then Landlord may, at its option, remove the same in any manner that Landlord shall choose and store such effects without liability to Tenant for loss thereof or damage thereto, and Tenant shall pay Landlord, upon demand, any costs and expenses incurred due to such removal and storage or Landlord may, at its sole option and without notice to

Tenant, sell such property or any portion thereof at private sale and without legal process for such price as Landlord may obtain and apply the proceeds of such sale against any (a) amounts due by Tenant to Landlord under this Lease and (b) any actual and reasonable expenses incident to the removal, storage and sale of such personal property.

17.10 Except with respect to Cosmetic Alterations, Tenant shall pay to Landlord (upon demand) any out-of-pocket third party costs incurred by Landlord for professional review of any plans or specifications for Alterations that require Landlord's consent. Tenant shall reimburse Landlord for any extra expenses incurred by Landlord by reason of faulty work done by Tenant or its contractors, or by reason of delays arising from such work (other than delays to the extent such delays were caused by Landlord), or by reason of inadequate clean-up.

17.11 Within sixty (60) days after final completion of any Alterations performed by Tenant with respect to the Premises, Tenant shall submit to Landlord documentation showing the amounts expended by Tenant with respect to such Alterations, together with supporting documentation reasonably acceptable to Landlord.

17.12 Tenant shall take, and shall cause its contractors to take, commercially reasonable steps to protect the Premises during the performance of any Alterations, including covering or temporarily removing any window coverings so as to guard against dust, debris or damage.

17.13 Tenant shall require its contractors and subcontractors performing work on the Premises to name Landlord and its affiliates and Lenders as additional insureds on their respective insurance policies.

17.14 Notwithstanding anything to the contrary in this Lease, Landlord and Tenant agree that Landlord shall be permitted to withhold its approval (in its sole and absolute discretion) of any Alteration that converts (office to lab or lab to office, as applicable) the office and lab zones identified on Exhibit A-2 attached hereto.

17.15 With respect to any Alterations related to building management systems ("BMS"), including without limitation, the Tenant Improvements, Tenant shall integrate tenant BMS for the Premises into the base building management system and utilize the same system for all of Tenant's HVAC control requirements. The base building management system is currently operated by Johnson Controls. No alternatives or BACnet protocol will be allowed. Tenant's BMS controls contractor shall be subject to Landlord's approval.

#### 18. Repairs and Maintenance.

18.1 Landlord shall repair and maintain the structural and exterior portions and Common Area of the Building and the Project, including roofing and covering materials; foundations (excluding any architectural slabs, but including any structural slabs); exterior walls; plumbing; fire sprinkler systems (if any); base Building HVAC systems; the HVAC system located within the Premises up to the first damper or isolation valve that serves the Premises (for purposes of clarity, the portion of the HVAC system that includes such first damper or isolation valve and extends into and through the Premises, including any distribution systems and any supplemental HVAC serving the Premises shall not be part of the base building HVAC and shall be Tenant's obligation to maintain and repair pursuant to Section 18.2 hereof); elevators; and base Building electrical systems installed or furnished by Landlord. Notwithstanding the foregoing, Landlord has elected to repair and maintain Air Handling Unit 4-5, which is located outside the Premises but exclusively serves the Premises (the "Landlord AHU").

18.2 Except for services of Landlord, if any, required by Section 18.1, Tenant shall at Tenant's sole cost and expense maintain and keep the Premises (including but not limited to any systems or equipment exclusively serving the Premises other than the Landlord AHU, but excluding the base Building HVAC systems up to the first damper or isolation valve that extends into and serves the Premises) and every part thereof in good condition and repair, damage thereto from ordinary wear and tear excepted, and shall, within ten (10) days after receipt of written notice from Landlord, provide to Landlord any maintenance records that Landlord reasonably requests. Tenant shall, upon the expiration or sooner termination of the Term, surrender the Premises to Landlord in as good a condition as existed when received, ordinary wear and tear excepted (except that, with respect to Alterations, in substantially the same condition as existed on the date such Alterations are substantially completed by Tenant). Upon Landlord's written request, Tenant shall remove all telephone and data systems, writing and equipment from the Premises and repair any damage to the Premises caused thereby. Landlord shall have no obligation to alter, remodel, improve, repair, decorate or paint the Premises or any part thereof.

18.3 Throughout the Term of the Lease, Tenant shall, at Tenant's sole cost and expense, maintain copies of all service contracts, service, repair and maintenance records, and inspection reports on all equipment installed by or maintained by Tenant. Tenant shall, within ten (10) days after receipt of written notice from Landlord, provide to Landlord any maintenance records, service or inspection reports that Landlord reasonably requests.

18.4 Landlord shall not be liable for any failure to make any repairs or to perform any maintenance that is Landlord's obligation pursuant to this Lease unless such failure shall persist for an unreasonable time after Tenant provides Landlord with written notice of the need of such repairs or maintenance. Except as otherwise set forth in Section 31.12 of this Lease, Tenant waives its rights under Applicable Laws now or hereafter in effect to make repairs at Landlord's expense.

18.5 If any excavation shall be made upon land adjacent to or under the Building, or shall be authorized to be made, Tenant shall afford to the person causing or authorized to cause such excavation, license to enter the Premises for the purpose of performing such work as such person shall deem necessary or desirable to preserve and protect the Building from injury or damage and to support the same by proper foundations, without any claim for damages or liability against Landlord and without reducing or otherwise affecting Tenant's obligations under this Lease.

18.6 This Article relates to repairs and maintenance arising in the ordinary course of operation of the Building and the Project. In the event of a casualty described in Article 24, Article 24 shall apply in lieu of this Article. In the event of eminent domain, Article 25 shall apply in lieu of this Article.

18.7 Costs incurred by Landlord pursuant to this Article shall constitute Operating Expenses, subject to the limitations on inclusion of certain costs associated with capital expenditures, as set forth in Section 9.1(c).

19. Liens.

19.1 Subject to the immediately succeeding sentence, Tenant shall keep the Premises, the Building and the Project free from any liens arising out of work or services performed, materials furnished to or obligations incurred by Tenant. Tenant further covenants and agrees that any mechanic's or materialman's lien filed against the Premises, the Building or the Project for work or services claimed to have been done for, or materials claimed to have been furnished to, or obligations incurred by Tenant shall be discharged or bonded by Tenant within ten (10) days after the filing thereof, at Tenant's sole cost and expense.

19.2 Should Tenant fail to discharge or bond against any lien of the nature described in Section 19.1, Landlord may, at Landlord's election, pay such claim or post a statutory lien bond or otherwise provide security to eliminate the lien as a claim against title, and Tenant shall immediately reimburse Landlord for the actual costs thereof as Additional Rent. Tenant shall Indemnify the Landlord Indemnitees from and against any Claims arising from any such liens, including any administrative, court or other legal proceedings related to such liens.

19.3 In the event that Tenant leases or finances the acquisition of office equipment, furnishings or other personal property of a removable nature utilized by Tenant in the operation of Tenant's business, Tenant warrants that any Uniform Commercial Code financing statement shall, upon its face or by exhibit thereto, indicate that such financing statement is applicable only to removable personal property of Tenant located within the Premises. In no event shall the address of the Premises, the Building or the Project be furnished on a financing statement without qualifying language as to applicability of the lien only to removable personal property located in an identified suite leased by Tenant. Should any holder of a financing statement record or place of record a financing statement that appears to constitute a lien against any interest of Landlord or against equipment that may be located other than within an identified suite leased by Tenant, Tenant shall, within ten (10) days after filing such financing statement, cause (a) a copy of the lender security agreement or other documents to which the financing statement pertains to be furnished to Landlord to facilitate Landlord's ability to demonstrate that the lien of such financing statement is not applicable to Landlord's interest and (b) Tenant's lender to amend such financing statement and any other documents of record to clarify that any liens imposed thereby are not applicable to any interest of Landlord in the Premises, the Building or the Project. If requested by Tenant or its lender, Landlord will agree to deliver a written statement to such lender providing that this Lease does not grant to Landlord a security interest in Tenant's personal property.

20. Estoppel Certificate. Tenant shall, within ten (10) business days after receipt of written notice from Landlord, execute, acknowledge and deliver a statement in writing substantially in the form attached to this Lease as Exhibit I, or on any other form reasonably requested by a current or proposed Lender or encumbrancer or proposed purchaser, (a) certifying that this Lease is unmodified and in full force and effect (or, if modified, stating the nature of such modification and certifying that this Lease as so modified is in full force and effect) and the dates to which rental and other charges are paid in advance, if any, (b) acknowledging that there are not, to Tenant's knowledge, any uncured defaults on the part of Landlord hereunder, or specifying such defaults if any are claimed, and (c) setting forth such further information with respect to this Lease or the Premises as may be reasonably requested thereon. Any such statements may be relied upon by any prospective purchaser or encumbrancer of all or any portion of the Property. Tenant's failure

to deliver any such statement within the prescribed time shall, at Landlord's option, constitute a Default (as defined below) under this Lease, and, in any event, shall be binding upon Tenant that the Lease is in full force and effect and without modification except as may be represented by Landlord in any certificate prepared by Landlord and delivered to Tenant for execution.

## 21. Hazardous Materials.

21.1 Tenant shall not cause or permit any Hazardous Materials (as defined below) to be brought upon, kept or used in or about the Premises, the Building or the Project in violation of Applicable Laws by Tenant or any of its employees, agents, contractors or invitees (collectively with Tenant, each a "Tenant Party"). If (a) Tenant breaches such obligation, (b) the presence of Hazardous Materials as a result of such a breach results in contamination of the Project, any portion thereof, or any adjacent property, (c) contamination of the Premises otherwise occurs during the Term or any extension or renewal hereof or holding over hereunder (other than if such contamination results from (i) migration of Hazardous Materials from outside the Premises arising from the acts or omissions of a Tenant Party or coming from property owned or leased by a Tenant Party, or (ii) to the extent such contamination is caused by Landlord's gross negligence or willful misconduct) or (d) contamination of the Project occurs as a result of Hazardous Materials that are placed on or under or are released into the Project by a Tenant Party, then Tenant shall Indemnify the Landlord Indemnitees from and against any and all Claims of any kind or nature, including (w) diminution in value of the Project or any portion thereof, (x) damages for the loss or restriction on use of rentable or usable space or of any amenity of the Project, (y) damages arising from any adverse impact on marketing of space in the Project or any portion thereof and (z) sums paid in settlement of Claims that arise before, during or after the Term as a result of such breach or contamination. This Indemnification by Tenant includes costs incurred in connection with any investigation of site conditions or any clean-up, remedial, removal or restoration work required by any Governmental Authority because of Hazardous Materials present in the air, soil or groundwater above, on, under or about the Project. Without limiting the foregoing, if the presence of any Hazardous Materials in, on, under or about the Project, any portion thereof or any adjacent property caused or permitted by any Tenant Party results in any contamination of the Project, any portion thereof or any adjacent property, then Tenant shall promptly take all actions at its sole cost and expense as are necessary to return the Project, any portion thereof or any adjacent property to its respective condition existing prior to the time of such contamination; provided that Landlord's written approval of such action shall first be obtained, which approval Landlord shall not unreasonably withhold or delay; and provided, further, that it shall be reasonable for Landlord to withhold its consent if such actions could have a material adverse long-term or short-term effect on the Project, any portion thereof or any adjacent property. Tenant's obligations under this Section shall not be affected, reduced or limited by any limitation on the amount or type of damages, compensation or benefits payable by or for Tenant under workers' compensation acts, disability benefit acts, employee benefit acts or similar legislation.

21.2 Landlord acknowledges that it is not the intent of this Article to prohibit Tenant from operating its business for the Permitted Use. Tenant may operate its business according to the custom of Tenant's industry so long as the use or presence of Hazardous Materials is strictly and properly monitored in accordance with Applicable Laws. As a material inducement to Landlord to allow Tenant to use Hazardous Materials in connection with its business, Tenant agrees to deliver to Landlord (a) a list identifying each type of Hazardous Material to be present

at the Premises that is subject to regulation under any environmental Applicable Laws in the form of a Tier II form pursuant to Section 312 of the Emergency Planning and Community Right-to-Know Act of 1986 (or any successor statute) or any other form reasonably requested by Landlord, (b) a list of any and all approvals or permits from Governmental Authorities required in connection with the presence of such Hazardous Material at the Premises and (c) correct and complete copies of (i) notices of violations of Applicable Laws related to Hazardous Materials and (ii) plans relating to the installation of any storage tanks to be installed in, on, under or about the Project (provided that installation of storage tanks shall only be permitted after Landlord has given Tenant its written consent to do so, which consent Landlord may withhold in its sole and absolute discretion) and closure plans or any other documents required by any and all Governmental Authorities for any storage tanks installed in, on, under or about the Project for the closure of any such storage tanks (collectively, "Hazardous Materials Documents"). Tenant shall deliver to Landlord updated Hazardous Materials Documents, within fourteen (14) days after receipt of a written request therefor from Landlord, not more often than once per year, unless (m) there are any changes to the Hazardous Materials Documents or (n) Tenant initiates any Alterations or changes its business, in either case in a way that involves any material increase in the types or amounts of Hazardous Materials, in which case Tenant shall deliver updated Hazardous Materials documents (without Landlord having to request them) before or, if not practicable to do so before, as soon as reasonably practicable after the occurrence of the events in Subsection 21.2(m) or (n). For each type of Hazardous Material listed, the Hazardous Materials Documents shall include (t) the chemical name, (u) the material state (e.g., solid, liquid, gas or cryogen), (v) the concentration, (w) the storage amount and storage condition (e.g., in cabinets or not in cabinets), (x) the use amount and use condition (e.g., open use or closed use), (y) the location (e.g., room number or other identification) and (z) if known, the chemical abstract service number. Notwithstanding anything in this Section to the contrary, Tenant shall not be required to provide Landlord with any documents containing information of a proprietary nature, unless such documents contain a reference to Hazardous Materials or activities related to Hazardous Materials. Landlord may, at Landlord's expense, cause the Hazardous Materials Documents to be reviewed by a person or firm qualified to analyze Hazardous Materials to confirm compliance with the provisions of this Lease and with Applicable Laws. In the event that a review of the Hazardous Materials Documents indicates non-compliance with this Lease or Applicable Laws, Tenant shall, at its expense, diligently take steps to bring its storage and use of Hazardous Materials into compliance. Notwithstanding anything in this Lease to the contrary or Landlord's review into Tenant's Hazardous Materials Documents or use or disposal of hazardous materials, however, Landlord shall not have and expressly disclaims any liability related to Tenant's or other tenants' use or disposal of Hazardous Materials, it being acknowledged by Tenant that Tenant is best suited to evaluate the safety and efficacy of its Hazardous Materials usage and procedures.

21.3 Tenant represents and warrants to Landlord that is not nor has it been, in connection with the use, disposal or storage of Hazardous Materials, (a) subject to a material enforcement order issued by any Governmental Authority or (b) required to take any remedial action.

21.4 At any time, and from time to time, prior to the expiration of the Term, Landlord shall have the right to conduct appropriate tests of the Project or any portion thereof to demonstrate that Hazardous Materials are present or that contamination has occurred due to the acts or omissions of a Tenant Party. Tenant shall pay all reasonable costs of such tests if such tests reveal that Hazardous Materials exist at the Project in violation of this Lease.



21.5 If underground or other storage tanks storing Hazardous Materials installed or utilized by Tenant are located on the Premises, or are hereafter placed on the Premises by Tenant (or by any other party, if such storage tanks are utilized by Tenant), then Tenant shall monitor the storage tanks, maintain appropriate records, implement reporting procedures, properly close any underground storage tanks, and take or cause to be taken all other steps necessary or required under the Applicable Laws. Tenant shall have no responsibility or liability for underground or other storage tanks installed by anyone other than Tenant unless Tenant utilizes such tanks, in which case Tenant's responsibility for such tanks shall be as set forth in this Section.

21.6 Tenant shall promptly report to Landlord any actual or suspected presence of mold or water intrusion at the Premises.

21.7 Tenant's obligations under this Article shall survive the expiration or earlier termination of the Lease. During any period of time needed by Tenant or Landlord after the termination of this Lease to complete the removal from the Premises of any such Hazardous Materials for which Tenant is responsible under this Lease, Tenant shall be deemed a holdover tenant and subject to the provisions of Article 27.

21.8 As used herein, the term "Hazardous Material" means any toxic, explosive, corrosive, flammable, infectious, radioactive, carcinogenic, mutagenic or otherwise hazardous substance, material or waste that is or becomes regulated by Applicable Laws or any Governmental Authority.

21.9 Notwithstanding anything to the contrary in this Lease, Landlord shall have sole control over the allocation of fire control areas (as defined in the Uniform Building Code as adopted by the city or municipality(ies) in which the Project is located (the "UBC")) within the Project for the storage of Hazardous Materials. Tenant shall be allocated (a) one (1) control area within the Fourth Floor Premises, and (b) one (1) control area within the First Floor Premises, and the boundaries of each control area shall be at Tenant's discretion. Notwithstanding anything to the contrary in this Lease, the quantity of Hazardous Materials allowed by this Section is specific to Tenant and shall not run with the Lease in the event of a Transfer (as defined in Article 29). In the event of a Transfer, if the use of Hazardous Materials by such new tenant ("New Tenant") is such that New Tenant utilizes fire control areas in the Project in excess of the area allocated by Landlord, then New Tenant shall, at its sole cost and expense and upon Landlord's written request, establish and maintain a separate area of the Premises classified by the UBC as an "H" occupancy area for the use and storage of Hazardous Materials, or take such other action as is necessary to ensure that its share of the fire control areas of the Building and the Project is not greater than the area allocated by Landlord. Notwithstanding anything in this Lease to the contrary, Landlord shall not have and expressly disclaims any liability related to Tenant's or other tenants' use or disposal of fire control areas, it being acknowledged by Tenant that Tenant and other tenants are best suited to evaluate the safety and efficacy of its Hazardous Materials usage and procedures.

22. Odors and Exhaust. Tenant acknowledges that Landlord would not enter into this Lease with Tenant unless Tenant assured Landlord that under no circumstances will any other occupants of the Building or the Project (including persons legally present in any outdoor areas of the Project) be subjected to odors or fumes (whether or not noxious), and that the Building and the Project will not be damaged by any exhaust, in each case from Tenant's operations. Landlord and Tenant therefore agree as follows:

22.1 Tenant shall not cause or permit (or conduct any activities that would cause) any release of any odors or fumes of any kind from the Premises.

22.2 If the Building has a ventilation system that, in Landlord's judgment, is adequate, suitable, and appropriate to vent the Premises in a manner that does not release odors affecting any indoor or outdoor part of the Project, Tenant shall vent the Premises through such system. If Landlord at any time determines that any existing ventilation system is inadequate, or if no ventilation system exists, Tenant shall in compliance with Applicable Laws vent all fumes and odors from the Premises (and remove odors from Tenant's exhaust stream) as Landlord requires. The placement and configuration of all ventilation exhaust pipes, louvers and other equipment shall be subject to Landlord's approval. Tenant acknowledges Landlord's legitimate desire to maintain the Project (indoor and outdoor areas) in an odor-free manner, and Landlord may require Tenant, consistent with Landlord's non-discriminatory requirements for the Building, to abate and remove all odors in a manner that goes beyond the requirements of Applicable Laws.

22.3 Tenant shall, at Tenant's sole cost and expense, provide odor eliminators and other devices (such as filters, air cleaners, scrubbers and whatever other equipment may in Landlord's judgment be necessary or appropriate from time to time) to completely remove, eliminate and abate any odors, fumes or other substances in Tenant's exhaust stream that, in Landlord's judgment, emanate from Tenant's Premises. Any work Tenant performs under this Section shall constitute Alterations.

22.4 Tenant's responsibility to remove, eliminate and abate odors, fumes and exhaust shall continue throughout the Term. Landlord's approval of the Tenant Improvements shall not preclude Landlord from requiring additional measures to eliminate odors, fumes and other adverse impacts of Tenant's exhaust stream (as Landlord may reasonably designate in Landlord's discretion). Tenant shall install additional equipment as Landlord reasonably requires from time to time under the preceding sentence. Such installations shall constitute Alterations. Tenant shall have no obligation or liabilities for odors, fumes or exhaust arising or emanating from portions of the Project that are not the Premises unless arising from the actions or omissions of Tenant or another Tenant Party.

22.5 If Tenant fails to install satisfactory odor control equipment within ten (10) business days after Landlord's demand made at any time, then Landlord may, without limiting Landlord's other rights and remedies, require Tenant to cease and suspend any operations in the Premises that, in Landlord's reasonable determination, cause odors, fumes or exhaust. For the purpose of the immediately foregoing sentence, Landlord's determination shall be "reasonable" if Landlord has received a complaint regarding such odors, fumes or exhaust. For example, if Landlord determines that Tenant's production of a certain type of product causes odors, fumes or exhaust, and Tenant does not install satisfactory odor control equipment within ten (10) business days after Landlord's request, then Landlord may require Tenant to stop producing such type of product in the Premises unless and until Tenant has installed odor control equipment satisfactory to Landlord.

23. Insurance.

23.1 Landlord shall maintain insurance for the Building and the Project in amounts equal to full replacement cost (exclusive of the costs of excavation, foundations and footings, engineering costs or such other costs to the extent the same are not incurred in the event of a rebuild and without reference to depreciation taken by Landlord upon its books or tax returns) or such lesser coverage as Landlord may elect, provided that such coverage shall not be less than the amount of such insurance Landlord's Lender, if any, requires Landlord to maintain, providing protection against any peril generally included within the classification "Fire and Extended Coverage," together with insurance against sprinkler damage (if applicable), vandalism and malicious mischief. Landlord, subject to availability thereof, shall further insure, if Landlord deems it appropriate, coverage against flood, environmental hazard, earthquake, loss or failure of building equipment, rental loss during the period of repairs or rebuilding, Workers' Compensation insurance and fidelity bonds for employees employed to perform services. Notwithstanding the foregoing, Landlord may, but shall not be deemed required to, provide insurance for any improvements installed by Tenant or that are in addition to the standard improvements customarily furnished by Landlord, without regard to whether or not such are made a part of or are affixed to the Building.

23.2 In addition, Landlord shall carry Commercial General Liability insurance with combined single limits of not less than One Million Dollars (\$1,000,000) per occurrence/general aggregate and an umbrella policy of not less than Five Million Dollars (\$5,000,000) for bodily injury (including death), or property damage with respect to the Project, written on an occurrence basis; provided that such coverage is at least as broad as the primary coverages required herein.

23.3 Tenant shall, at its own cost and expense, procure and maintain during the Term the following insurance for the benefit of Tenant and Landlord (as their interests may appear) with insurers financially acceptable and lawfully authorized to do business in the state where the Premises are located:

(a) Commercial General Liability insurance on a broad-based occurrence coverage form, with coverages including but not limited to bodily injury (including death), property damage (including loss of use resulting therefrom), premises/operations, personal & advertising injury, and contractual liability with limits of liability of not less than \$2,000,000 for bodily injury and property damage per occurrence, \$2,000,000 general aggregate, which limits may be met by use of excess and/or umbrella liability insurance provided that such coverage is at least as broad as the primary coverages required herein.

(b) Commercial Automobile Liability insurance covering liability arising from the use or operation of any auto on behalf of Tenant or invited by Tenant (including those owned, hired, rented, leased, borrowed, scheduled or non-owned). Coverage shall be on a broad-based occurrence form in an amount not less than \$2,000,000 combined single limit per accident for bodily injury and property damage. Such coverage shall apply to all vehicles and persons, whether accessing the property with active or passive consent.

(c) Commercial Property insurance covering property damage to the full replacement cost value and business interruption. Covered property shall include all tenant improvements in the Premises (to the extent not insured by Landlord pursuant to Section 23.1) and Tenant's Property including personal property, furniture, fixtures, machinery, equipment, stock, inventory and improvements and betterments, which may be owned by Tenant or Landlord and required to be insured hereunder, or which may be leased, rented, borrowed or in the care custody or control of Tenant, or Tenant's agents, employees or subcontractors. Such insurance, with respect only to all Tenant Improvements, Alterations or other work performed on the Premises by Tenant (collectively, "Tenant Work"), shall name Landlord and Landlord's current and future mortgagees as loss payees as their interests may appear. Such insurance shall be written on an "all risk" of physical loss or damage basis including the perils of fire, extended coverage, electrical injury, mechanical breakdown, windstorm, vandalism, malicious mischief, sprinkler leakage, back-up of sewers or drains, terrorism and such other risks Landlord may from time to time designate, for the full replacement cost value of the covered items with an agreed amount endorsement with no co-insurance. Business interruption coverage shall have limits sufficient to cover Tenant's necessary continuing expenses, including rents due Landlord under the Lease. The minimum period of indemnity for business interruption coverage shall be twelve (12) months, plus twelve (12) months' extended period of indemnity.

(d) Workers' Compensation insurance as is required by statute or law, or as may be available on a voluntary basis and Employers' Liability insurance with limits of not less than the following: each accident, Five Hundred Thousand Dollars (\$500,000); disease (\$500,000); disease (each employee), Five Hundred Thousand Dollars (\$500,000).

(e) Medical malpractice insurance at limits of not less than \$1,000,000 each claim during such periods, if any, that Tenant engages in the practice of medicine at the Premises or conducts clinical trials on human subjects at the Premises.

(f) During all construction by Tenant at the Premises, with respect to tenant improvements being constructed (including the Tenant Improvements and any Alterations, insurance required in Exhibit B-1 must be in place.

23.4 The insurance required of Tenant by this Article shall be with companies at all times having a current rating of not less than A- and financial category rating of at least Class VII in "A.M. Best's Insurance Guide" current edition. Tenant shall obtain for Landlord from the insurance companies/broker or cause the insurance companies/broker to furnish certificates of insurance evidencing all coverages required herein to Landlord. Landlord reserves the right to require complete, certified copies of all required insurance policies including any endorsements. No such policy shall be cancelable or subject to reduction of coverage or other modification or cancellation except after thirty (30) days' prior written notice to Landlord from Tenant or its insurers (except in the event of non-payment of premium, in which case ten (10) days' written notice shall be given). All such policies shall be written as primary policies, not contributing with and not in excess of the coverage that Landlord may carry. Tenant's required policies shall contain severability of interests clauses stating that, except with respect to limits of insurance, coverage shall apply separately to each insured or additional insured. Tenant shall, on the date of expiration of such policies, furnish Landlord with renewal certificates of insurance or binders. Tenant agrees that if Tenant does not take out and maintain such insurance and such failure continues for five (5) business days after written notice to Tenant, Landlord may (but shall not be required to) procure such insurance on Tenant's behalf and at its cost to be paid by Tenant as Additional Rent.

Commercial General Liability, Commercial Automobile Liability, Umbrella Liability and Pollution Legal Liability insurance as required above shall name Landlord, BioMed Realty LLC, BioMed Realty, L.P., BRE Edison L.P., BRE Edison LLC, BRE Edison Holdings L.P., BRE Edison Holdings LLC, and BRE Edison Parent L.P. and their respective officers, employees, agents, general partners, members, subsidiaries, affiliates and Lenders ("Landlord Parties") as additional insureds as respects liability arising from work or operations performed by or on behalf of Tenant, Tenant's use or occupancy of Premises, and ownership, maintenance or use of vehicles by or on behalf of Tenant.

23.5 In each instance where insurance is to name Landlord Parties as additional insureds, Tenant shall, upon Landlord's written request, also designate and furnish certificates evidencing such Landlord Parties as additional insureds to (a) any Lender of Landlord holding a security interest in the Building or the Project, (b) the landlord under any lease whereunder Landlord is a tenant of the real property upon which the Building is located if the interest of Landlord is or shall become that of a tenant under a ground lease rather than that of a fee owner and (c) any management company retained by Landlord to manage the Project.

23.6 Subject to Section 23.7 below, Tenant assumes the risk of damage to any fixtures, goods, inventory, merchandise, equipment and leasehold improvements, and Landlord shall not be liable for injury to Tenant's business or any loss of income therefrom, relative to such damage, all as more particularly set forth within this Lease. Tenant shall, at Tenant's sole cost and expense, carry such insurance as Tenant desires for Tenant's protection with respect to personal property of Tenant or business interruption.

23.7 Tenant, on behalf of itself and its insurers, hereby waives any and all rights of recovery against the Landlord Parties with respect to any loss, damage, claims, suits or demands, howsoever caused, that are covered, or should have been covered, by valid and collectible workers' compensation, employer's liability insurance and other liability insurance required to be obtained and carried by Tenant pursuant to this Article, including any deductibles or self-insurance maintained thereunder. Tenant agrees to endorse the required workers' compensation, employer's liability and other liability insurance policies to permit waivers of subrogation as required hereunder and hold harmless and indemnify the Landlord Parties for any loss or expense incurred as a result of a failure to obtain such waivers of subrogation from insurers. Such waivers shall continue so long as Tenant's insurers so permit. Any termination of such a waiver shall be by written notice to Landlord, containing a description of the circumstances hereinafter set forth in this Section. Tenant, upon obtaining the policies of workers' compensation, employer's liability and other liability insurance required or permitted under this Lease, shall give notice to its insurance carriers that the foregoing waiver of subrogation is contained in this Lease. If such policies shall not be obtainable with such waiver or shall be so obtainable only at a premium over that chargeable without such waiver, then Tenant shall notify Landlord of such conditions. In addition, each of Landlord and Tenant, on behalf of itself and its insurers, hereby waives all rights of subrogation against the other party or such other party's insurers with respect to any Claims covered by any other insurance policies required to be obtained and maintained by the non-waiving party pursuant to this Lease, or that would have been covered had the non-waiving party obtained and maintained such policies, except to the extent of the non-waiving party's willful misconduct.

23.8 Landlord may require insurance policy limits required under this Lease to be raised to conform with requirements of Landlord's Lender or to bring coverage limits to levels then being required of new tenants within the Project.

23.9 Any costs incurred by Landlord pursuant to this Article shall constitute a portion of Operating Expenses.

23.10 The provisions of this Article 23 shall survive the expiration or earlier termination of this Lease.

24. Damage or Destruction.

24.1 In the event of a partial destruction of (a) the Premises or (b) the Common Area of the Building or the Project ((a) and (b) together, the "Affected Areas") by fire or other perils covered by extended coverage insurance not exceeding twenty-five percent (25%) of the full insurable value thereof, and provided that (w) the damage thereto is such that the Affected Areas may be repaired, reconstructed or restored within a period of twelve (12) months from the date of the happening of such casualty, (x) Landlord shall receive insurance proceeds from its insurer or Lender sufficient to cover the cost of such repairs, reconstruction and restoration (except for any deductible amount provided by Landlord's policy, which deductible amount, if paid by Landlord, shall constitute an Operating Expense), (y) the repair, reconstruction or restoration of the Affected Areas is permitted by all applicable Loan Documents or otherwise consented to by any and all Lenders whose consent is required thereunder, and (z) such casualty was not intentionally caused by a Tenant Party, then Landlord shall commence and proceed diligently with the work of repair, reconstruction and restoration of the Affected Areas (including all Tenant Improvements) and this Lease shall continue in full force and effect.

24.2 In the event of any damage to or destruction of the Building or the Project other than as described in Section 24.1, Landlord may elect to repair, reconstruct and restore the Building or the Project, as applicable, in which case this Lease shall continue in full force and effect. If Landlord elects not to repair, reconstruct and restore the Building or the Project, as applicable, then this Lease shall terminate as of the date of such damage or destruction. In the event of any damage or destruction (regardless of whether such damage is governed by Section 24.1 or this Section), if (a) in Landlord's determination as set forth in the Damage Repair Estimate (as defined below), the Affected Areas cannot be repaired, reconstructed or restored within twelve (12) months after the date of the Damage Repair Estimate, (b) subject to Section 24.6, the Affected Areas are not actually repaired, reconstructed and restored within eighteen (18) months after the date of the Damage Repair Estimate, or (c) the damage and destruction occurs within the last twelve (12) months of the then-current Term, then Tenant shall have the right to terminate this Lease, effective as of the date of such damage or destruction, by delivering to Landlord its written notice of termination (a "Termination Notice") (y) with respect to Subsections 24.2(a) and (c), no later than fifteen (15) days after Landlord delivers to Tenant Landlord's Damage Repair Estimate and (z) with respect to Subsection 24.2(b), no later than fifteen (15) days after such twelve (12) month period (as the same may be extended pursuant to Section 24.6) expires. If Tenant provides Landlord with a Termination Notice pursuant to Subsection 24.2(z), Landlord shall have an additional thirty (30) days after receipt of such Termination Notice to complete the repair, reconstruction and restoration. If Landlord does not complete such repair, reconstruction and

restoration within such thirty (30) day period, then Tenant may terminate this Lease by giving Landlord written notice within two (2) business days after the expiration of such thirty (30) day period. If Landlord does complete such repair, reconstruction and restoration within such thirty (30) day period, then this Lease shall continue in full force and effect.

24.3 As soon as reasonably practicable, but in any event within sixty (60) days following the date of damage or destruction, Landlord shall notify Tenant of Landlord's good faith estimate of the period of time in which the repairs, reconstruction and restoration will be completed (the "Damage Repair Estimate"), which estimate shall be based upon the opinion of a contractor reasonably selected by Landlord and experienced in comparable repair, reconstruction and restoration of similar buildings. Additionally, Landlord shall give written notice to Tenant within sixty (60) days following the date of damage or destruction of its election not to repair, reconstruct or restore the Building or the Project, as applicable.

24.4 Upon any termination of this Lease under any of the provisions of this Article, the parties shall be released thereby without further obligation to the other from the date possession of the Premises is surrendered to Landlord, except with regard to (a) items occurring prior to the damage or destruction and (b) provisions of this Lease that, by their express terms, survive the expiration or earlier termination hereof.

24.5 In the event of repair, reconstruction and restoration as provided in this Article, all Rent to be paid by Tenant under this Lease shall be abated proportionately based on the extent to which Tenant's use of the Premises is impaired during the period of such repair, reconstruction or restoration, unless Landlord provides Tenant with other space during the period of repair, reconstruction and restoration that, in Tenant's reasonable opinion, is suitable for the temporary conduct of Tenant's business; provided, however, that the amount of such abatement shall be reduced by the amount of Rent that is received by Tenant as part of the business interruption or loss of rental income with respect to the Premises from the proceeds of business interruption or loss of rental income insurance.

24.6 Notwithstanding anything to the contrary contained in this Article, (a) Landlord shall not be required to repair, reconstruct or restore any damage or destruction to the extent that Landlord is prohibited from doing so by any applicable Loan Document or any Lender whose consent is required thereunder withholds its consent, and (b) should Landlord be delayed or prevented from completing the repair, reconstruction or restoration of the damage or destruction to the Premises after the occurrence of such damage or destruction by Force Majeure or delays caused by a Lender or Tenant Party, then the time for Landlord to commence or complete repairs, reconstruction and restoration shall be extended on a day-for-day basis; provided, however, that, at Landlord's election, Landlord shall be relieved of its obligation to make such repairs, reconstruction and restoration.

24.7 If Landlord is obligated to or elects to repair, reconstruct or restore as herein provided, then Landlord shall be obligated to make such repairs, reconstruction or restoration only with regard to (a) those portions of the Premises that were originally provided at Landlord's expense and (b) the Common Area portion of the Affected Areas. The repairs, reconstruction or restoration of improvements not originally provided by Landlord or at Landlord's expense shall be the obligation of Tenant. In the event Tenant has elected to upgrade certain improvements from

Landlord's building standards (the "Building Standard"), Landlord shall, upon the need for replacement due to an insured loss, provide only the Building Standard, unless Tenant again elects to upgrade such improvements and pay any incremental costs related thereto, except to the extent that excess insurance proceeds, if received, are adequate to provide such upgrades, in addition to providing for basic repairs, reconstruction and restoration of the Premises, the Building and the Project.

24.8 Notwithstanding anything to the contrary contained in this Article, Landlord shall not have any obligation whatsoever to repair, reconstruct or restore the Premises if the damage resulting from any casualty covered under this Article occurs (a) during the thirteenth (13th) through the twenty-fourth (24th) months prior to the expiration of the Term and the Damage Repair Estimate indicates that more than six (6) months will be required for such repair, reconstruction or restoration, (b) during the seventh (7th) through twelfth (12th) months prior to the expiration of the Term and the Damage Repair Estimate indicates that more than thirty (30) days will be required for such repair, reconstruction or restoration, (c) during the last six (6) months of the Term or (d) to the extent that insurance proceeds are not available therefor.

24.9 Landlord's obligation, should it elect or be obligated to repair, reconstruct or restore, shall be limited to the Affected Areas, and shall be conditioned upon Landlord receiving any permits or authorizations required by Applicable Laws. Tenant shall, at its expense, replace or fully repair all of Tenant's personal property and any Alterations installed by Tenant existing at the time of such damage or destruction. If Affected Areas are to be repaired, reconstructed or restored in accordance with the foregoing, Landlord shall make available to Tenant any portion of insurance proceeds it receives that are allocable to the Alterations constructed by Tenant pursuant to this Lease; provided that Landlord shall not be required to do so while Tenant is in default under this Lease, and subject to the requirements of any Lender of Landlord.

24.10 This Article sets forth the terms and conditions upon which this Lease may terminate in the event of any damage or destruction. Accordingly, the parties hereby waive the provisions of any Applicable Laws (and any successor statutes) permitting the parties to terminate this Lease as a result of any damage or destruction.

## 25. Eminent Domain.

25.1 In the event (a) the whole of all Affected Areas or (b) such part thereof as shall substantially interfere with Tenant's use and occupancy of the Premises for the Permitted Use shall be taken for any public or quasi-public purpose by any lawful power or authority by exercise of the right of appropriation, condemnation or eminent domain, or sold to prevent such taking, Tenant or Landlord may terminate this Lease effective as of the date possession is required to be surrendered to such authority, except with regard to (y) items occurring prior to the taking and (z) provisions of this Lease that, by their express terms, survive the expiration or earlier termination hereof.

25.2 In the event of a partial taking of (a) the Building or the Project or (b) drives, walkways or parking areas serving the Building or the Project for any public or quasi-public purpose by any lawful power or authority by exercise of right of appropriation, condemnation, or eminent domain, or sold to prevent such taking, then, without regard to whether any portion of the



Premises occupied by Tenant was so taken, Landlord may elect to terminate this Lease (except with regard to (y) items occurring prior to the taking and (z) provisions of this Lease that, by their express terms, survive the expiration or earlier termination hereof) as of such taking if such taking is, in Landlord's sole opinion, of a material nature such as to make it uneconomical to continue use of the unappropriated portion for purposes of renting office or laboratory space.

25.3 To the extent permitted under all applicable Loan Documents or otherwise consented to by any and all Lenders whose consent is required thereunder, Tenant shall be entitled to any award that is specifically awarded as compensation for (a) the taking of Tenant's personal property that was installed at Tenant's expense and (b) the costs of Tenant moving to a new location. Except as set forth in the previous sentence, any award for such taking shall be the property of Landlord.

25.4 If, upon any taking of the nature described in this Article, this Lease continues in effect, then Landlord shall promptly proceed to restore the Affected Areas to substantially their same condition prior to such partial taking. To the extent such restoration is infeasible, as determined by Landlord in its sole and absolute discretion, the Rent shall be decreased proportionately to reflect the loss of any portion of the Premises no longer available to Tenant. Notwithstanding anything to the contrary contained in this Article, Landlord shall not be required to restore the Affected Areas to the extent that Landlord is prohibited from doing so by any applicable Loan Document or any Lender whose consent is required thereunder withholds its consent.

25.5 This Article sets forth the terms and conditions upon which this Lease may terminate in the event of any damage or destruction. Accordingly, the parties hereby waive the provisions of any Applicable Laws (and any successor statutes) permitting the parties to terminate this Lease as a result of any damage or destruction.

## 26. Surrender.

26.1 At least thirty (30) days prior to Tenant's surrender of possession of any part of the Premises, Tenant shall provide Landlord with a facility decommissioning and Hazardous Materials closure plan for the Premises ("Exit Survey.") prepared by an independent third party state-certified professional with appropriate expertise, which Exit Survey must be reasonably acceptable to Landlord. The Exit Survey shall comply with the American National Standards Institute's Laboratory Decommissioning guidelines (ANSI/AIHA Z9.11-2008) or any successor standards published by ANSI or any successor organization (or, if ANSI and its successors no longer exist, a similar entity publishing similar standards). In addition, at least ten (10) days prior to Tenant's surrender of possession of any part of the Premises, Tenant shall (a) provide Landlord with written evidence of all appropriate governmental releases obtained by Tenant in accordance with Applicable Laws, including laws pertaining to the surrender of the Premises, (b) place Laboratory Equipment Decontamination Forms on all decommissioned equipment to assure safe occupancy by future users and (c) conduct a site inspection with Landlord. In addition, Tenant agrees to remain responsible after the surrender of the Premises for the remediation of any recognized environmental conditions set forth in the Exit Survey and comply with any recommendations set forth in the Exit Survey. Tenant's obligations under this Section shall survive the expiration or earlier termination of the Lease.

26.2 No surrender of possession of any part of the Premises shall release Tenant from any of its obligations hereunder, unless such surrender is accepted in writing by Landlord.

26.3 The voluntary or other surrender of this Lease by Tenant shall not effect a merger with Landlord's fee title or leasehold interest in the Premises, the Building, the Property or the Project, unless Landlord consents in writing, and shall, at Landlord's option, operate as an assignment to Landlord of any or all subleases.

26.4 The voluntary or other surrender of any ground or other underlying lease that now exists or may hereafter be executed affecting the Building or the Project, or a mutual cancellation thereof or of Landlord's interest therein by Landlord and its lessor shall not effect a merger with Landlord's fee title or leasehold interest in the Premises, the Building or the Property and shall, at the option of the successor to Landlord's interest in the Building or the Project, as applicable, operate as an assignment of this Lease.

27. Holding Over.

27.1 If, with Landlord's prior written consent, Tenant holds possession of all or any part of the Premises after the Term, Tenant shall become a tenant from month to month after the expiration or earlier termination of the Term, and in such case Tenant shall continue to pay (a) Base Rent in accordance with Article 7, as adjusted in accordance with Article 8, and (b) any amounts for which Tenant would otherwise be liable under this Lease if the Lease were still in effect, including payments for Tenant's Adjusted Share of Operating Expenses. Any such month-to-month tenancy shall be subject to every other term, covenant and agreement contained herein.

27.2 Notwithstanding the foregoing, if Tenant remains in possession of the Premises after the expiration or earlier termination of the Term without Landlord's prior written consent, (a) Tenant shall become a tenant at sufferance subject to the terms and conditions of this Lease, except that the monthly rent shall be equal to one hundred fifty percent (150%) of the Rent in effect during the last thirty (30) days of the Term, and (b) if such holdover persists for more than thirty (30) days after the earlier of (i) the expiration or earlier termination of the Term and (ii) the date Landlord notifies Tenant that Landlord has procured a tenant that is ready, willing and able to sign a lease for the Premises (or a portion thereof), Tenant shall be liable to Landlord for any and all damages suffered by Landlord as a result of such holdover, including any lost rent or consequential, special and indirect damages (in each case, regardless of whether such damages are foreseeable).

27.3 Acceptance by Landlord of Rent after the expiration or earlier termination of the Term shall not result in an extension, renewal or reinstatement of this Lease.

27.4 The foregoing provisions of this Article are in addition to and do not affect Landlord's right of reentry or any other rights of Landlord hereunder or as otherwise provided by Applicable Laws.

27.5 The provisions of this Article shall survive the expiration or earlier termination of this Lease.

## 28. Indemnification and Exculpation.

28.1 Tenant agrees to Indemnify the Landlord Indemnitees from and against any and all Claims of any kind or nature, real or alleged, arising from (a) injury to or death of any person or damage to any property occurring within or about the Premises, the Building, the Property or the Project, arising directly or indirectly out of (i) the presence at or use or occupancy of the Premises or Project by a Tenant Party, (ii) an act or omission on the part of any Tenant Party, (b) a breach or default by Tenant in the performance of any of its obligations hereunder (including any Claim asserted by any Lender against any Landlord Indemnitees under any Loan Document as a direct result of such breach or default by Tenant) or (c) injury to or death of persons or damage to or loss of any property, real or alleged, arising from the serving of alcoholic beverages at the Premises or Project, including liability under any dram shop law, host liquor law or similar Applicable Law, except to the extent directly caused by Landlord's negligence or willful misconduct. Tenant's obligations under this Section shall not be affected, reduced or limited by any limitation on the amount or type of damages, compensation or benefits payable by or for Tenant under workers' compensation acts, disability benefit acts, employee benefit acts or similar legislation. Tenant's obligations under this Section shall survive the expiration or earlier termination of this Lease. Subject to Sections 23.6, 28.2 and 31.12 and any subrogation provisions contained in the Work Letter, Landlord agrees to Indemnify the Tenant Parties from and against any and all Claims arising from injury to or death of any person or damage to or loss of any physical property occurring within or about the Premises, the Building, the Property or the Project to the extent directly arising out of Landlord's gross negligence or willful misconduct.

28.2 Notwithstanding anything in this Lease to the contrary, Landlord shall not be liable to Tenant for and Tenant assumes all risk of (a) damage or losses arising from fire, electrical malfunction, gas explosion or water damage of any type (including broken water lines, malfunctioning fire sprinkler systems, roof leaks or stoppages of lines), unless any such loss is due to Landlord's willful disregard of written notice by Tenant of need for a repair that Landlord is responsible to make for an unreasonable period of time, and (b) damage to personal property or scientific research, including loss of records kept by Tenant within the Premises (in each case, regardless of whether such damages are foreseeable). Tenant further waives any claim for injury to Tenant's business or loss of income relating to any such damage or destruction of personal property as described in this Section. Notwithstanding anything in the foregoing or this Lease to the contrary, except (x) as otherwise provided herein (including Section 27.2), (y) as may be provided by Applicable Laws or (z) in the event of Tenant's breach of Article 21 or Section 26.1, in no event shall Landlord or Tenant be liable to the other for any consequential, special or indirect damages arising out of this Lease, including lost profits (provided that this Subsection 28.2(z) shall not limit Tenant's liability for Base Rent or Additional Rent pursuant to this Lease).

28.3 Landlord shall not be liable for any damages arising from any act, omission or neglect of any other tenant in the Building or the Project, or of any other third party.

28.4 Tenant acknowledges that security devices and services, if any, while intended to deter crime, may not in given instances prevent theft or other criminal acts. Landlord shall not be liable for injuries or losses arising from criminal acts of third parties, and Tenant assumes the risk that any security device or service may malfunction or otherwise be circumvented by a criminal. If Tenant desires protection against such criminal acts, then Tenant shall, at Tenant's sole cost and expense, obtain appropriate insurance coverage. Tenant's security programs and equipment for the Premises shall be coordinated with Landlord and subject to Landlord's reasonable approval.

28.5 The provisions of this Article shall survive the expiration or earlier termination of this Lease.

29. Assignment or Subletting.

29.1 Except as hereinafter expressly permitted, none of the following (each, a "Transfer"), either voluntarily or by operation of Applicable Laws, shall be directly or indirectly performed without Landlord's prior written consent, which consent shall not be unreasonably withheld, conditioned or delayed: (a) Tenant selling, hypothecating, assigning, pledging, encumbering or otherwise transferring this Lease or subletting the Premises or (b) a controlling interest in Tenant being sold, assigned or otherwise transferred (other than as a result of shares in Tenant being sold on a public stock exchange). For purposes of the preceding sentence, "control" means (a) owning (directly or indirectly) more than fifty percent (50%) of the stock or other equity interests of another person or (b) possessing, directly or indirectly, the power to direct or cause the direction of the management and policies of such person. Tenant shall have the right to Transfer, without Landlord's prior written consent, Tenant's interest in this Lease or the Premises or any part thereof to (i) any person that as of the date of determination and at all times thereafter directly, or indirectly through one or more intermediaries, controls, is controlled by or is under common control with Tenant ("Tenant's Affiliate") or (ii) any person or any entity with which Tenant is merged or consolidated, or to which all or substantially all of Tenant's assets or all or substantially all of the ownership interests in Tenant are sold, or which results from a direct spin-off from Generation Bio Co.; provided that (in each instance under the foregoing clauses (i) and (ii)) Tenant shall notify Landlord in writing at least thirty (30) days prior to the effectiveness of such Transfer (an "Exempt Transfer") and otherwise comply with the requirements of this Lease regarding such Transfer; and provided, further, that the person that will be the tenant under this Lease after the Exempt Transfer has a net worth (as of both the day immediately prior to and the day immediately after the Exempt Transfer) that is equal to or greater than the net worth (as of both the Execution Date and the date of the Exempt Transfer) of the transferring Tenant. For purposes of the immediately preceding sentence, "control" requires both (a) owning (directly or indirectly) more than fifty percent (50%) of the stock or other equity interests of another person and (b) possessing, directly or indirectly, the power to direct or cause the direction of the management and policies of such person. In no event shall Tenant perform a Transfer (other than an Exempt Transfer (a) to or with an entity that is a tenant at the Project, unless, upon Tenant's written request, Landlord confirms in writing to Tenant that Landlord does not have available space at the Project or (b) if the proposed transferee is then in active discussions or negotiations with Landlord or an affiliate of Landlord to lease premises at the Project or a property owned by Landlord or an affiliate of Landlord in Cambridge, Massachusetts.

29.2 In the event Tenant desires to effect a Transfer, then, at least thirty (30) but not more than ninety (90) days prior to the date when Tenant desires the Transfer to be effective (the "Transfer Date"), Tenant shall provide written notice to Landlord (the "Transfer Notice") containing information (including references) concerning the character of the proposed transferee, assignee or sublessee; the Transfer Date; the most recent unconsolidated financial statements of Tenant and of the proposed transferee, assignee or sublessee satisfying the requirements of

Section 40.2 (“Required Financials”); any ownership or commercial relationship between Tenant and the proposed transferee, assignee or sublessee; copies of Hazardous Materials Documents for the proposed transferee, assignee or sublessee; and the consideration and all other material terms and conditions of the proposed Transfer, all in such detail as Landlord shall reasonably require. Without limiting any other factors that Landlord may consider in determining whether to withhold, condition or delay its consent to any Transfer in accordance with Section 29.1 of this Lease, Tenant hereby acknowledges and agrees that (a) if Tenant does not deliver to Landlord the most recent consolidated financial statements of Tenant (or if Tenant is not the ultimate parent company, the unconsolidated financial statements of Tenant) and the most recent unconsolidated financial statements of such proposed transferee, then it shall be reasonable for Landlord to withhold its consent to any Transfer to such proposed transferee, (b) if Landlord reasonably determines that the proposed Transfer will diminish the value of Landlord’s interest under this Lease, it shall be reasonable for Landlord to withhold its consent to any such Transfer and (c) if Landlord reasonably determines that such proposed Transferee’s financial condition is not satisfactory, it shall be reasonable for Landlord to condition its consent to any such Transfer upon the proposed transferee’s ultimate parent company providing a guaranty to Landlord of such transferee’s obligations under this Lease, in a form acceptable to Landlord, which guaranty shall be executed and delivered to Landlord by the applicable guarantor prior to the Transfer Date, or upon Tenant or the proposed transferee providing other credit enhancements acceptable to Landlord (including without limitation an increased Security Deposit). For purposes of the immediately foregoing clause (c), such transferee’s financial condition will be deemed not satisfactory if Landlord determines that such transferee is not capable of satisfying all of the obligations of the Tenant under this Lease.

29.3 Landlord, in determining whether consent should be given to a proposed Transfer, may give consideration to (a) the financial strength of Tenant and of such transferee, assignee or sublessee (notwithstanding Tenant remaining liable for Tenant’s performance), (b) any change in use that such transferee, assignee or sublessee proposes to make in the use of the Premises and (c) Landlord’s desire to exercise its rights under Section 29.7 to cancel this Lease. In no event shall Landlord be deemed to be unreasonable for declining to consent to a Transfer if any applicable Loan Document prohibits such assignment or any Lender whose consent is required thereunder withholds its consent, or if the Transfer is to a transferee, assignee or sublessee of poor reputation, lacking financial qualifications or seeking a change in the Permitted Use, or jeopardizing directly or indirectly the status of Landlord or any of Landlord’s affiliates as a Real Estate Investment Trust under the Internal Revenue Code of 1986 (as the same may be amended from time to time, the “Revenue Code”). Notwithstanding anything contained in this Lease to the contrary, (w) no Transfer shall be consummated on any basis such that the rental or other amounts to be paid by the occupant, assignee, manager or other transferee thereunder would be based, in whole or in part, on the income or profits derived by the business activities of such occupant, assignee, manager or other transferee; (x) Tenant shall not furnish or render any services to an occupant, assignee, manager or other transferee with respect to whom transfer consideration is required to be paid, or manage or operate the Premises or any capital additions so transferred, with respect to which transfer consideration is being paid; (y) Tenant shall not consummate a Transfer with any person in which Landlord owns an interest, directly or indirectly (by applying constructive ownership rules set forth in Section 856(d) (5) of the Revenue Code); and (z) Tenant shall not consummate a Transfer with any person or in any manner that could cause any portion of the amounts received by Landlord pursuant to this Lease or any sublease, license or other arrangement for the right to

use, occupy or possess any portion of the Premises to fail to qualify as “rents from real property” within the meaning of Section 856(d) of the Revenue Code, or any similar or successor provision thereto or which could cause any other income of Landlord to fail to qualify as income described in Section 856(c)(2) of the Revenue Code. Notwithstanding anything in this Lease to the contrary, if (a) any proposed transferee, assignee or sublessee of Tenant has been required by any prior landlord, Lender or Governmental Authority to take material remedial action in connection with Hazardous Materials contaminating a property if the contamination resulted from such party’s action or omission or use of the property in question or (b) any proposed transferee, assignee or sublessee is subject to a material enforcement order issued by any Governmental Authority in connection with the use, disposal or storage of Hazardous Materials, then it shall not be unreasonable for Landlord to withhold its consent to any proposed transfer, assignment or subletting (with respect to any such matter involving a proposed transferee, assignee or sublessee).

29.4 The following are conditions precedent to a Transfer or to Landlord considering a request by Tenant to a Transfer:

(a) Tenant shall remain fully liable under this Lease. Tenant agrees that it shall not be (and shall not be deemed to be) a guarantor or surety of this Lease, however, and waives its right to claim that it is a guarantor or surety or to raise in any legal proceeding any guarantor or surety defenses permitted by this Lease or by Applicable Laws;

(b) [Intentionally omitted];

(c) In the case of an Exempt Transfer, Tenant shall provide Landlord with evidence reasonably satisfactory to Landlord that the Transfer qualifies as an Exempt Transfer;

(d) Tenant shall provide Landlord with evidence reasonably satisfactory to Landlord that the value of Landlord’s interest under this Lease shall not be diminished or reduced by the proposed Transfer. Such evidence shall include evidence respecting the relevant business experience and financial responsibility and status of the proposed transferee, assignee or sublessee;

(e) Tenant shall reimburse Landlord for Landlord’s actual costs and expenses, including reasonable attorneys’ fees, charges and disbursements incurred in connection with the review, processing and documentation of such request;

(f) Except with respect to an Exempt Transfer, if Tenant’s transfer of rights or sharing of the Premises provides for the receipt by, on behalf of or on account of Tenant of any consideration of any kind whatsoever (including a premium rental for a sublease or lump sum payment for an assignment, but excluding Tenant’s reasonable costs in marketing and subleasing the Premises) in excess of the rental and other charges due to Landlord under this Lease, Tenant shall pay fifty percent (50%) of all of such excess to Landlord, after making deductions for all costs associated with the subject transfer, including any reasonable marketing expenses, tenant improvement funds expended by Tenant, alterations, cash concessions, brokerage commissions, attorneys’ fees and free rent actually paid by Tenant. If such consideration consists of cash paid to Tenant, payment to Landlord shall be made upon receipt by Tenant of such cash payment;

(g) The proposed transferee, assignee or sublessee shall agree that, in the event Landlord gives such proposed transferee, assignee or sublessee notice that Tenant is in default under this Lease, such proposed transferee, assignee or sublessee shall thereafter make all payments otherwise due Tenant directly to Landlord, which payments shall be received by Landlord without any liability being incurred by Landlord, except to credit such payment against those due by Tenant under this Lease, and any such proposed transferee, assignee or sublessee shall agree to attorn to Landlord or its successors and assigns should this Lease be terminated for any reason; provided, however, that in no event shall Landlord or its Lenders, successors or assigns be obligated to accept such attornment;

(h) Landlord's consent to any such Transfer shall be effected on Landlord's forms, which shall be commercially reasonable;

(i) Tenant shall not then be in default of any monetary obligation or any material non-monetary obligation hereunder in any respect;

(j) Such proposed transferee, assignee or sublessee's use of the Premises shall be the same as the Permitted Use;

(k) Landlord shall not be bound by any provision of any agreement pertaining to the Transfer, except for Landlord's written consent to the same;

(l) Tenant shall pay all transfer and other taxes (including interest and penalties) assessed or payable for any Transfer;

(m) Landlord's consent (or waiver of its rights) for any Transfer shall not waive Landlord's right to consent or refuse consent to any later Transfer;

(n) Tenant shall deliver to Landlord one executed copy of any and all written instruments evidencing or relating to the Transfer; and

(o) Tenant shall deliver to Landlord a list of Hazardous Materials (as defined below), certified by the proposed transferee, assignee or sublessee to be true and correct, that the proposed transferee, assignee or sublessee intends to use or store in the Premises. Additionally, Tenant shall deliver to Landlord, on or before the date any proposed transferee, assignee or sublessee takes occupancy of the Premises, all of the items relating to Hazardous Materials of such proposed transferee, assignee or sublessee as described in Section 21.2.

29.5 Any Transfer that is not in compliance with the provisions of this Article or with respect to which Tenant does not fulfill its obligations pursuant to this Article shall be void and shall, at the option of Landlord, terminate this Lease.

29.6 Notwithstanding any Transfer, Tenant shall remain fully and primarily liable for the payment of all Rent and other sums due or to become due hereunder, and for the full performance of all other terms, conditions and covenants to be kept and performed by Tenant. The acceptance of Rent or any other sum due hereunder, or the acceptance of performance of any other term, covenant or condition thereof, from any person or entity other than Tenant shall not be deemed a waiver of any of the provisions of this Lease or a consent to any Transfer.

29.7 If Tenant delivers to Landlord a Transfer Notice indicating a desire to transfer all or substantially all of the Premises and/or for all or substantially all of the remainder of the Term to a proposed transferee, assignee or sublessee other than pursuant to an Exempt Transfer, then Landlord shall have the option, exercisable by giving notice to Tenant at any time within fifteen (15) business days after Landlord's receipt of such Transfer Notice, to terminate this Lease as of the date specified in the Transfer Notice as the Transfer Date, except for those provisions that, by their express terms, survive the expiration or earlier termination hereof. If Landlord exercises such option, then Tenant shall have the right to withdraw such Transfer Notice by delivering to Landlord written notice of such election within five (5) business days after Landlord's delivery of notice electing to exercise Landlord's option to terminate this Lease. In the event Tenant withdraws the Transfer Notice as provided in this Section, this Lease shall continue in full force and effect. No failure of Landlord to exercise its option to terminate this Lease shall be deemed to be Landlord's consent to a proposed Transfer.

29.8 If Tenant sublets the Premises or any portion thereof, Tenant hereby immediately and irrevocably assigns to Landlord, as security for Tenant's obligations under this Lease, all rent from any such subletting, and appoints Landlord as assignee and attorney-in-fact for Tenant, and Landlord (or a receiver for Tenant appointed on Landlord's application) may collect such rent and apply it toward Tenant's obligations under this Lease; provided that, until the occurrence of a Default (as defined below) by Tenant, Tenant shall have the right to collect such rent.

29.9 In the event that Tenant enters into a sublease for the entire Premises in accordance with this Article that expires within two (2) days of the Term Expiration Date, the term expiration date of such sublease shall, notwithstanding anything in this Lease, the sublease or any consent to the sublease to the contrary, be deemed to be the date that is two (2) days prior to the Term Expiration Date.

### 30. Subordination and Attornment.

30.1 This Lease shall be subject and subordinate to the lien of any mortgage, deed of trust, or lease in which Landlord is tenant now or hereafter in force against the Building or the Project and to all advances made or hereafter to be made upon the security thereof without the necessity of the execution and delivery of any further instruments on the part of Tenant to effectuate such subordination.

30.2 Notwithstanding the foregoing, Tenant shall execute and deliver upon demand such further instrument or instruments evidencing such subordination of this Lease to the lien of any such mortgage or mortgages or deeds of trust or lease in which Landlord is tenant as may be required by Landlord. If any Lender so elects, however, this Lease shall be deemed prior in lien to any such lease, mortgage, or deed of trust upon or including the Premises regardless of date and Tenant shall execute a statement in writing to such effect at Landlord's request. If Tenant fails to execute any document required from Tenant under this Section within ten (10) business days after written request therefor, it shall be a default hereunder, subject to applicable notice and cure periods. For the avoidance of doubt, "Lenders" shall also include historic tax credit investors and new market tax credit investors.



30.3 Upon written request of Landlord and opportunity for Tenant to review, Tenant agrees to execute any Lease amendments not materially altering the terms of this Lease or increasing Tenant's monetary obligations or materially increasing Tenant's other material nonmonetary obligations hereunder, if required by a Lender incident to the financing of the real property of which the Premises constitute a part.

30.4 In the event any proceedings are brought for foreclosure, or in the event of the exercise of the power of sale under any mortgage or deed of trust made by Landlord covering the Premises, Tenant shall at the election of the purchaser at such foreclosure or sale attorn to the purchaser upon any such foreclosure or sale and recognize such purchaser as Landlord under this Lease.

30.5 Landlord agrees to use commercially reasonable efforts to request from its current Lender of the Property that such Lender enter into its standard subordination, non-disturbance and attornment agreement (the "SNDA") with Tenant. If Lender so agrees to enter into the SNDA, Tenant shall pay the reasonable charges or fees which may be required by such Lender in order to obtain such agreement.

### 31. Defaults and Remedies.

31.1 Late payment by Tenant to Landlord of Rent and other sums due shall cause Landlord to incur costs not contemplated by this Lease, the exact amount of which shall be extremely difficult and impracticable to ascertain. Such costs include processing and accounting charges and late charges that may be imposed on Landlord by the terms of any mortgage or trust deed covering the Premises. Therefore, if any installment of Rent due from Tenant is not received by Landlord within three (3) business days after the date such payment is due, Tenant shall pay to Landlord (a) an additional sum of five percent (5%) of the overdue Rent as a late charge plus (b) interest at an annual rate (the "Default Rate") equal to the lesser of (a) twelve percent (12%) and (b) the highest rate permitted by Applicable Laws. The parties agree that this late charge represents a fair and reasonable estimate of the costs that Landlord shall incur by reason of late payment by Tenant and shall be payable as Additional Rent to Landlord due with the next installment of Rent or within five (5) business days after Landlord's demand, whichever is earlier. Landlord's acceptance of any Additional Rent (including a late charge or any other amount hereunder) shall not be deemed an extension of the date that Rent is due or prevent Landlord from pursuing any other rights or remedies under this Lease, at law or in equity.

31.2 No payment by Tenant or receipt by Landlord of a lesser amount than the Rent payment herein stipulated shall be deemed to be other than on account of the Rent, nor shall any endorsement or statement on any check or any letter accompanying any check or payment as Rent be deemed an accord and satisfaction, and Landlord may accept such check or payment without prejudice to Landlord's right to recover the balance of such Rent or pursue any other remedy provided in this Lease or in equity or at law. If a dispute shall arise as to any amount or sum of money to be paid by Tenant to Landlord hereunder, Tenant shall have the right to make payment "under protest," such payment shall not be regarded as a voluntary payment, and there shall survive the right on the part of Tenant to institute suit for recovery of the payment paid under protest.

31.3 If Tenant fails to pay any sum of money required to be paid by it hereunder or perform any other act on its part to be performed hereunder, in each case within the applicable cure period (if any) described in Section 31.4, then Landlord may (but shall not be obligated to), without waiving or releasing Tenant from any obligations of Tenant, make such payment or perform such act; provided that such failure by Tenant unreasonably interfered with the use of the Building or the Project by any other tenant or with the efficient operation of the Building or the Project, or resulted or could have resulted in a violation of Applicable Laws or the cancellation of an insurance policy maintained by Landlord. Notwithstanding the foregoing, in the event of an emergency, Landlord shall have the right to enter the Premises and act in accordance with its rights as provided elsewhere in this Lease. In addition to the late charge described in Section 31.1, Tenant shall pay to Landlord as Additional Rent all sums so paid or incurred by Landlord, together with interest at the Default Rate, computed from the date such sums were paid or incurred.

31.4 The occurrence of any one or more of the following events shall constitute a "Default" hereunder by Tenant:

(a) If Tenant (i) abandons the Premises; or (ii)(A) Landlord receives notice of Tenant's vacation of or Tenant's intention to vacate the Premises prior to the scheduled expiration or earlier termination of this Lease, other than in accordance with a right expressly granted to Tenant under this Lease, and such vacation (or intention to vacate) is related to financial hardship or Tenant's inability to pay its debts as they become due, a dissolution of Tenant, or the liquidation or winding up of Tenant's business operations; or (B) Tenant vacates the Premises prior to the scheduled expiration or earlier termination of this Lease, other than in accordance with a right expressly granted to Tenant under this Lease, within the one hundred twenty (120) day period following the filing of any involuntary petition against Tenant or the attachment of Tenant's interest in this Lease (notwithstanding anything to the contrary in Sections 31.4(g) and 31.4(i));

(b) Tenant fails to make any payment of Rent, as and when due, or to satisfy its obligations under Article 19, where such failure shall continue for a period of three (3) business days after written notice thereof from Landlord to Tenant;

(c) Tenant fails to observe or perform any obligation or covenant contained herein (other than described in Sections 31.4(a) and 31.4(b)) to be performed by Tenant, where such failure continues for a period of thirty (30) days after written notice thereof from Landlord to Tenant; provided that, if the nature of Tenant's default is such that it reasonably requires more than thirty (30) days to cure, Tenant shall not be deemed to be in Default if Tenant commences such cure within such thirty (30) day period and thereafter diligently prosecutes the same to completion; and provided, further, that such cure is completed no later than ninety (90) days after Tenant's receipt of written notice from Landlord;

(d) Tenant makes an assignment for the benefit of creditors;

(e) A receiver, trustee or custodian is appointed to or does take title, possession or control of all or substantially all of Tenant's assets;

(f) Tenant files a voluntary petition under the United States Bankruptcy Code or any successor statute (as the same may be amended from time to time, the "Bankruptcy Code") or an order for relief is entered against Tenant pursuant to a voluntary or involuntary proceeding commenced under any chapter of the Bankruptcy Code;

(g) Any involuntary petition is filed against Tenant under any chapter of the Bankruptcy Code and is not dismissed within one hundred twenty (120) days;

(h) Tenant fails to deliver an estoppel certificate in accordance with Article 20;

or

(i) Tenant's interest in this Lease is attached, executed upon or otherwise judicially seized and such action is not released within one hundred twenty (120) days of the action.

Notices given under this Section shall specify the alleged default and shall demand that Tenant perform the provisions of this Lease or pay the Rent that is in arrears, as the case may be, within the applicable period of time, or quit the Premises. No such notice shall be deemed a forfeiture or a termination of this Lease unless Landlord elects otherwise in such notice.

31.5 In the event of a Default by Tenant, and at any time thereafter, with or without notice or demand and without limiting Landlord in the exercise of any right or remedy that Landlord may have, Landlord has the right to do any or all of the following:

(a) Halt any Tenant Improvements or Alterations and order Tenant's contractors, subcontractors, consultants, designers and material suppliers to stop work;

(b) Terminate Tenant's right to possession of the Premises by written notice to Tenant or by any lawful means, in which case Tenant shall immediately surrender possession of the Premises to Landlord. In such event, Landlord shall have the immediate right to re-enter and remove all persons and property, and such property may be removed and stored in a public warehouse or elsewhere at the cost and for the account of Tenant, all without service of notice or resort to legal process and without being deemed guilty of trespass or becoming liable for any loss or damage that may be occasioned thereby; and

(c) Terminate this Lease, in which event Tenant shall immediately surrender possession of the Premises to Landlord. In such event, Landlord shall have the immediate right to re-enter and remove all persons and property, and such property may be removed and stored in a public warehouse or elsewhere at the cost and for the account of Tenant, all without service of notice or resort to legal process and without being deemed guilty of trespass or becoming liable for any loss or damage that may be occasioned thereby. In the event that Landlord shall elect to so terminate this Lease, then Landlord shall be entitled to recover from Tenant all damages incurred by Landlord by reason of Tenant's default, including the sum of:

(i) The worth at the time of award of any unpaid Rent that had accrued at the time of such termination; plus

(ii) The costs of restoring the Premises to the condition required under the terms of this Lease; plus

(iii) An amount (the "Election Amount") equal to either (A) the positive difference (if any, and measured at the time of such termination) between (1) the then-present value of the total Rent and other benefits that would have accrued to Landlord under this Lease for the remainder of the Term if Tenant had fully complied with the Lease minus (2) the then-present cash rental value of the Premises as determined by Landlord for what would be the then-unexpired Term if the Lease remained in effect, computed using the discount rate of the Federal Reserve Bank of San Francisco at the time of the award plus one (1) percentage point (the "Discount Rate") or (B) twelve (12) months (or such lesser number of months as may then be remaining in the Term) of Base Rent and Additional Rent at the rate last payable by Tenant pursuant to this Lease, in either case as Landlord specifies in such election. Landlord and Tenant agree that the Election Amount represents a reasonable forecast of the minimum damages expected to occur in the event of a breach, taking into account the uncertainty, time and cost of determining elements relevant to actual damages, such as fair market rent, time and costs that may be required to re-lease the Premises, and other factors; and that the Election Amount is not a penalty.

As used in Section 31.5(c)(i), "worth at the time of award" shall be computed by allowing interest at the Default Rate.

31.6 In addition to any other remedies available to Landlord at law or in equity and under this Lease, Landlord may continue this Lease in effect after Tenant's Default or abandonment and recover Rent as it becomes due. In addition, Landlord shall not be liable in any way whatsoever for its failure or refusal to relet the Premises. For purposes of this Section, the following acts by Landlord will not constitute the termination of Tenant's right to possession of the Premises:

(a) Acts of maintenance or preservation or efforts to relet the Premises, including alterations, remodeling, redecorating, repairs, replacements or painting as Landlord shall consider advisable for the purpose of reletting the Premises or any part thereof; or

(b) The appointment of a receiver upon the initiative of Landlord to protect Landlord's interest under this Lease or in the Premises.

Notwithstanding the foregoing, in the event of a Default by Tenant, Landlord may elect at any time to terminate this Lease and to recover damages to which Landlord is entitled.

31.7 If Landlord does not elect to terminate this Lease as provided in Section 31.5, then Landlord may, from time to time, recover all Rent as it becomes due under this Lease. At any time thereafter, Landlord may elect to terminate this Lease and to recover damages to which Landlord is entitled.

31.8 In the event Landlord elects to terminate this Lease and relet the Premises, Landlord may execute any new lease in its own name. Tenant hereunder shall have no right or authority whatsoever to collect any Rent from such tenant. The proceeds of any such reletting shall be applied as follows:

(a) First, to the payment of any indebtedness other than Rent due hereunder from Tenant to Landlord, including storage charges or brokerage commissions owing from Tenant to Landlord as the result of such reletting;

(b) Second, to the payment of the costs and expenses of reletting the Premises, including (i) alterations and repairs that Landlord deems reasonably necessary and advisable and (ii) reasonable attorneys' fees, charges and disbursements incurred by Landlord in connection with the retaking of the Premises and such reletting;

(c) Third, to the payment of Rent and other charges due and unpaid hereunder; and

(d) Fourth, to the payment of future Rent and other damages payable by Tenant under this Lease.

31.9 All of Landlord's rights, options and remedies hereunder shall be construed and held to be nonexclusive and cumulative. Landlord shall have the right to pursue any one or all of such remedies, or any other remedy or relief that may be provided by Applicable Laws, whether or not stated in this Lease. No waiver of any default of Tenant hereunder shall be implied from any acceptance by Landlord of any Rent or other payments due hereunder or any omission by Landlord to take any action on account of such default if such default persists or is repeated, and no express waiver shall affect defaults other than as specified in such waiver. Notwithstanding any provision of this Lease to the contrary, in no event shall Landlord be required to mitigate its damages with respect to any default by Tenant, except as required by Applicable Laws. Any such obligation imposed by Applicable Laws upon Landlord to relet the Premises after any termination of this Lease shall be subject to the reasonable requirements of Landlord to (a) lease to high quality tenants on such terms as Landlord may from time to time deem appropriate in its discretion and (b) develop the Project in a harmonious manner with a mix of uses, tenants, floor areas, terms of tenancies, etc., as determined by Landlord. Landlord shall not be obligated to relet the Premises to (y) any Tenant's Affiliate or (z) any party (i) unacceptable to a Lender, (ii) that requires Landlord to make improvements to or re-demise the Premises, (iii) that desires to change the Permitted Use, (iv) that desires to lease the Premises for more or less than the remaining Term or (v) to whom Landlord or an affiliate of Landlord may desire to lease other available space in the Project or at another property owned by Landlord or an affiliate of Landlord.

31.10 Landlord's termination of (a) this Lease or (b) Tenant's right to possession of the Premises shall not relieve Tenant of any liability to Landlord that has previously accrued or that shall arise based upon events that occurred prior to the later to occur of (y) the date of Lease termination and (z) the date Tenant surrenders possession of the Premises.

31.11 To the extent permitted by Applicable Laws, Tenant waives any and all rights of redemption granted by or under any present or future Applicable Laws if Tenant is evicted or dispossessed for any cause, or if Landlord obtains possession of the Premises due to Tenant's default hereunder or otherwise.

31.12 Landlord shall not be in default or liable for damages under this Lease unless Landlord fails to perform obligations required of Landlord within a reasonable time, but in no event shall such failure continue for more than thirty (30) days after written notice from Tenant specifying the nature of Landlord's failure; provided, however, that if the nature of Landlord's obligation is such that more than thirty (30) days are required for its performance, then Landlord shall not be in default if Landlord commences performance within such thirty (30) day period and

thereafter diligently prosecutes the same to completion. If Landlord fails to commence to cure any default by Landlord within the period provided above in this paragraph and if, as a result the default, Tenant is incapable despite commercially reasonable efforts to continue operations within the Premises, Tenant may give Landlord an additional written notice confirming that the default has not been cured and that Tenant intends to cure such default, and, if Landlord fails to cure such default within thirty (30) days after such notice, Tenant may take such steps within the confines of its Premises as are reasonably appropriate to cure the default and seek to recover from Landlord the reasonable cost of such cure. Tenant shall have no right to perform any obligation of Landlord in lieu of Landlord to the extent the same involves or may impact any base building system, any structural element of the Building, or any area of the Building outside of the Premises, including, without limitation, the Common Area or the premises of any other tenant or occupant of the Building (collectively, the “Excluded Systems/Areas”). Landlord’s liability to keep, maintain, and repair shall always be limited to the cost of making such repair or accomplishing such maintenance or repair and Landlord shall not be liable for any consequential or any indirect damages. In no event shall Tenant have the right to terminate or cancel this Lease or offset from Rent as a result of Landlord’s default. Notwithstanding the above provisions of this Section 31.12 to the contrary, in emergency situations such that the prior written notice to Landlord provided for above is not practical, Tenant may, upon such shorter period of written notice or contemporaneous written and oral notice as is appropriate under the circumstances, and excluding in all instances any work or access to Excluded Systems/Areas, take such steps as are reasonably appropriate to cure the default, in which event Tenant’s rights with respect to recovering the cost of such cure shall be as provided above.

31.13 In the event of any default by Landlord, Tenant shall give notice by registered or certified mail to any (a) beneficiary of a deed of trust or (b) mortgagee under a mortgage covering the Premises, the Building or the Project and to any landlord of any lease of land upon or within which the Premises, the Building or the Project is located, and shall offer such beneficiary, mortgagee or landlord a reasonable opportunity to cure the default, including time to obtain possession of the Building or the Project by power of sale or a judicial action if such should prove necessary to effect a cure; provided that Landlord shall furnish to Tenant in writing, upon written request by Tenant, the names and addresses of all such persons who are to receive such notices.

32. Bankruptcy. In the event a debtor, trustee or debtor in possession under the Bankruptcy Code, or another person with similar rights, duties and powers under any other Applicable Laws, proposes to cure any default under this Lease or to assume or assign this Lease and is obliged to provide adequate assurance to Landlord that (a) a default shall be cured, (b) Landlord shall be compensated for its damages arising from any breach of this Lease and (c) future performance of Tenant’s obligations under this Lease shall occur, then such adequate assurances shall include any or all of the following, as designated by Landlord in its sole and absolute discretion:

32.1 Those acts specified in the Bankruptcy Code or other Applicable Laws as included within the meaning of “adequate assurance,” even if this Lease does not concern a shopping center or other facility described in such Applicable Laws;

32.2 A prompt cash payment to compensate Landlord for any monetary defaults or actual damages arising directly from a breach of this Lease;

32.3 A cash deposit in an amount at least equal to the then-current amount of the Security Deposit; or

32.4 The assumption or assignment of all of Tenant's interest and obligations under this Lease.

33. Brokers.

33.1 Landlord and Tenant each represents and warrants to the other that it has had no dealings with any real estate broker or agent in connection with the negotiation of this Lease other than CBRE | New England ("Broker"), and that it knows of no other real estate broker or agent that is or might be entitled to a commission in connection with this Lease. Landlord shall compensate Broker in relation to this Lease pursuant to a separate agreement between Landlord and Broker.

33.2 Tenant represents and warrants that no broker or agent has made any representation or warranty relied upon by Tenant in Tenant's decision to enter into this Lease, other than as contained in this Lease.

33.3 Tenant acknowledges and agrees that the employment of brokers by Landlord is for the purpose of solicitation of offers of leases from prospective tenants and that no authority is granted to any broker to furnish any representation (written or oral) or warranty from Landlord unless expressly contained within this Lease. Landlord is executing this Lease in reliance upon Tenant's representations, warranties and agreements contained within Sections 33.1 and 33.2.

33.4 Landlord and Tenant each agree to Indemnify, respectively, the Tenant Indemnitees and the Landlord Indemnitees harmless from any and all cost or liability for compensation claimed by any broker or agent, other than Broker, employed or engaged by Landlord and Tenant or claiming to have been employed or engaged by Landlord or Tenant.

34. Definition of Landlord. With regard to obligations imposed upon Landlord pursuant to this Lease, the term "Landlord," as used in this Lease, shall refer only to Landlord or Landlord's then-current successor-in-interest. In the event of any transfer, assignment or conveyance of Landlord's interest in this Lease or in Landlord's fee title to or leasehold interest in the Property, as applicable, Landlord herein named (and in case of any subsequent transfers or conveyances, the subsequent Landlord) shall be automatically freed and relieved, from and after the date of such transfer, assignment or conveyance, from all liability for the performance of any covenants or obligations contained in this Lease thereafter to be performed by Landlord and, without further agreement, the transferee, assignee or conveyee of Landlord's in this Lease or in Landlord's fee title to or leasehold interest in the Property, as applicable, shall be deemed to have assumed and agreed to observe and perform any and all covenants and obligations of Landlord hereunder during the tenure of its interest in the Lease or the Property. Landlord or any subsequent Landlord may transfer its interest in the Premises or this Lease without Tenant's consent.

35. Limitation of Landlord's Liability.

35.1 If Landlord is in default under this Lease and, as a consequence, Tenant recovers a monetary judgment against Landlord, the judgment shall be satisfied only out of (a) the proceeds of sale received on execution of the judgment and levy against the right, title and interest of Landlord in the Building and the Project, (b) rent or other income from such real property receivable by Landlord or (c) the consideration received by Landlord from the sale, financing, refinancing or other disposition of all or any part of Landlord's right, title or interest in the Building or the Project.

35.2 Neither Landlord nor any of its affiliates, nor any of their respective partners, shareholders, directors, officers, employees, members or agents shall be personally liable for Landlord's obligations or any deficiency under this Lease, and service of process shall not be made against any shareholder, director, officer, employee or agent of Landlord or any of Landlord's affiliates. No partner, shareholder, director, officer, employee, member or agent of Landlord or any of its affiliates shall be sued or named as a party in any suit or action, and service of process shall not be made against any partner or member of Landlord except as may be necessary to secure jurisdiction of the partnership, joint venture or limited liability company, as applicable. No partner, shareholder, director, officer, employee, member or agent of Landlord or any of its affiliates shall be required to answer or otherwise plead to any service of process, and no judgment shall be taken or writ of execution levied against any partner, shareholder, director, officer, employee, member or agent of Landlord or any of its affiliates.

35.3 Tenant's directors, officers or employees shall not be personally liable for Tenant's obligations or any deficiency under this Lease. No director, officer or employee of Tenant shall be sued or named as a party in any suit or action, and service of process shall not be made against any director, officer or employee. No director, officer or employee of Tenant shall be required to answer or otherwise plead to any service of process, and no judgment shall be taken or writ of execution levied against any director, officer or employee.

35.4 Each of the covenants and agreements of this Article shall be applicable to any covenant or agreement either expressly contained in this Lease or imposed by Applicable Laws and shall survive the expiration or earlier termination of this Lease.

36. Joint and Several Obligations. If more than one person or entity executes this Lease as Tenant, then:

36.1 Each of them is jointly and severally liable for the keeping, observing and performing of all of the terms, covenants, conditions, provisions and agreements of this Lease to be kept, observed or performed by Tenant, and such terms, covenants, conditions, provisions and agreements shall be binding with the same force and effect upon each and all of the persons executing this Agreement as Tenant; and

36.2 The term "Tenant," as used in this Lease, shall mean and include each of them, jointly and severally. The act of, notice from, notice to, refund to, or signature of any one or more of them with respect to the tenancy under this Lease, including any renewal, extension, expiration, termination or modification of this Lease, shall be binding upon each and all of the persons executing this Lease as Tenant with the same force and effect as if each and all of them had so acted, so given or received such notice or refund, or so signed.



37. Representations. Tenant warrants and represents that (a) Tenant is duly incorporated or otherwise established or formed and validly existing under the laws of its state of incorporation, establishment or formation, (b) Tenant has and is duly qualified to do business in the state in which the Property is located, (c) Tenant has full corporate, partnership, trust, association or other appropriate power and authority to enter into this Lease and to perform all Tenant's obligations hereunder, (d) each person (and all of the persons if more than one signs) signing this Lease on behalf of Tenant is duly and validly authorized to do so and (e) neither (i) the execution, delivery or performance of this Lease nor (ii) the consummation of the transactions contemplated hereby will violate or conflict with any provision of documents or instruments under which Tenant is constituted or to which Tenant is a party. In addition, Tenant represents that Tenant, and to Tenant's current, actual knowledge, its members, shareholders or other equity owners (without duty of inquiry) is not an entity with whom U.S. persons or entities are restricted from doing business under regulations of the Office of Foreign Asset Control ("OFAC") of the Department of the Treasury (including those named on OFAC's Specially Designated and Blocked Persons List) or under any statute, executive order (including the September 24, 2001, Executive Order Blocking Property and Prohibiting Transactions with Persons Who Commit, Threaten to Commit, or Support Terrorism) or other similar governmental action. Landlord represents that, to its current, actual knowledge (without duty of inquiry), it is not an entity with whom U.S. persons or entities are restricted from doing business under regulations of OFAC of the Department of the Treasury (including those named on OFAC's Specially Designated and Blocked Persons List) or under any statute, executive order (including the September 24, 2001, Executive Order Blocking Property and Prohibiting Transactions with Persons Who Commit, Threaten to Commit, or Support Terrorism) or other similar governmental action.

38. Confidentiality. Tenant shall keep the terms and conditions of this Lease and any information provided to Tenant or its employees, agents or contractors pursuant to Article 9 confidential and shall not (a) disclose to any third party any terms or conditions of this Lease or any other Lease-related document (including subleases, assignments, work letters, construction contracts, letters of credit, subordination agreements, non-disturbance agreements, brokerage agreements or estoppels) or the contents of any documents, reports, surveys or evaluations related to the Project or any portion thereof or (b) provide to any third party an original or copy of this Lease (or any Lease-related document) or another document referenced in Subsection 38(a). Landlord shall not release to any third party any non-public financial information or non-public information about Tenant's ownership structure that Tenant gives Landlord. Notwithstanding the foregoing, confidential information under this Section may be released by Landlord or Tenant under the following circumstances: (x) if required by Applicable Laws or in any judicial proceeding; provided that the releasing party has given the other party reasonable notice of such requirement, if feasible, (y) to a party's attorneys, accountants, brokers, lenders, potential lenders, investors, potential investors and other bona fide consultants or advisers (with respect to this Lease only); provided such third parties agree to be bound by this Section or (z) to bona fide prospective assignees or subtenants of this Lease; provided they agree in writing to be bound by this Section.

39. Notices. Except as otherwise stated in this Lease, any notice, consent, demand, invoice, statement or other communication required or permitted to be given hereunder shall be in writing and shall be given by (a) personal delivery or (b) overnight delivery with a reputable international overnight delivery service, such as FedEx, or (c) email transmission, so long as such transmission is followed within one (1) business day by delivery utilizing one of the methods described in

Subsection 39(a) or (b). Any such notice, consent, demand, invoice, statement or other communication shall be deemed delivered (x) upon receipt, if given in accordance with Subsection 39(a); (y) one (1) business day after deposit with a reputable international overnight delivery service, if given in accordance with Subsection 39(b); or (z) upon transmission, if given in accordance with Subsection 39(c). Except as otherwise stated in this Lease, any notice, consent, demand, invoice, statement or other communication required or permitted to be given pursuant to this Lease shall be addressed to Tenant at the Premises, or to Landlord or Tenant at the addresses shown in Sections 2.9 and 2.10 or 2.11, respectively. Either party may, by notice to the other given pursuant to this Section, specify additional or different addresses for notice purposes.

#### 40. Miscellaneous.

40.1 Landlord reserves the right to change the name of the Building or the Project in its sole discretion.

40.2 To induce Landlord to enter into this Lease, Tenant agrees that it shall promptly furnish to Landlord, from time to time, upon Landlord's written request, the most recent year-end unconsolidated financial statements reflecting Tenant's current financial condition audited by a nationally recognized accounting firm. In addition, upon Landlord's written request, Tenant shall provide copies of its latest quarterly financial reports. Additionally, Tenant shall, within one hundred twenty (120) days after the end of Tenant's financial year, furnish Landlord with Tenant's year-end unconsolidated financial statements for the previous year audited by a nationally recognized accounting firm. Tenant represents and warrants that all financial statements, records and information furnished by Tenant to Landlord in connection with this Lease are true, correct and complete in all respects. If audited financials are not otherwise prepared, unaudited financials complying with generally accepted accounting principles and certified by the chief financial officer of Tenant as true, correct and complete in all respects shall suffice for purposes of this Section. The provisions of this Section shall not apply at any time while Tenant is a corporation whose shares are traded on any nationally recognized stock exchange. If Tenant fails to deliver to Landlord any financial statement within the time period required under this Section, then Tenant shall be required to pay to Landlord an administrative fee equal to Five Hundred Dollars (\$500) within ten (10) business days after receiving written notice from Landlord advising Tenant of such failure (provided, however, that Landlord's acceptance of such fee shall not prevent Landlord from pursuing any other rights or remedies under this Lease, at law or in equity).

40.3 Submission of this instrument for examination or signature by Tenant does not constitute a reservation of or option for a lease, and shall not be effective as a lease or otherwise until execution by and delivery to both Landlord and Tenant.

40.4 The terms of this Lease are intended by the parties as a final, complete and exclusive expression of their agreement with respect to the terms that are included herein, and may not be contradicted or supplemented by evidence of any other prior or contemporaneous agreement.

40.5 Upon the request of either Landlord or Tenant, the parties shall execute a document in recordable form containing only such information as is necessary to constitute a Notice of Lease under Massachusetts law. All costs of preparing and recording such notice shall be borne by the requesting party. Within ten (10) days after receipt of written request from Landlord after the expiration or earlier termination of this Lease, Tenant shall execute a termination of any Notice of Lease recorded with respect hereto. Neither party shall record this Lease.

40.6 Where applicable in this Lease, the singular includes the plural and the masculine or neuter includes the masculine, feminine and neuter. The words “include,” “includes,” “included” and “including” mean “include,” etc., without limitation.” The word “shall” is mandatory and the word “may” is permissive. The section headings of this Lease are not a part of this Lease and shall have no effect upon the construction or interpretation of any part of this Lease. Landlord and Tenant have each participated in the drafting and negotiation of this Lease, and the language in all parts of this Lease shall be in all cases construed as a whole according to its fair meaning and not strictly for or against either Landlord or Tenant.

40.7 Except as otherwise expressly set forth in this Lease, each party shall pay its own costs and expenses incurred in connection with this Lease and such party’s performance under this Lease; provided that, if either party commences an action, proceeding, demand, claim, action, cause of action or suit against the other party arising out of or in connection with this Lease, then the substantially prevailing party shall be reimbursed by the other party for all reasonable costs and expenses, including reasonable attorneys’ fees and expenses, incurred by the substantially prevailing party in such action, proceeding, demand, claim, action, cause of action or suit, and in any appeal in connection therewith (regardless of whether the applicable action, proceeding, demand, claim, action, cause of action, suit or appeal is voluntarily withdrawn or dismissed).

40.8 Time is of the essence with respect to the performance of every provision of this Lease.

40.9 Each provision of this Lease performable by Tenant shall be deemed both a covenant and a condition.

40.10 Notwithstanding anything to the contrary contained in this Lease, Tenant’s obligations under this Lease are independent and shall not be conditioned upon performance by Landlord.

40.11 Whenever consent or approval of either party is required, that party shall not unreasonably withhold, condition or delay such consent or approval, except as may be expressly set forth to the contrary.

40.12 Any provision of this Lease that shall prove to be invalid, void or illegal shall in no way affect, impair or invalidate any other provision hereof, and all other provisions of this Lease shall remain in full force and effect and shall be interpreted as if the invalid, void or illegal provision did not exist.

40.13 Each of the covenants, conditions and agreements herein contained shall inure to the benefit of and shall apply to and be binding upon the parties hereto and their respective heirs; legatees; devisees; executors; administrators; and permitted successors and assigns. This Lease is for the sole benefit of the parties and their respective heirs, legatees, devisees, executors, administrators and permitted successors and assigns, and nothing in this Lease shall give or be construed to give any other person or entity any legal or equitable rights. Nothing in this Section shall in any way alter the provisions of this Lease restricting assignment or subletting.

40.14 This Lease shall be governed by, construed and enforced in accordance with the laws of the state in which the Premises are located, without regard to such state's conflict of law principles.

40.15 Each party hereto guarantees, warrants and represents that the individual or individuals signing this Lease have the power, authority and legal capacity to sign this Lease on behalf of and to bind all entities, corporations, partnerships, limited liability companies, joint venturers or other organizations and entities on whose behalf such individual or individuals have signed.

40.16 This Lease may be executed in one or more counterparts, each of which, when taken together, shall constitute one and the same document. The parties acknowledge and agree that this Lease may be executed via .pdf format (including computer-scanned or other electronic reproduction of the actual signatures) and that delivery of a signature by electronic or physical means shall be effective to the same extent as delivery of an original signature. Notwithstanding the foregoing, originally signed documents shall be provided upon either party's request.

40.17 No provision of this Lease may be modified, amended or supplemented except by an agreement in writing signed by Landlord and Tenant.

40.18 No waiver of any term, covenant or condition of this Lease shall be binding upon Landlord unless executed in writing by Landlord. The waiver by Landlord of any breach or default of any term, covenant or condition contained in this Lease shall not be deemed to be a waiver of any preceding or subsequent breach or default of such term, covenant or condition or any other term, covenant or condition of this Lease.

40.19 To the extent permitted by Applicable Laws, the parties waive trial by jury in any action, proceeding or counterclaim brought by the other party hereto related to matters arising out of or in any way connected with this Lease; the relationship between Landlord and Tenant; Tenant's use or occupancy of the Premises; or any claim of injury or damage related to this Lease or the Premises.

#### 41. Rooftop Installation Area.

41.1 Tenant may use a portion of the Building allocated by Landlord and depicted on Exhibit A (the "Rooftop Installation Area") solely to operate, maintain, repair and replace rooftop antennae, mechanical equipment, communications antennas, a generator, and other equipment installed by Tenant in the Rooftop Installation Area in accordance with this Article ("Tenant's Rooftop Equipment"). Tenant's Rooftop Equipment shall be only for Tenant's use of the Premises for the Permitted Use.

41.2 Tenant shall install Tenant's Rooftop Equipment at its sole cost and expense, at such times and in such manner as Landlord may reasonably designate, and in accordance with this Article and the applicable provisions of this Lease regarding Alterations. Tenant's Rooftop Equipment and the installation thereof shall be subject to Landlord's prior written approval, which approval shall not be unreasonably withheld, delayed or conditioned. Among other reasons, Landlord may withhold approval if the installation or operation of Tenant's Rooftop Equipment could reasonably be expected to damage the structural integrity of the Building or to transmit vibrations or noise or cause other adverse effects beyond the Premises to an extent not customary in first class laboratory buildings, unless Tenant implements measures that are acceptable to Landlord in its reasonable discretion to avoid any such damage or transmission.

41.3 Tenant shall comply with any roof or roof-related warranties. Tenant shall obtain a letter from Landlord's roofing contractor within thirty (30) days after completion of any Tenant work on the rooftop stating that such work did not affect any such warranties. Tenant, at its sole cost and expense, shall inspect the Rooftop Installation Area at least annually, and correct any loose bolts, fittings or other appurtenances and repair any damage to the roof arising from the installation or operation of Tenant's Rooftop Equipment. Tenant shall not permit the installation, maintenance or operation of Tenant's Rooftop Equipment to violate any Applicable Laws or constitute a nuisance. Tenant shall pay Landlord within thirty (30) days after demand (a) all applicable taxes, charges, fees or impositions imposed on Landlord by Governmental Authorities as the result of Tenant's use of the Rooftop Installation Areas in excess of those for which Landlord would otherwise be responsible for the use or installation of Tenant's Rooftop Equipment and (b) the amount of any increase in Landlord's insurance premiums as a result of the installation of Tenant's Rooftop Equipment. Upon Tenant's written request to Landlord, Landlord shall use commercially reasonable efforts to cause other tenants to remedy any interference in the operation of Tenant's Rooftop Equipment arising from any such tenants' equipment installed after the applicable piece of Tenant's Rooftop Equipment; provided, however, that Landlord shall not be required to request that such tenants waive their rights under their respective leases.

41.4 If Tenant's Equipment (a) causes physical damage to the structural integrity of the Building, (b) interferes with any telecommunications, mechanical or other systems located at or near or servicing the Building or the Project that were installed prior to the installation of Tenant's Rooftop Equipment, (c) interferes with any other service provided to other tenants in the Building or the Project by rooftop or penthouse installations that were installed prior to the installation of Tenant's Rooftop Equipment or (d) interferes with any other tenants' business, in each case in excess of that permissible under Federal Communications Commission regulations, then Tenant shall cooperate with Landlord to determine the source of the damage or interference and promptly repair such damage and eliminate such interference, in each case at Tenant's sole cost and expense, within ten (10) business days after receipt of notice of such damage or interference (which notice may be oral; provided that Landlord also delivers to Tenant written notice of such damage or interference within twenty-four (24) hours after providing oral notice).

41.5 Landlord reserves the right to cause Tenant to relocate Tenant's Rooftop Equipment to comparably functional space on the roof or in the penthouse of the Building by giving Tenant prior written notice thereof. Landlord agrees to pay the reasonable costs thereof. Tenant shall arrange for the relocation of Tenant's Rooftop Equipment within sixty (60) days after receipt of Landlord's notification of such relocation. In the event Tenant fails to arrange for relocation within such sixty (60)-day period, Landlord shall have the right to arrange for the relocation of Tenant's Rooftop Equipment in a manner that does not unnecessarily interrupt or interfere with Tenant's use of the Premises for the Permitted Use.

42. Option to Extend Term. Tenant shall have one (1) option (the “Option”) to extend the Term by five (5) years with respect to either the entire Fourth Floor Premises or the entire Premises upon the following terms and conditions. Any extension of the Term pursuant to the Option shall be on all the same terms and conditions as this Lease, except as follows:

42.1 Base Rent at the commencement of the Option term shall equal the greater of the then-current Base Rent, or (b) the then-current fair market value for comparable office and laboratory space in the East Cambridge submarket of comparable age, quality, level of finish and proximity to amenities and public transit, and containing the systems and improvements present in the Premises as of the date that Tenant gives Landlord written notice of Tenant’s election to exercise the Option. Such fair market value shall be determined for such initial year of the Option term assuming the initial Base Rent shall be subject to 3% annual increases as provided in Section 8 of this Lease (and for the avoidance of doubt, such annual increase is not intended to double count, or to have the Base Rent during the Option term multiplied by an additional 3% in excess of market). Tenant may, no more than eighteen (18) months prior to the date the Term is then scheduled to expire, request Landlord’s estimate of the FMV for the Option term. Landlord shall, within fifteen (15) days after receipt of such request, give Tenant a written proposal of such FMV. If Tenant gives written notice to exercise the Option, such notice shall specify whether Tenant accepts Landlord’s proposed estimate of FMV. If Tenant does not accept the FMV, then the parties shall endeavor to agree upon the FMV, taking into account all relevant factors, including (v) the size of the Premises, (w) the length of the Option term, (x) rent in comparable buildings in the relevant submarket, including concessions offered to new tenants, such as free rent, tenant improvement allowances and moving allowances, (y) Tenant’s creditworthiness and (z) the quality and location of the Building and the Project. In the event that the parties are unable to agree upon the FMV within thirty (30) days after Tenant notifies Landlord that Tenant is exercising the Option, then either party may request that the same be determined as follows: a senior officer of a nationally recognized leasing brokerage firm with local knowledge of the East Cambridge laboratory/research and development leasing submarket (the “Baseball Arbitrator”) shall be selected and paid for jointly by Landlord and Tenant. If Landlord and Tenant are unable to agree upon the Baseball Arbitrator, then the same shall be designated by the local chapter of the Judicial Arbitration and Mediation Services or any successor organization thereto (the “JAMS”). The Baseball Arbitrator selected by the parties or designated by JAMS shall (y) have at least ten (10) years’ experience in the leasing of laboratory/research and development space in the East Cambridge submarket and (z) not have been employed or retained by either Landlord or Tenant or any affiliate of either for a period of at least ten (10) years prior to appointment pursuant hereto. Each of Landlord and Tenant shall submit to the Baseball Arbitrator and to the other party its determination of the FMV. The Baseball Arbitrator shall grant to Landlord and Tenant a hearing and the right to submit evidence. The Baseball Arbitrator shall determine which of the two (2) FMV determinations more closely represents the actual FMV. The arbitrator may not select any other FMV for the Premises other than one submitted by Landlord or Tenant. The FMV selected by the Baseball Arbitrator shall be binding upon Landlord and Tenant and shall serve as the basis for determination of Base Rent payable for the Option term. If, as of the commencement date of the Option term, the amount of Base Rent payable during the Option term shall not have been determined, then, pending such determination, Tenant shall pay Base Rent equal to the Base Rent payable with respect to the last year of the then-current Term. After the final determination of Base Rent payable for the Option term, the parties shall promptly execute a written amendment to this Lease specifying the amount of Base Rent to be paid during the Option term. Any failure of the parties to execute such amendment shall not affect the validity of the FMV determined pursuant to this Section.

42.2 The Option is not assignable separate and apart from this Lease.

42.3 The Option is conditional upon Tenant giving Landlord written notice of its election to exercise the Option at least fifteen (15) months prior to the end of the expiration of the then-current Term. Time shall be of the essence as to Tenant's exercise of the Option. Tenant assumes full responsibility for maintaining a record of the deadlines to exercise the Option. Tenant acknowledges that it would be inequitable to require Landlord to accept any exercise of the Option after the date provided for in this Section.

42.4 Notwithstanding anything contained in this Article to the contrary, Tenant shall not have the right to exercise the Option:

(a) During the time commencing from the date Landlord delivers to Tenant a written notice that Tenant is in default under any provisions of this Lease and continuing until Tenant has cured the specified default;

(b) At any time after any Default as described in Article 31 of the Lease (provided, however, that, for purposes of this Section 42.4(b), Landlord shall not be required to provide Tenant with notice of such Default other than any notice from Landlord that may be required under Article 31 of this Lease) and continuing until Tenant cures any such Default, if such Default is susceptible to being cured;

(c) In the event that Tenant has defaulted in the performance of its obligation to pay Base Rent, Operating Expenses or the Property Management Fee two (2) or more times during the twelve (12)-month period immediately prior to the date that Tenant intends to exercise the Option, whether or not Tenant has cured such defaults;

(d) In the event that Tenant has been in Default of any material nonmonetary obligations under this Lease or its obligations to pay Additional Rent (excluding Operating Expenses and the Property Management Fee) two (2) or more times during the twelve (12) month period immediately prior to the date that Tenant intends to exercise the Option, whether or not Tenant has cured such defaults; and

(e) At the time Tenant exercises its Option and as of the last day of the initial Term, Tenant has not (i) subleased more than 50% of the Rentable Area of the Premises, and (ii) assigned this Lease, except in connection with an Exempt Transfer.

42.5 The period of time within which Tenant may exercise the Option shall not be extended or enlarged by reason of Tenant's inability to exercise such Option because of the provisions of Section 42.4.

42.6 All of Tenant's rights under the provisions of the Option shall terminate and be of no further force or effect even after Tenant's due and timely exercise of the Option if, after such exercise, but prior to the commencement date of the new term, (a) Tenant fails to pay to Landlord a monetary obligation of Tenant for a period of twenty (20) days after written notice from Landlord to Tenant, (b) Tenant fails to commence to cure a default (other than a monetary default) within thirty (30) days after the date Landlord gives notice to Tenant of such default or (c) Tenant has been in Default under this Lease two (2) or more times and a service or late charge under Section 31.1 has become payable for any such Default, whether or not Tenant has cured such Defaults.

43. Right of First Offer. Subject to any other parties' pre-existing rights with respect to Available ROFO Premises (as defined below), Tenant shall have a one-time right of first offer ("ROFO") as to any rentable premises on the fourth (4th) floor in the Building for which Landlord is seeking a Tenant ("Available ROFO Premises"); provided, however, that in no event shall Landlord be required to lease any Available ROFO Premises to Tenant for any period past the date on which this Lease expires or is terminated pursuant to its terms. To the extent that Landlord renews or extends a then-existing lease with any then-existing tenant or subtenant of any space, or enters into a new lease with such then-existing tenant or subtenant, the affected space shall not be deemed to be Available ROFO Premises. In the event Landlord intends to market and lease Available ROFO Premises (and in any event prior to Landlord's marketing thereof), Landlord shall provide written notice thereof to Tenant (the "Notice of Availability"). The Notice of Availability shall include as Base Rent for the Available ROFO Premises, the then-current fair market value for comparable office and laboratory space in the East Cambridge submarket of comparable age, quality, level of finish and proximity to amenities and public transit, and containing the systems and improvements present in the Premises.

43.1 Within ten (10) business days following its receipt of a Notice of Availability, Tenant shall advise Landlord in writing whether Tenant elects to lease all (not just a portion) of the Available ROFO Premises upon the terms and conditions set forth therein. If Tenant fails to notify Landlord of Tenant's election within such ten (10) business day period, then Tenant shall be deemed to have elected not to lease the Available ROFO Premises.

43.2 If Tenant timely notifies Landlord that Tenant elects to lease all of the Available ROFO Premises upon the terms and conditions set forth in the Notice of Availability ("Tenant's Notice") (provided that Tenant shall be required to lease the Available ROFO Premises for at least the remainder of the then-current Term), then Landlord shall lease the Available ROFO Premises to Tenant upon the terms and conditions set forth in the Notice of Availability, and Tenant's ROFO under this Lease shall expire.

43.3 If (a) Tenant notifies Landlord that Tenant elects not to lease the Available ROFO Premises, or (b) Tenant fails to notify Landlord of Tenant's election within the ten (10) business day period described above, then Landlord shall have the right to consummate a lease of the Available ROFO Premises at base rent not less than ninety-five percent (95%) of that stated in Tenant's Offer, if applicable, and Tenant's ROFO under this Lease shall expire.

43.4 Notwithstanding anything in this Article to the contrary, Tenant shall not exercise the ROFO during such period of time that Tenant is in Default under any provision of this Lease. Any attempted exercise of the ROFO during a period of time in which Tenant is so in Default shall be void and of no effect.

43.5 Notwithstanding anything in this Lease to the contrary, Tenant shall not assign or transfer the ROFO, either separately or in conjunction with an assignment or transfer of Tenant's interest in the Lease, without Landlord's prior written consent (except in connection with an Exempt Transfer), which consent Landlord shall not unreasonably withhold, condition, or delay if such consent is requested in connection with a proposed assignment to an assignee that has the financial capacity and wherewithal to perform the remaining obligations of Tenant under this Lease.



43.6 If Tenant exercises the ROFO, Landlord does not guarantee that the Available ROFO Premises will be available on the anticipated commencement date for the Lease as to such Premises due to a holdover by the then-existing occupants of the Available ROFO Premises or for any other reason beyond Landlord's reasonable control.

43.7 At the time Tenant exercises the ROFO, Tenant shall not have subleased more than thirty percent (30%) of the Premises or assigned this Lease, except in connection with an Exempt Transfer.

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IN WITNESS WHEREOF, the parties hereto have executed this Lease as a sealed Massachusetts instrument as of the date first above written.

LANDLORD:

BMR-ROGERS STREET LLC,  
a Delaware limited liability company

By: /s/ William Kane

Name: William Kane

Title: Senior Vice President, East Coast Leasing

TENANT:

GENERATION BIO CO.,  
a Delaware corporation

By: /s/ Geoffrey McDonough

Name: Geoffrey McDonough

Title: Chief Executive Officer

**FIRST AMENDMENT TO LEASE**

THIS FIRST AMENDMENT TO LEASE (this "Amendment") is entered into as of this 12<sup>th</sup> day of July, 2019 (the "Effective Date"), by and between BMR-ROGERS STREET LLC, a Delaware limited liability company ("Landlord"), and GENERATION BIO CO., a Delaware corporation ("Tenant").

**RECITALS**

A. WHEREAS, Landlord and Tenant are parties to that certain Lease dated as of August 2, 2018 (as the same may have been amended, supplemented or modified from time to time, the "Existing Lease"), whereby Tenant leases certain Premises from Landlord located at 301 Binney Street, Cambridge, Massachusetts;

B. WHEREAS, Landlord and Tenant desire to increase the tenant improvement allowance and Base Rent, and substitute the Premises plans attached to the Existing Lease; and

C. WHEREAS, Landlord and Tenant desire to modify and amend the Existing Lease only in the respects and on the conditions hereinafter stated.

**AGREEMENT**

NOW, THEREFORE, Landlord and Tenant, in consideration of the mutual promises contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, and intending to be legally bound, agree as follows:

44. Definitions. For purposes of this Amendment, capitalized terms shall have the meanings ascribed to them in the Existing Lease unless otherwise defined herein. The Existing Lease, as amended by this Amendment, is referred to collectively herein as the "Lease." From and after the Effective Date, the term "Lease," as used in the Existing Lease, shall mean the Existing Lease, as amended by this Amendment.

45. Base Rent for First Floor Premises. Notwithstanding anything to the contrary set forth in the Existing Lease, from and after the Effective Date, monthly and annual installments of Base Rent for the First Floor Premises as of July 1, 2019 and through the Term of the Lease shall be as set forth in the chart below, which chart shall replace in its entirety the chart set forth in Section 2.4 of the Existing Lease. Furthermore, the provisions of Article 8 of the Existing Lease shall not apply to the Base Rent for the First Floor Premises for the period commencing on July 1, 2019 and ending on April 30, 2029.

| <u>Dates</u>        | <u>Square Feet of Rentable Area</u> | <u>Base Rent per Square Foot of Rentable Area</u> | <u>Monthly Base Rent</u> | <u>Annual (or Annualized) Base Rent</u> |
|---------------------|-------------------------------------|---|--------------------------|---|
| 7/1/2019-7/17/2019  | 19,310                              | \$ 6.18   | \$ 9,944.65              | \$ 5,558.11                             |
| 7/18/2019-7/17/2020 | 19,310                              | \$ 89.18 annually                                 | \$143,505.48             | \$1,722,065.80                          |
| 7/18/2020-7/17/2021 | 19,310                              | \$ 91.67 annually                                 | \$147,512.31             | \$1,770,147.70                          |
| 7/18/2021-7/17/2022 | 19,310                              | \$ 94.23 annually                                 | \$151,631.78             | \$1,819,581.30                          |
| 7/18/2022-7/17/2023 | 19,310                              | \$ 96.88 annually                                 | \$155,896.07             | \$1,870,752.80                          |
| 7/18/2023-7/17/2024 | 19,310                              | \$ 99.60 annually                                 | \$160,273.00             | \$1,923,276.00                          |
| 7/18/2024-7/17/2025 | 19,310                              | \$102.40 annually                                 | \$164,778.67             | \$1,977,344.00                          |
| 7/18/2025-7/17/2026 | 19,310                              | \$105.28 annually                                 | \$169,413.07             | \$2,032,956.80                          |
| 7/18/2026-7/17/2027 | 19,310                              | \$108.26 annually                                 | \$174,208.38             | \$2,090,500.60                          |
| 7/18/2027-7/17/2028 | 19,310                              | \$111.32 annually                                 | \$179,132.43             | \$2,149,589.20                          |
| 7/18/2028-4/30/2029 | 19,310                              | \$114.48 annually                                 | \$184,217.40*            | \$2,210,608.80*                         |

\* Tenant to pay pro-rated amount for partial month/year

46. Base Rent for Fourth Floor Premises. Notwithstanding anything to the contrary set forth in the Existing Lease, from and after the Effective Date, monthly and annual installments of Base Rent for the Fourth Floor Premises as of July 1, 2019 and through the Term of the Lease shall be as set forth in the chart below, which chart shall replace in its entirety the chart set forth in Section 2.3 of the Existing Lease. Furthermore, the provisions of Article 8 of the Existing Lease shall not apply to the Base Rent for the Fourth Floor Premises for the period commencing on July 1, 2019 and ending on April 30, 2029.

| <u>Dates</u>       | <u>Square Feet of Rentable Area</u> | <u>Base Rent per Square Foot of Rentable Area</u> | <u>Monthly Base Rent</u> | <u>Annual (or Annualized) Base Rent</u> |
|--------------------|-------------------------------------|---|--------------------------|---|
| 7/1/2019-4/3/2020  | 52,252                              | \$ 92.18 annually                                 | \$401,382.45             | \$3,639,200.85                          |
| 4/4/2020-4/3/2021  | 52,252                              | \$ 94.76 annually                                 | \$412,616.63             | \$4,951,399.52                          |
| 4/4/2021-4/3/2022  | 52,252                              | \$ 97.42 annually                                 | \$424,199.15             | \$5,090,389.84                          |
| 4/4/2022-4/3/2023  | 52,252                              | \$100.15 annually                                 | \$436,086.48             | \$5,233,037.80                          |
| 4/4/2023-4/3/2024  | 52,252                              | \$102.97 annually                                 | \$448,365.70             | \$5,380,388.44                          |
| 4/4/2024-4/3/2025  | 52,252                              | \$105.88 annually                                 | \$461,036.81             | \$5,532,441.76                          |
| 4/4/2025-4/3/2026  | 52,252                              | \$108.87 annually                                 | \$474,056.27             | \$5,688,675.24                          |
| 4/4/2026-4/3/2027  | 52,252                              | \$ 111.95 annually                                | \$487,467.62             | \$5,849,611.40                          |
| 4/4/2027-4/3/2028  | 52,252                              | \$115.12 annually                                 | \$501,270.85             | \$6,015,250.24                          |
| 4/4/2028-4/3/2029  | 52,252                              | \$118.39 annually                                 | \$515,509.52             | \$6,181,877.20                          |
| 4/4/2029-4/30/2029 | 52,252                              | \$121.76 annually                                 | \$530,183.63*            | \$6,362,203.52*                         |

\* Tenant to pay pro-rated amount for partial month/year

47. First Floor Additional TI Allowance.

47.1 Notwithstanding the provisions of the Existing Lease, Landlord shall provide to Tenant an additional tenant improvement allowance for additional work to be performed to the First Floor Premises in accordance with the plans attached as Exhibit B to this Amendment (the “Additional First Floor Tenant Improvements”) in an amount equal to Two Million Eight Hundred Ninety-Six Thousand Five Hundred and 00/100 Dollars (\$2,896,500.00) (based upon One Hundred Fifty and No/100 Dollars (\$150.00) per square foot of Rentable Area of the First Floor Premises) (the “Additional First Floor TI Allowance”), which funds shall be available to Tenant upon the Effective Date. Tenant shall, at its sole cost and expense (but subject to the Additional First Floor TI Allowance) cause the Additional First Floor Improvements to be constructed in the First Floor Premises. The Additional First Floor Premises TI Allowance may be applied to the costs of (a) construction, including demising the Premises and submetering, (b) actual out-of-pocket costs incurred in connection with project review by Landlord, (c) commissioning of mechanical, electrical and plumbing systems by a licensed, qualified commissioning agent hired by Tenant, and review of such party’s commissioning report by a licensed, qualified

commissioning agent hired by Landlord, (d) space planning, architect, engineering and other related professional or consulting services performed by third parties unaffiliated with, Tenant, (e) building permits and other taxes, fees, charges and levies by Governmental Authorities for permits, approvals or for inspections of the Tenant Improvements, and (f) costs and expenses for labor, material, equipment and fixtures. In no event shall the Additional First Floor TI Allowance be used for (u) the cost of work that is not authorized by the Approved Plans for the Additional First Floor Tenant Improvements or the First Floor Tenant Improvements or otherwise approved in writing by Landlord, (v) payments to Tenant or any affiliates of Tenant, (w) the purchase of any furniture, personal property or other non-building system equipment, (x) costs resulting from any default by Tenant of its obligations under the Lease, (y) costs that are recoverable by Tenant from a third party (e.g., insurers, warrantors, or tortfeasors), or (z) costs related to the Fourth Floor Tenant Improvements. In addition, and in addition to the First Floor A/E Allowance described by Section 4.5 of the Existing Lease, Landlord shall provide an allowance to Tenant to be used solely for architectural and engineering costs related to the First Floor Premises in an amount not to exceed Two Thousand Three Hundred Seventeen and 20/100 Dollars (\$2,317.20) (based upon 12/100 Dollars (\$0.12) per square foot of Rentable Area for the First Floor Premises) (the "Additional First Floor A/E Allowance"; together with the Additional First Floor TI Allowance, the "Additional First Floor Allowance").

47.2 Tenant shall have until the First Floor TI Deadline to submit Fund Requests (as defined in the Work Letter) to Landlord for disbursement of the unused portion of the Additional First Floor Allowance, after which date Landlord's obligation to fund any such costs for which Tenant has not submitted a Fund Request to Landlord shall expire. In no event shall any unused Additional First Floor Allowance entitle Tenant to a credit against Rent payable under the Lease.

47.3 For the avoidance of doubt, the provisions of the Work Letter shall govern the Additional First Floor Tenant Improvements and the disbursement of the Additional First Floor Allowance, except to the extent that such provisions are directly inconsistent with the provisions of this Amendment. Landlord has previously approved (x) the construction plans for the First Floor Tenant Improvements pursuant to that certain letter dated May 22, 2019 (subject to the conditions set forth therein), and (y) the schedule and budget for the Additional First Floor Tenant Improvements pursuant to that certain letter dated May 23, 2019. Tenant shall submit separate Fund Requests for the Additional First Floor Allowance. As of the Effective Date, the term "Tenant Improvements" as used in the Existing Lease (including the Work Letter) shall be deemed to mean collectively, the Fourth Floor Tenant Improvements, the First Floor Tenant Improvements, and the Additional First Floor Tenant Improvements. The Existing Lease (including the Work Letter) shall be amended to include: (i) the Additional First Floor Tenant Improvements in any reference to the First Floor Tenant Improvements and the Fourth Floor Tenant Improvements, as applicable, and (ii) the Additional First Floor Allowance in any reference to the First Floor Allowance and the Fourth Floor Allowance, as applicable. The Additional First Floor Tenant Improvements shall not constitute a Change to the Tenant Improvements (as such term is defined in Section 2.3 of the Work Letter).

48. Premises. The parties acknowledge and agree that the Premises plans attached to the Existing Lease as Exhibit A are hereby deleted in their entirety and replaced with the plans attached hereto and incorporated herein as Exhibit A.

49. Notices. Tenant confirms that, notwithstanding anything in the Lease to the contrary, notices delivered to Tenant pursuant to the Lease should be sent to:

Generation Bio Co.  
301 Binney Street  
Cambridge, Massachusetts 02142  
Attn: Chief Financial Officer

50. Broker. Tenant represents and warrants to Landlord that Tenant has not dealt with any broker or agent in the negotiation for or the obtaining of this Amendment other than CBRE, Inc. (the "Broker") and agrees to reimburse, indemnify, save, defend (at Landlord's option and with counsel reasonably acceptable to Landlord, at Tenant's sole cost and expense) and hold harmless the Landlord Parties for, from and against any and all cost or liability for compensation claimed by any such broker or agent employed or engaged by Tenant or claiming to have been employed or engaged by Tenant other than Broker. Landlord represents and warrants to Tenant that Landlord has not dealt with any broker or agent in the negotiation for or the obtaining of this Amendment other than Broker and agrees to reimburse, indemnify, save, defend (at Tenant's option and with counsel reasonably acceptable to Tenant, at Landlord's sole cost and expense) and hold harmless the Tenant Parties for, from and against any and all cost or liability for compensation claimed by any such broker or agent employed or engaged by Landlord or claiming to have been employed or engaged by Landlord other than Broker. The parties acknowledge and agree that no commissions are due to Broker based on this Amendment.

51. Effect of Amendment. Except as modified by this Amendment, the Existing Lease and all the covenants, agreements, terms, provisions and conditions thereof shall remain in full force and effect and are hereby ratified and affirmed. In the event of any conflict between the terms contained in this Amendment and the Existing Lease, the terms herein contained shall supersede and control the obligations and liabilities of the parties.

52. Successors and Assigns. Each of the covenants, conditions and agreements contained in this Amendment shall inure to the benefit of and shall apply to and be binding upon the parties hereto and their respective heirs, legatees, devisees, executors, administrators and permitted successors and assigns and sublessees. Nothing in this section shall in any way alter the provisions of the Lease restricting assignment or subletting.

53. Miscellaneous. This Amendment becomes effective only upon execution and delivery hereof by Landlord and Tenant. The captions of the paragraphs and subparagraphs in this Amendment are inserted and included solely for convenience and shall not be considered or given any effect in construing the provisions hereof. All exhibits hereto are incorporated herein by reference. Submission of this instrument for examination or signature by Tenant does not constitute a reservation of or option for a lease, and shall not be effective as a lease, lease amendment or otherwise until execution by and delivery to both Landlord and Tenant.

54. Authority. Tenant guarantees, warrants and represents that the individual or individuals signing this Amendment on Tenant's behalf have the power, authority and legal capacity to sign this Amendment on behalf of and to bind all entities, corporations, partnerships, limited liability companies, joint venturers or other organizations and entities on whose behalf such individual or individuals have signed. Landlord guarantees, warrants and represents that the individual or individuals signing this Amendment on Landlord's behalf have the power, authority and legal capacity to sign this Amendment on behalf of and to bind all entities, corporations, partnerships, limited liability companies, joint venturers or other organizations and entities on whose behalf such individual or individuals have signed.

55. Counterparts; Facsimile and PDF Signatures. This Amendment may be executed in one or more counterparts, each of which, when taken together, shall constitute one and the same document. A portable document format (PDF) signature on this Amendment shall be equivalent to, and have the same force and effect as, an original signature.

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IN WITNESS WHEREOF, Landlord and Tenant have executed this Amendment as of the Effective Date first above written.

**LANDLORD:**

BMR-ROGERS STREET LLC,  
a Delaware limited liability company

By: /s/ Kevin M. Simonsen  
Name: Kevin M. Simonsen  
Title: Sr. VP, General Counsel & Secretary

**TENANT:**

GENERATION BIO CO.,  
a Delaware corporation

By: /s/ Geoffrey McDonough  
Name: Geoffrey McDonough  
Title: Chief Executive Officer

Certain identified information has been marked in the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed.  
Double asterisks denote omissions.

## EXCLUSIVE LICENSE AGREEMENT

This Agreement, effective as of June 23, 2017 (the “Effective Date”), is between the University of Massachusetts (“University”), a not-for-profit, public institution of higher education of the Commonwealth of Massachusetts, established by Chapter 75 of the Massachusetts General Laws, as represented by and solely on behalf of its Medical School (Worcester campus), and Torus Therapeutics, Inc. (“Company”), a Delaware corporation.

### RECITALS

WHEREAS, University owns the intellectual property described in University’s docket UNIVERSITY [\*\*] entitled, “[\*\*]” and associated patent applications, as further described in Exhibit A;

WHEREAS, Company is engaged in business relating to the development and commercialization of products that use or incorporate University’s intellectual property rights and has the capability of developing commercial applications of the intellectual property;

WHEREAS, Company desires to obtain an exclusive license to University’s intellectual property rights, and University is willing to grant an exclusive license to its intellectual property rights under the following conditions so that these intellectual property rights may be developed to their fullest and the benefits enjoyed by the general public; and

WHEREAS, the license that is granted in this Agreement promotes the development of publicly funded intellectual property to practical application for the public good.

THEREFORE, University and Company agree as follows:

#### 1. Definitions.

1.1 “Affiliate” means an entity that controls, is controlled by, or is under common control with a party to this Agreement. The term “control” as used in the preceding sentence means possession of the power to direct or call for the direction of the management and policies of an entity, whether through ownership of a majority of the outstanding voting securities, by contract, or otherwise.

1.2 “Biological Materials” means tangible biological materials that are necessary for the effective exercise of the Patent Rights, which materials are described on Exhibit B, as well as tangible materials that are produced by or on behalf of Company, its Affiliates or Sublicensees through use of the original materials, including, for example, any progeny derived from a cell line, monoclonal antibodies produced by hybridoma cells, DNA or RNA replicated from isolated DNA or RNA, recombinant proteins produced through use of isolated DNA or RNA, and substances purified from a source material included in the original materials (such as, recombinant proteins isolated from a cell extract or supernatant by non-proprietary affinity purification methods).

1.3. “Confidential Information” means any confidential or proprietary information furnished by one party (the “Disclosing Party”) to the other party (the “Receiving Party”) in connection with this Agreement that is specifically designated as confidential, as further described in Article 7.

1.4. “Field” means the treatment, prevention or palliation of any human disease, disorder or condition.

1.5. “Licensed Product” means any product, the manufacture, use, offer for sale, sale or importation of which would, in the absence of a license hereunder, infringe a Valid Claim within the Patent Rights.

1.6 “Licensed Processes” means any method or process of manufacture, the use or performance of which would infringe a Valid Claim within the Patent Rights.

1.7. “Net Sales” means the gross amount billed or invoiced on sales of Licensed Products by Company, its Affiliates and Sublicensees, less the following: (a) customary trade, quantity, or cash discounts to non-affiliated customers, brokers or agents to the extent actually allowed and taken; (b) price reductions, rebates and chargeback payments granted to managed health care organizations, pharmacy benefit managers (or equivalents thereof), national, state/provincial, local and other governments, their agencies and purchasers and reimbursers, and to trade customers (including Medicare, Medicaid, managed care and similar types of rebates and chargebacks); (c) amounts repaid or credited by reason of rejection or return; (d) to the extent separately stated on purchase orders, invoices, or other documents of sale, any taxes or other governmental charges levied on the production, sale, transportation, delivery, or use of a Licensed Product which is paid by or on behalf of Company, its Affiliates and Sublicensees; and (e) outbound transportation costs prepaid or allowed and costs of insurance in transit.

In any transfers of Licensed Products between any of Company and Affiliates and Sublicensees, Net Sales are calculated based on the final sale of the Licensed Product to an independent third party. If Company or an Affiliate or Sublicensee receives non-monetary consideration for any Licensed Products, Net Sales are calculated based on the fair market value of that consideration. If Company or its Affiliates or Sublicensees uses or disposes of a Licensed Product in the provision of a commercial service, the Licensed Product is sold and the Net Sales are calculated based on the sales price of the Licensed Product to an independent third party during the same Royalty Period or, in the absence of sales, on the fair market value of the Licensed Product as determined by the parties in good faith.

1.8. “Patent Rights” means the United States patent applications listed in Exhibit A, patent applications covering invention disclosures listed in Exhibit A, if any, and any patent applications validly claiming priority to the patent applications listed in Exhibit A (including any divisional, continuation, or continuation-in-part of those patent applications) to the extent the claims are directed to subject matter specifically described therein as well as any patents issued on these patent applications and any reissues or reexaminations or extensions of the patents (including any patent term extension and supplemental protection certificates), and any foreign counterparts to any of the foregoing.

1.9. "Royalty Period" means, on a Licensed Product-by-Licensed Product and country-by-country basis, the partial calendar quarter commencing on the date on which the first Licensed Product is sold or used and every complete or partial calendar quarter thereafter during which either (a) this Agreement remains in effect or (b) Company has the right to complete and sell work-in-progress and inventory of Licensed Products pursuant to Section 8.5.

1.10. "Sublicense Agreement" means any agreement or transaction in which Company grants, or promises to grant, rights to the Patent Rights pursuant to Section 2.2. For the avoidance of doubt, any agreement or transaction that confers or promises to confer rights under this Agreement, including, but not limited to, an option for a Sublicense Agreement shall be deemed to be a Sublicense Agreement.

1.11. "Sublicense Income" any payments or other value that Company receives from a third party in consideration of a Sublicense Agreement or other promise of the rights granted to Company under the Patent Rights, including without limitation, license fees, option fees, equity, milestone payments, and license maintenance fees, but excluding the following payments: (a) payments made in consideration for the issuance of equity or debt securities of Company at fair market value, (b) payments specifically committed to the future development of Licensed Products and/or Licensed Processes, reimbursement of patent prosecution, defense, enforcement and maintenance costs, (c) payments for milestones that are substantially similar to those payable by Company to University for Licensed Products and (d) royalties or other profit sharing.

1.12. "Sublicensee" means any permitted sublicensee of the rights granted Company under this Agreement, as further described in Section 2.2.

1.13 "Territory" means all countries of the world.

1.14. "Valid Claim" means mean any (i) claim that has been pending for no more than [\*\*] from the earliest substantive office action or (ii) issued claim of any Patent Rights that has not been permanently revoked, nor held unenforceable or invalid by a decision of a court or other governmental agency of competent jurisdiction that is unappealable, or unappealed in the time allowed for appeal.

## 2. Grant of Rights

2.1. License Grant. University grants to Company an exclusive license to University's rights in and to the Patent Rights for the sole purpose of researching, developing, manufacturing, having manufactured, using, offering for sale and selling and importing Licensed Products in the Field in the Territory and to practice and have practiced any Licensed Processes in the Field in the Territory.

2.2. Sublicenses. Company may grant sublicenses of its rights under Section 2.1. All Sublicense Agreements executed by Company pursuant to this Section 2.2 shall expressly bind the Sublicensee to the terms of this Agreement applicable to Sublicensees. Company shall promptly furnish University with a fully executed, un-redacted copy of any Sublicense Agreement, or any other agreement (option, research and development, etc., other than agreements that grant rights only to the extent necessary to enable contract research organizations, contract manufacturing organizations and other services providers to perform services for Company, its Affiliates and Sublicensees and for which Company, its Affiliates and Sublicensees do not receive any consideration other than the contracted service) that Company executes with a third party that promises rights to the Patent Rights. Any agreement provided by Company to University hereunder shall constitute Company's Confidential Information, and University shall not disclose any such agreement to any third party, except as required by law or regulation. For the avoidance of doubt, the audit rights described in Section 5.5 shall apply to all Sublicense Agreements in order for University to monitor compliance with this Agreement.

### 2.3. Retained Rights.

(a) University. University retains the right to use the Patent Rights for academic research, teaching, and non-commercial patient care, without payment of compensation to Company. University may license its retained rights under this Subsection 2.3(a) to research collaborators of University faculty members, post-doctoral fellows, and students.

(b) Federal Government. If the federal government has funded any invention claimed in the Patent Rights, this Agreement and the grant of any rights in Patent Rights are subject to the federal law set forth in 35 U.S.C. §§ 201-211 and the regulations promulgated thereunder, as amended, or any successor statutes or regulations. Company acknowledges that these statutes and regulations reserve to the federal government a royalty-free, non-exclusive, non-transferrable license to practice any government-funded invention claimed in the Patent Rights. If any term of this Agreement fails to conform to those laws and regulations, the relevant term is invalid, and the parties shall modify the term pursuant to Section 10.11.

### 3. Company Obligations Relating to Commercialization.

3.1. Diligence Requirements. Company shall use diligent efforts or cause its Affiliates and Sublicensees to use diligent efforts to develop Licensed Products and to introduce Licensed Products into the commercial market. Thereafter, Company or its Affiliates or Sublicensees shall make Licensed Products reasonably available to the public. Specifically, Company shall fulfill the following obligations:

(a) Prior to execution of the License Agreement, Licensee shall furnish University with a written research and development plan under which Licensee intends to develop Licensed Products.

(b) Within [\*\*] after the start of each calendar year, Licensee shall furnish University with a written report on the progress of its efforts during the prior year to develop and commercialize Licensed Products, including without limitation research and development efforts, efforts to obtain regulatory approval, and marketing efforts. The report shall also contain a discussion of intended efforts and general, non-binding guidance on sales for the current year.

(c) Within [\*\*] after the Effective Date, Licensee, its Affiliates or Sublicensees shall [\*\*].

(d) Within [\*\*] after the Effective Date, Licensee, its Affiliates or Sublicensees shall [\*\*].

(e) Within [\*\*] after the Effective Date, Licensee, its Affiliates or Sublicensees shall [\*\*].

(f) Within [\*\*] after the Effective Date, Licensee, its Affiliates or Sublicensees shall [\*\*].

(g) Within [\*\*] after [\*\*], Licensee, its Affiliates or Sublicensees shall [\*\*].

3.2. Failure to Fulfill Diligence Requirements. If University determines that Company has not fulfilled its obligations under Subsections 3.1(b)-3.1(g), University shall furnish Company with written notice of its determination. Within [\*\*] after receipt of the notice, Company shall either (a) respond to such notice with evidence that the relevant obligation has been fulfilled, (b) fulfill the relevant obligation, or (c) negotiate with University a mutually acceptable schedule of revised diligence obligations, which University shall not unreasonably decline to accept, failing which University may, immediately upon written notice to Company, terminate this Agreement or convert the exclusive license to a non-exclusive license.

### 3.3. Indemnification.

(a) Indemnity. Company shall indemnify, defend, and hold harmless University and its trustees, officers, faculty, students, employees, and agents and their respective successors, heirs and assigns (the "Indemnitees"), against any liability, damage, loss, or expense (including reasonable attorneys' fees and expenses of litigation) incurred by or imposed upon any of the Indemnitees in connection with any third party claims, suits, actions, demands or judgments arising out of any theory of liability (including without limitation actions in the form of tort, warranty, or strict liability and regardless of whether the action has any factual basis) concerning any product, process, or service that is made, used, or sold pursuant to any right or license granted under this Agreement. However, indemnification does not apply to any liability, damage, loss, or expense to the extent directly attributable to (i) the gross negligence or intentional misconduct of the Indemnitees or (ii) the settlement of a claim, suit, action, or demand by Indemnitees without the prior written approval of Company.

(b) Procedures. The Indemnitees agree to provide Company with prompt written notice of any claim, suit, action, demand, or judgment for which indemnification is sought under this Agreement. Company agrees, at its own expense, to provide attorneys reasonably acceptable to University to defend against any claim. The Indemnitees shall cooperate fully with Company in the defense and will permit Company to conduct and control the defense and the disposition of the claim, suit, or action (including all decisions relative to litigation, appeal, and settlement). However, any Indemnitee may retain its own counsel, at the expense of Company, if representation of the Indemnitee by the counsel retained by Company would be inappropriate because of actual or potential conflicts in the interests of the Indemnitee and any other party represented by that counsel. Company agrees to keep University informed of the progress in the defense and disposition of the claim and to consult with University regarding any proposed settlement.

(c) Insurance. Company shall maintain insurance or self-insurance that is reasonably adequate to fulfill any potential obligation to the Indemnitees, but not less than [\*\*] dollars (\$[\*\*]) for injuries to any one person arising out of a single occurrence and [\*\*] dollars (\$[\*\*]) for injuries to all persons arising out of a single occurrence, provided that Company shall increase the coverage to at least [\*\*] dollars (\$[\*\*]) for injuries to all persons arising out of a single occurrence immediately prior to commencing clinical trials with a Licensed Product. Company shall provide University with written evidence of insurance or self-insurance. Company shall continue to maintain the insurance or self-insurance after the expiration or termination of this Agreement while Company, its Affiliate or Sublicensee continues to make, use, or sell a Licensed Product and thereafter for [\*\*].

3.4. Use of University Name. In accordance with Section 7.2., Company and its Affiliates and Sublicensees may not use the name “University of Massachusetts” or any variation of that name in connection with the marketing or sale of any Licensed Products.

3.5. Marking of Licensed Products. To the extent commercially feasible and consistent with prevailing business practices, Company shall mark and shall cause its Affiliates and Sublicensees to mark all Licensed Products that are manufactured or sold under this Agreement with the number of each issued patent under the Patent Rights that applies to a Licensed Product.

3.6. Compliance with Law. Company shall comply with, and shall ensure that its Affiliates and Sublicensees comply with, all local, state, federal, and international laws and regulations relating to the development, manufacture, use, and sale of Licensed Products. Company expressly agrees to comply with the following:

(a) Company or its Affiliates or Sublicensees shall obtain all necessary approvals from the United States Food & Drug Administration and any similar foreign governmental authorities in which Company or Affiliate or Sublicensee intends to make, use, or sell Licensed Products.

(b) Company and its Affiliates and Sublicensees shall comply with all United States laws and regulations controlling the export of commodities and technical data, including without limitation all Export Administration Regulations of the United States Department of Commerce. Among other things, these laws and regulations prohibit or require a license for the export of certain types of commodities and technical data to specified countries and foreign nationals. Company hereby gives written assurance that it will comply with and will cause its Affiliates and Sublicensees to comply with all United States export control laws and regulations, that it bears sole responsibility for any violation of those laws and regulations by itself or its Affiliates or Sublicensees, and that it will indemnify, defend, and hold University harmless (in accordance with Section 3.3.) for the consequences of any violation.

(c) If any invention claimed in the Patent Rights has been funded by the United States government, and only to the extent required by applicable laws and regulations, Company agrees that any Licensed Products used or sold in the United States will be manufactured substantially in the United States or its territories. Current law provides that if domestic manufacture is not commercially feasible under the circumstances, University may seek a waiver of this requirement from the relevant federal agency on behalf of Company.

#### 4. Consideration for Grant of Rights.

4.1. License Fee. In partial consideration of the rights granted Company under this Agreement, Company shall pay to University on the Effective Date a license fee of Forty Thousand US Dollars (\$40,000.00). This license fee payment is nonrefundable and is not creditable against any other payments due to University under this Agreement.

4.2. Equity Position. In partial consideration of the rights granted Company under this Agreement, within [\*\*] after such time as Company has issued and sold in one or more financing transactions from and after the Effective Date securities of the Company having an aggregate purchase price of at least \$[\*\*] and subject to the execution by University of a subscription agreement and the voting agreement entered into by the purchasers in the financing transaction that results in the issuance of shares hereunder (or if none, an agreement containing a customary initial public offering lock-up provision and drag-along provision), Company shall issue to University such number of shares of common stock of Company that constitutes [\*\*] percent ([\*\*]%) of the sum total of all common stock of the Company following such issuance (including for this purpose all shares of capital stock outstanding on an as-converted to common stock basis). For purposes of clarity, Company shall have no obligation to issue any such shares with respect to any securities issued and sold that generate proceeds in excess of such \$[\*\*], including securities sold in the financing transaction that results in the issuance of shares hereunder. Notwithstanding the foregoing, University's obligation to enter into agreements pursuant to this Section 4.2 shall be subject to any limitations relating to the University's status as an agency of the Commonwealth of Massachusetts, e.g., prohibition on indemnification, governing law, and jurisdiction. To the extent University has not held such stock for more than one year at such time, Company shall use commercially reasonable efforts to register the stock issued to University pursuant to this Subsection 4.2 as soon as possible following Company's initial public offering, subject to customary terms in connection with the registration.

4.3. License Maintenance Fee. Within [\*\*] of the beginning of each calendar year during the term of this Agreement, commencing on January 1, 2018, Company shall pay to University a fee of [\*\*] US Dollars (\$[\*\*]). This annual license maintenance fee is creditable against any earned royalty payments due to University in the same year.

4.4. Milestone Payments. Subject to Section 4.8 (Change In Consideration), Company shall pay University the following milestone payments within [\*\*] after the occurrence of each event for each Licensed Product as listed below (other than the [\*\*] milestone payment, which shall be payable only once and for which University shall be required to submit an invoice to Company for payment and as notification of such occurrence). One-time milestones are not payable for Follow-ons to Licensed Products, which shall be defined as Licensed Products that encode a



transgene with substantially similar protein function as a Licensed Product for which one-time milestone payments have already been paid (“Follow-ons”):

| Event  | Payment |
|--|---------|
| [**]   | [**]    |
| [**]   | [**]    |
| [**]   | [**]    |
| [**]   | [**]    |
| [**]   | [**]    |
| [**]   | [**]    |
| [**]   | [**]    |
| 1st [**] in Net Sales of the applicable Licensed Product in a single calendar year | [**]    |

Total milestones payable University per Licensed Product developed and/or commercialized under the Agreement, regardless of the number of indications in which such Licensed Product might be pursued or approved, shall not exceed \$762,500.

These milestone payments are nonrefundable and are not creditable against any other payments due to University under this Agreement. For each Licensed Product, Company shall make all milestone payments, even if an earlier milestone event has not occurred. For example, if Company proceeds from [\*\*] to [\*\*], the milestone payments for both [\*\*] are due upon achievement of the [\*\*] milestone event. Also, as further example, if Company uses a [\*\*] to [\*\*], then upon approval of the [\*\*], both the [\*\*] payments are due.

4.5. Royalties. Subject to Section 4.8 (Change in Consideration), the Company will pay a royalty on Net Sales of Licensed Products equal to [\*\*]% of all Net Sales of Licensed Products by the Company, its affiliates and its sublicensees on a Licensed Product-by-Licensed Product and country-by-country basis until the expiration of the last-to-expire Valid Claim of the Patent Rights covering such Licensed Product in such country; such royalty rate will be the maximum royalty rate payable on Net Sales of Licensed Products.

4.6. Minimum Royalty. Within [\*\*] after the beginning of each calendar year during the term of this Agreement, beginning in the year of FDA approval of a License Product Company shall pay to University a minimum royalty according to the following schedule:

- i. Years 1 – [\*\*]: \$[\*\*]
- ii. Years [\*\*] – expiration: \$[\*\*]

Company may credit the minimum royalty paid under this Section 4.6 against actual royalties due and payable for the same calendar year. Waiver of any minimum royalty payment by University is not a waiver of any subsequent minimum royalty payment. If Company fails to make any minimum royalty payment within the [\*\*] period, that failure is a material breach of its obligations under this Agreement, and University may terminate this Agreement in accordance with Section 8.3.

4.7. **Sublicense Income.** Company shall pay University the following percentages of all Allocated Sublicense Income as provided in the Table below. "Allocated Sublicense Income" means Sublicense Income reasonably allocable, as determined in good faith by Company, to the grant of a sublicense or other grant or promise of the rights granted to Company under the Patent Rights. Company shall pay to University either Allocated Sublicense Income multiplied by the relevant percentage below, or [\*\*]% of Sublicense Income, whichever is greater ("Sublicense Income Payments").

**Sublicense Income Table**

| <b>When Sublicense Agreement to Third Party is Executed</b> | <b>Percentage of Sublicense Income to be Paid to University</b> |
|---|---|
| Before [**] of the Effective Date                           | [**]%   |
| Between [**] and [**] of Effective Date                     | [**]%   |
| After the [**] of Effective Date                            | [**]%   |

Sublicense Income Payments are due within [\*\*] after the conclusion of the Royalty Period during which Company receives the relevant payment from the Sublicensee.

4.8. **Change in Consideration.** If a third party retains any right to practice or grant licenses under the Patent Rights as a co-owner or preexisting licensee (excluding retained non-commercial academic and government use rights), then, among other provisions within this Agreement, the following changes to the license consideration described above shall apply.

(a) Milestones payable under Section 4.4, above, shall each, individually, be adjusted downward by [\*\*]%, such that total milestones payable to University per Licensed Product developed and/or commercialized under this Agreement, regardless of the number of indications in which such Licensed Product might be pursued or approved, shall not exceed \$381,250.

(b) Royalties payable under Section 4.5 above, shall be adjusted downward by [\*\*]%, from [\*\*]% to [\*\*]%.

(c) Company's reimbursement obligation for Patent Expenses, as outlined in Section 6.3, will be divided by the number of co-owners and pre-existing licensees of the Patent Rights.

4.9. **Resolution of Potential Third Party Ownership Claims.**

(a) Each party agrees to notify the other party in writing after becoming aware of any claim of ownership or co-ownership by any third party in or to any of the Patent Rights, including any assertion of such a claim in any action or proceeding. [\*\*].

(b) If a third party is held to be a co-owner of any of the Patent Rights, and the license granted to Company in Section 2.1 is held to be invalid or otherwise ineffective with respect to such Patent Rights due to the withholding of consent by such co-owner(s) to the grant of such license and solely where such consent is legally required in a territory in order to grant a valid license, provided that Company pays, and/or continues to pay, University in accordance with the terms of this Agreement as if the license in such territory were in effect and binding, University hereby covenants not to enforce such Patent Rights against Company or any of Company's Affiliates or Sublicensees based on any making or having made, research, having researched, use or having used, sale or having sold, offering to sell, or importation of any Licensed Products in the Field or practice or having practiced any Licensed Processes in the Field by or on behalf of Company or any of Company's Affiliates or Sublicensees, for so long as this Agreement remains in force, and University further covenants not to consent or agree to, and not to grant or transfer any right permitting or enabling the undertaking of, any such enforcement by any third party. Should Company fail to make payments to University in accordance with the terms of this Agreement with respect to the applicable territory and fail to cure such failure to make payments within [\*\*] of notice from University thereof, the covenants granted under this section shall immediately terminate.

5. Royalty Reports; Payments; Records.

5.1. First Sale. Company shall report to University the date of first commercial sale of each Licensed Product within [\*\*] after occurrence in each country.

5.2. Reports and Payments.

(a) Within [\*\*] after the conclusion of each Royalty Period, Company shall deliver to University a report containing the following information:

(i) the number of Licensed Products sold to independent third parties in each country and the number of Licensed Products used by Company, its Affiliates and Sublicensees in the provision of services in each country;

(ii) the aggregate gross sales for each Licensed Product by Company, its Affiliates and Sublicensees during the applicable Royalty Period in each country;

(iii) a calculation of Net Sales for the applicable Royalty Period in each country, including a listing of applicable deductions;

(iv) total royalty payable on Net Sales in United States dollars, together with the exchange rates used for conversion; and

(v) Sublicense Income Payments due to University for the applicable Royalty Period from each Sublicensee.

(b) Concurrent with this report, Company shall remit to University any payment due for the applicable Royalty Period. If no royalties are due to University for any Royalty Period, the report shall so state.

5.3. Payments in United States Dollars. Company shall make all payments in United States dollars. Company shall convert foreign currency to United States dollars at the conversion rate existing in the United States (as reported in the *Wall Street Journal*) on the last working day of the calendar quarter preceding the applicable Royalty Period. Company may not deduct exchange, collection, or other charges.

5.4. Payments in Other Currencies. If by law, regulation, or fiscal policy of a particular country, conversion into United States dollars or transfer of funds of a convertible currency to the United States is restricted or forbidden, Company shall give University prompt written notice of the restriction within the sixty-day payment deadline described in Section 5.2. Company shall pay any amounts due University through whatever lawful methods University reasonably designates. However, if University fails to designate a payment method within [\*\*] after University is notified of the restriction, Company may deposit payment in local currency to the credit of University in a recognized banking institution selected by Company and identified by written notice to University, and that deposit fulfills all obligations of Company to University with respect to that payment.

5.5. Records. Company shall maintain and shall cause its Affiliates and Sublicensees to maintain complete and accurate records of Sublicense Agreements and Licensed Products that are made, used, or sold under this Agreement and any amounts payable to University in relation to Licensed Products with sufficient information to permit University to confirm the accuracy of any reports delivered to University under Section 5.2. The relevant party shall retain records relating to a given Royalty Period for at least [\*\*] after the conclusion of that Royalty Period, during which time University may, at its expense, cause its internal accountants or an independent, certified public accountant reasonably acceptable to Company to inspect records during normal business hours for the sole purpose of verifying any reports and payments delivered under this Agreement. The accountant may not disclose to University any information other than information relating to accuracy of reports and payments delivered under this Agreement. The parties shall reconcile any underpayment or overpayment within [\*\*] after the accountant delivers the results of the audit. If any audit performed under this Section 5.5 reveals an underpayment in excess of [\*\*] percent ([\*\*]%) for the period audited, Company shall bear the full cost of the audit. University may exercise its rights under this Section 5.5 only [\*\*] and only with reasonable prior notice to Company.

5.6. Late Payments. Any payments by Company that are not paid on or before the date payments are due under this Agreement bear interest at [\*\*]% per month calculated on the number of days that payment is delinquent.

5.7. Method of Payment. All payments under this Agreement should be made to the “University of Massachusetts” and sent to the address identified below. Each payment should reference this Agreement and identify the obligation under this Agreement that the payment satisfies.

5.8. Withholding and Similar Taxes. Royalty payments and other payments due to University under this Agreement may not be reduced by reason of any withholding or similar taxes applicable to payments to University. Therefore all amounts owed to University under this Agreement are net amounts and shall be grossed-up to account for any withholding taxes, value-added taxes or other taxes, levies or charges.

## 6. Patents and Infringement.

### 6.1. Responsibility for Patent Rights.

(a) University has primary responsibility at the expense of Company for the preparation, filing, prosecution, and maintenance of all Patent Rights, using patent counsel reasonably acceptable to Company. University shall consult with Company as to the preparation, filing, prosecution, and maintenance of all Patent Rights reasonably prior to any deadline or action with the United States Patent & Trademark Office or any foreign patent office and shall furnish Company with copies of relevant documents reasonably in advance of consultation. University shall consider in good faith any comments of Company on any patent filings for the Patent Rights.

(b) If University desires to abandon any patent or patent application within the Patent Rights, University shall provide Company with reasonable prior notice of the intended abandonment, and Company may, at its expense, prepare, file, prosecute, and maintain the relevant Patent Rights.

6.2. Cooperation. Each party shall provide reasonable cooperation in the preparation, filing, prosecution, and maintenance of all Patent Rights. Cooperation includes, without limitation, promptly informing the other party of matters that may affect the preparation, filing, prosecution, or maintenance of Patent Rights (such as, becoming aware of an additional inventor who is not listed as an inventor in a patent application).

### 6.3. Payment of Expenses.

(a) Subject to Section 4.8 (Change in Consideration), within [\*\*] after University invoices Company, Company shall reimburse University for all previously unreimbursed expenses incurred as of the Effective Date in connection with obtaining the Patent Rights.

(b) Subject to Section 4.8 (Change in Consideration), within [\*\*] after University invoices Company, Company shall reimburse University for all patent-related expenses that have not been paid under Subsection 6.3(a) and that are incurred by University pursuant to Section 6.1. Company may elect, upon [\*\*] written notice to University, to cease payment of the expenses associated with obtaining or maintaining patent protection for one or more Patent Rights in one or more countries. If Company elects to cease payment of any patent expenses, Company loses all rights under this Agreement with respect to the particular Patent Rights in those one or more countries.

#### 6.4. Infringement.

(a) Notification of Infringement. Each party agrees to provide written notice to the other party promptly after becoming aware of any infringement of the Patent Rights.

(b) Company Right to Prosecute. As long as Company remains the exclusive licensee of the Patent Rights in the Field, Company may, under its own control and at its own expense, prosecute any third party infringement of the Patent Rights in the Field or, together with licensees of the Patent Rights in other fields (if any), defend the Patent Rights in any declaratory judgment action brought by a third party which alleges invalidity, unenforceability, or infringement of the Patent Rights. Prior to commencing any action, Company shall consult with University and shall consider the views of University regarding the advisability of the proposed action and its effect on the public interest. Company may not enter into any settlement, consent judgment, or other voluntary final disposition of any infringement action under this Subsection 6.4(b) without the prior written consent of University, which consent may not be unreasonably withheld or delayed. Any recovery obtained in an action under this Subsection 6.4(b) shall be distributed as follows: (i) each party shall be reimbursed for any expenses incurred in the action; (ii) as to ordinary damages, Company shall receive an amount equal to its lost profits or a reasonable royalty on the infringing sales (whichever measure of damages the court applied); less a reasonable approximation of the royalties that Company would have paid to University if Company had sold the infringing products and services rather than the infringer; and (iii) as to special or punitive damages, the parties shall share equally in any award.

(c) University as Indispensable Party. University shall permit any action under Subsection 6.4(b) to be brought in its name if required by law, provided that Company shall hold University harmless from, and if necessary indemnify University against, any costs, expenses, or liability that University may incur in connection with the action.

(d) University Right to Prosecute. If Company fails to initiate an infringement action within a reasonable time after it first becomes aware of the basis for the action, or to answer a declaratory judgment action within a reasonable time after the action is filed, University may prosecute the infringement or answer the declaratory judgment action under its sole control and at its sole expense, and any recovery obtained shall be given to University. If University takes action under this Subsection 6.4(d), University shall keep Company reasonably informed of material actions taken by University pursuant to the infringement or declaratory action.

(e) Cooperation. Both parties shall cooperate fully in any action under this Section 6.4. that is controlled by the other party, provided that the controlling party reimburses the cooperating party promptly for any reasonable costs and expenses incurred by the cooperating party in connection with providing assistance.

## 7. Confidential Information; Publications; Publicity.

### 7.1. Confidential Information.

(a) Designation. The Disclosing Party shall mark Confidential Information that is disclosed in writing with a legend indicating its confidential status (such as, "Confidential" or "Proprietary"). The Disclosing party shall document Confidential Information that is disclosed orally or visually in a written notice and deliver the notice to the Receiving Party within [\*\*] of the date of disclosure. The notice shall summarize the Confidential Information that was disclosed and reference the time and place of disclosure.

(b) Obligations. For [\*\*] after disclosure of any portion of Confidential Information, the Receiving Party shall (i) maintain Confidential Information in confidence, except that the Receiving Party may disclose or permit the disclosure of any Confidential Information to its trustees or directors, officers, employees, consultants, and advisors who are obligated to maintain the confidential nature of Confidential Information and who need to know Confidential Information for the purposes of this Agreement; (ii) use Confidential Information solely for the purposes of this Agreement; and (iii) allow its trustees or directors, officers, employees, consultants, and advisors to reproduce the Confidential Information only to the extent necessary for the purposes of this Agreement, with all reproductions being Confidential Information.

(c) Exceptions. The obligations of the Receiving Party under Subsection 7.1(b) do not apply to the extent that the Receiving Party can demonstrate that Confidential Information (i) was in the public domain prior to the time of its disclosure under this Agreement; (ii) entered the public domain after the time of its disclosure under this Agreement through means other than an unauthorized disclosure resulting from an act or omission by the Receiving Party; (iii) was already known or independently developed or discovered by the Receiving Party without use of the Confidential Information; (iv) is or was disclosed to the Receiving Party at any time, whether prior to or after the time of its disclosure under this Agreement, by a third party having no fiduciary relationship with the Disclosing Party and having no obligation of confidentiality with respect to the Confidential Information; or (v) is required to be disclosed to comply with applicable laws or regulations or with a court or administrative order, provided that the Disclosing Party receives reasonable prior written notice of the disclosure.

(d) Ownership and Return. The Receiving Party acknowledges that the Disclosing Party (or a third party entrusting its own information to the Disclosing Party) owns the Confidential Information in the possession of the Receiving Party. Upon expiration or termination of this Agreement, or at the request of the Disclosing Party, the Receiving Party shall return to the Disclosing Party all originals, copies, and summaries of documents, materials, and other tangible manifestations of Confidential Information in the possession or control of the Receiving Party, except that the Receiving Party may retain one copy of the Confidential Information in the possession of its legal counsel solely for the purpose of monitoring its obligations under this Agreement. Notwithstanding the foregoing, without limiting the parties' confidentiality and non-use obligations hereunder, the parties will not be obligated to purge computer systems of Confidential Information, as the parties recognize that a systemic purge of such systems would likely be impracticable.

7.2. Publicity Restrictions. Company may not use the name of University or any of its trustees, officers, faculty, students, employees, or agents, or any adaptation of their names, or any terms of this Agreement in any promotional material or other public announcement or disclosure without the prior written consent of University. The foregoing notwithstanding, Company may disclose that information without the consent of University in any prospectus, offering memorandum, or other document or filing required by applicable securities laws or other applicable law or regulation, provided that Company provides University at least [\*\*] (or a shorter period in order to enable Company to make a timely announcement to fulfill applicable securities laws or other applicable law or regulation, while affording University the maximum feasible time to review the announcement) prior written notice of the proposed text for the purpose of giving University the opportunity to comment on the text.

## 8. Term and Termination.

8.1. Term. The Agreement will continue in full force and effect until the last to expire Valid Claim of the Licensed Patents, unless earlier terminated.

8.2. Voluntary Termination by Company. Company may terminate this Agreement for any reason upon thirty (30) days' prior written notice to University; provided, however that if Company elects to terminate this Agreement during the applicable royalty term set forth in Section 4.5, Company agrees that it shall lose all rights to make, use, sell, have made, have used or have sold Licensed Products or Licensed Services.

8.3. Termination for Default. If Company commits a material breach of its obligations under this Agreement and fails to cure that breach within [\*\*] in the case of payment breaches), University may terminate this Agreement immediately upon written notice to Company. As to alleged breaches involving nonpayment of amounts due University under this Agreement, Company shall have only [\*\*] opportunities to cure such material breaches for which it receives notice as described above. A [\*\*] or subsequent material breach by Company of its payment obligations hereunder will entitle University to terminate this Agreement immediately upon written notice to Company, without a [\*\*] cure period.

8.4. Force Majeure. Neither party is responsible for delays resulting from causes beyond its reasonable control, including without limitation fire, explosion, flood, war, strike, act of terrorism or riot, provided that the nonperforming party uses commercially reasonable efforts to avoid or remove those causes of nonperformance and continues performance under this Agreement with reasonable dispatch whenever the causes are removed.

8.5. Effect of Termination. The following provisions survive the expiration or termination of this Agreement: Articles 1 and 9; Sections 3.3., 3.4., 3.6., 5.2. (obligation to provide final report and payment), 4.9., 5.3., 5.4., 5.5., 5.6., 5.7., 5.8., 6.3., 7.1., 7.2., 8.5. and 10.9. Upon the early termination of this Agreement, Company and its Affiliates and Sublicensees may complete and sell any work-in-progress and inventory of Licensed Products that exist as of the effective date of termination, provided that (a) Company is current in payment of all amounts due University under this Agreement, (b) Company pays University the applicable royalty and Sublicense Income on sales of Licensed Products in accordance with the terms of



this Agreement, and (c) Company and its Affiliates and Sublicensees complete and sell all work-in-progress and inventory of Licensed Products within [\*\*] after the effective date of termination. Upon the expiration or termination of this Agreement, any sublicense granted by Company shall survive such termination if the applicable Sublicensee is not in breach of its obligations under the applicable Sublicense Agreement, provided that the terms of Section 4.8 shall be void and University shall continue to be entitled to receive all amounts payable hereunder with respect to such Sublicensee's exercise of such surviving rights as if this Agreement remained in force.

## 9. Dispute Resolution.

9.1. Procedures Mandatory. The parties shall resolve any dispute arising out of or relating to this Agreement solely by means of the procedures set forth in this Article. These procedures constitute legally binding obligations that are an essential provision of this Agreement. If either party fails to observe the procedures of this Article, as modified by their written agreement, the other party may bring an action for specific performance in any court of competent jurisdiction.

### 9.2. Dispute Resolution Procedures.

(a) Negotiation. In the event of any dispute arising out of or relating to this Agreement, the affected party shall notify the other party, and the parties shall attempt in good faith to resolve the matter within [\*\*] after the date of notice (the "Notice Date"). Any disputes not resolved by good faith discussions shall be referred to senior executives of each party, who shall meet at a mutually acceptable time and location within [\*\*] after the Notice Date and attempt to negotiate a settlement.

(b) Mediation. If the matter remains unresolved within [\*\*] after the Notice Date, or if the senior executives fail to meet within [\*\*] after the Notice Date, either party may initiate mediation upon written notice to the other party, and both parties shall engage in a mediation proceeding under the then current CPR Institute for Dispute Resolution ("CPR") Model Procedure for Mediation of Business Disputes. Specific provisions of this Subsection 9.2(b) override inconsistent provisions of the CPR Model Procedure. The parties shall select the mediator from the CPR Panels of Neutrals. If the parties cannot agree upon the selection of a mediator within [\*\*] after the Notice Date, then upon the request of either party, the CPR shall appoint the mediator. The parties shall attempt to resolve the dispute through mediation until one of the following occurs: (i) the parties reach a written settlement; (ii) the mediator notifies the parties in writing that they have reached an impasse; (iii) the parties agree in writing that they have reached an impasse; or (iv) the parties have not reached a settlement within [\*\*] after the Notice Date.

(c) Trial Without Jury. If the parties fail to resolve the dispute through mediation, or if neither party elects to initiate mediation, each party may pursue any other remedies legally available to resolve the dispute. However, the parties expressly waive the right to a jury trial in the legal proceeding under this Subsection 9.2(c).

### 9.3. Preservation of Rights Pending Resolution.

(a) Performance to Continue. Each party shall continue to perform its obligations under this Agreement pending final resolution of any dispute arising out of or relating to this Agreement. However, a party may suspend performance of its obligations during any period in which the other party fails or refuses to perform its obligations.

(b) Provisional Remedies. Although the procedures specified in this Article are the exclusive procedures for resolution of disputes arising out of or relating to this Agreement, either party may seek a preliminary injunction or other provisional equitable relief if, in its reasonable judgment, that action is necessary to avoid irreparable harm to itself or to preserve its rights under this Agreement.

(c) Statute of Limitations. The parties agree that all applicable statutes of limitation and time-based defenses (such as, estoppel and laches) are tolled while the procedures set forth in Subsections 9.2.(a) and 9.2(b) are pending. The parties shall take any actions necessary to effectuate this result.

### 10. Miscellaneous.

10.1. Representations and Warranties. University represents that its employees and [\*\*] and [\*\*] have assigned to University their entire right, title, and interest in the Patent Rights, and that it has authority to grant the rights and licenses set forth in this Agreement, and that it has not granted any rights in the Patent Rights to any third party that is inconsistent with the grant of rights in this Agreement. UNIVERSITY MAKES NO OTHER WARRANTIES CONCERNING THE PATENT RIGHTS, INCLUDING WITHOUT LIMITATION ANY EXPRESS OR IMPLIED WARRANTY OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE. Specifically, University makes no warranty or representation (a) regarding the validity or scope of the Patent Rights, (b) that the exploitation of the Patent Rights or any Licensed Product will not infringe any patents or other intellectual property rights of a third party, and (c) that any third party is not currently infringing or will not infringe the Patent Rights.

10.2. Compliance with Law and Policies. Company agrees to comply with applicable law and the policies of University in the area of technology transfer and shall promptly notify University of any violation that Company knows or has reason to believe has occurred or is likely to occur. The University policies currently in effect at the Worcester campus are the Intellectual Property Policy, Policy on Conflicts of Interest Relating to Intellectual Property and Commercial Ventures, and Policy on Faculty Consulting and Outside Activities.

10.3. Tax-Exempt Status. Company acknowledges that University, as a public institution of the Commonwealth of Massachusetts, is an exempt organization under the United States Internal Revenue Code of 1986, as amended. Company also acknowledges that certain facilities in which the licensed inventions were developed may have been financed through offerings of tax-exempt bonds. If the Internal Revenue Service determines, or if counsel to University reasonably determines, that any term of this Agreement jeopardizes the tax-exempt status of University or the bonds used to finance University facilities, the relevant term is invalid and shall be modified in accordance with Section 10.11.

10.4. Counterparts. This Agreement may be executed in one or more counterparts, each of which is an original, and all of which together are one instrument.

10.5. Headings. All headings are for convenience only and do not affect the meaning of any provision of this Agreement.

10.6. Binding Effect. This Agreement is binding upon and inures to the benefit of the parties and their respective permitted successors and assigns.

10.7. Assignment. This Agreement may not be assigned by either party without the prior written consent of the other party, which consent may not be unreasonably withheld or delayed. Notwithstanding the foregoing, this Agreement may be assigned by either party in connection with a merger, consolidation, sale of all of the equity interests of the party, or a sale of all or substantially all of the assets of the party to which this Agreement relates.

10.8. Amendment and Waiver. The parties may only amend, supplement, or otherwise modify this Agreement through a written instrument signed by both parties. The waiver of any rights or failure to act in a specific instance relates only to that instance and is not an agreement to waive any rights or fail to act in any other instance.

10.9. Governing Law. This Agreement is governed by and construed in accordance with the laws of the Commonwealth of Massachusetts irrespective of any conflicts of law principles. The parties may only bring legal action that arises out of or in connection with this Agreement in the Massachusetts Superior Court in Suffolk County.

10.10. Notice. Any notices required or permitted under this Agreement shall be in writing, shall specifically refer to this Agreement, and shall be sent by recognized national overnight courier, or registered or certified mail, postage prepaid, return receipt requested, to the following addresses:

**If to University:**

Office of Technology Management  
University of Massachusetts  
Room# S4-110  
55 Lake Avenue North  
Shrewsbury, MA 01545

Attention: Executive Director

**If to Company:**

Torus Therapeutics, Inc.  
400 Technology Square, 10th Floor  
Cambridge, MA 02139

Attention: Chief Executive Officer

All notices under this Agreement are effective upon receipt. A party may change its contact information immediately upon written notice to the other party in the manner provided in this Section 10.10.

10.11. Severability. If any provision of this Agreement is held invalid or unenforceable for any reason, the invalidity or unenforceability does not affect any other provision of this Agreement, and the parties shall negotiate in good faith to modify the Agreement to preserve (to the extent possible) their original intent. If the parties fail to reach a modified agreement within [\*\*] after the relevant provision is held invalid or unenforceable, then the dispute shall be resolved in accordance with the procedures set forth in Article 9. While the dispute is pending resolution, this Agreement shall be construed as if the provision were deleted by agreement of the parties.

10.12. Entire Agreement. This Agreement constitutes the entire agreement between the parties with respect to its subject matter and supersedes all prior agreements or understandings between the parties relating to its subject matter.

THE PARTIES have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

**UNIVERSITY OF MASSACHUSETTS**

By: /s/ James P. McNamara, Ph.D.  
Name: James P. McNamara, Ph.D.,  
Title: Executive Director,  
Office of Technology Management  
Date: June 28, 2017

**TORUS THERAPEUTICS, INC.**

By: /s/ Jason Rhodes  
Name: Jason Rhodes  
Title: Chairman  
Date: 23 June 2017

Certain identified information has been marked in the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed.  
Double asterisks denote omissions.

**PUBLIC HEALTH SERVICE**

**PATENT LICENSE AGREEMENT NONEXCLUSIVE SUBLICENSABLE**

This **Agreement** is based on the model Patent License Non-Exclusive Sublicensable Agreement adopted by the U.S. Public Health Service (“**PHS**”) Technology Transfer Policy Board for use by components of the National Institutes of Health (“**NIH**”), the Centers for Disease Control and Prevention (“**CDC**”), and the Food and Drug Administration (“**FDA**”), which are agencies of the **PHS** within the Department of Health and Human Services (“**HHS**”).

This Cover Page identifies the Parties to this **Agreement**:

The U.S. Department of Health and Human Services, as represented by

The National Heart, Lung, and Blood Institute (NHLBI)

an Institute or Center (hereinafter referred to as the “**IC**”) of the

**NIH**

and

Torus Therapeutics, Inc.,

hereinafter referred to as the “**Licensee**”,

having offices at 400 Technology Square, Cambridge, MA 02139,

created and operating under the laws of the State of Delaware, United States of America

**Tax ID No.:** 814301284

License Number: L-124-2017/0

License Application Number: A-084-2017

Serial Number(s) of Licensed Patent(s) or Patent Application(s):

| O/Ref | Country | Application N° | Status |
|-------|---------|----------------|--------|
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |
| [**]  | [**]    | [**]           | [**]   |

All entitled: [\*\*].

HHS Reference: E-241-2010

**Licensee:** Torus Therapeutics

Cooperative Research and Development Agreement (CRADA) Number (if a subject invention): N/A

Additional Remarks: N/A

Public Benefit(s): Development of Gene Therapy Products

This Patent License Agreement, hereinafter referred to as the “**Agreement**”, consists of this Cover Page, an attached **Agreement**, a Signature Page, Appendix A (List of Patent(s) or Patent Application(s)), Appendix B (Fields of Use and Territory), Appendix C (Royalties), Appendix D

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The **IC** and the **Licensee** agree as follows:

1. BACKGROUND

- 1.1 In the course of conducting biomedical and behavioral research, the **IC** investigators made inventions that may have commercial applicability.
- 1.2 By assignment of rights from the **IC** employees, **HHS**, on behalf of the **Government**, and together with Association Institute de Myologie (AIM, Paris, FR), Centre National de la Recherche Scientifique (CNRS, Paris, FR), Universite Pierre et Marie Curie (UPMC, Paris, FR), and INSERM TRANSFERT SA (Paris, FR) (together "**Co-Owner**") owns intellectual property rights claimed in any United States or foreign patent applications or patents corresponding to the assigned inventions. **HHS** also owns any tangible embodiments of these inventions actually reduced to practice by the **IC**.
- 1.3 The Secretary of **HHS** has delegated to the **IC** the authority to enter into this **Agreement** for the licensing of rights to these inventions under 35 U.S.C. §§200-212, the Federal Technology Transfer Act of 1986, 15 U.S.C. §3710(a), and the regulations governing the licensing of **Government**-owned inventions, 37 CFR Part 404.
- 1.4 The **IC** desires to transfer these inventions to the private sector through commercialization licenses to facilitate the commercial development of products and processes for public use and benefit.
- 1.5 The **Licensee** desires to acquire commercialization rights to certain of these inventions in order to develop processes, methods, or marketable products for public use and benefit.

2. Definitions

- 2.1 "**Affiliate(s)**" means a corporation or other business entity, which directly or indirectly is controlled by or controls, or is under common control with the **Licensee**. For this purpose, the term "control" shall mean ownership of more than fifty percent (50%) of the voting stock or other ownership interest of the corporation or other business entity, or the power to elect or appoint more than fifty percent (50%) of the members of the governing body of the corporation or other business entity.
- 2.2 "**Benchmarks**" mean the performance milestones that are set forth in Appendix D.

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- 2.3 **“Commercial Development Plan”** means the written commercialization plan attached as Appendix E.
- 2.4 **“First Commercial Sale”** means the initial transfer by or on behalf of the **Licensee** or its sublicensees of **Licensed Products** or the initial practice of a **Licensed Process** by or on behalf of the **Licensee** or its sublicensees in exchange for cash or some equivalent to which value can be assigned for the purpose of determining **Net Sales**.
- 2.5 **“Government”** means the Government of the United States of America.
- 2.6 **“Licensed Fields of Use”** means the fields of use identified in Appendix B.
- 2.7 **“Licensed Patent Rights”** shall mean:
- (a) Patent applications (including provisional patent applications and PCT patent applications) or patents listed in Appendix A, all divisions and continuations of these applications, all patents issuing from these applications, divisions, and continuations, and any reissues, reexaminations, and extensions of all these patents;
  - (b) to the extent that the following contain one or more claims directed to the invention or inventions disclosed in 2.7(a):
    - (i) continuations-in-part of 2.7(a);
    - (ii) all divisions and continuations of these continuations-in-part;
    - (iii) all patents issuing from these continuations-in-part, divisions, and continuations;
    - (iv) priority patent application(s) of 2.7(a); and
    - (v) any reissues, reexaminations, and extensions of all these patents;
  - (c) to the extent that the following contain one or more claims directed to the invention or inventions disclosed in 2.7(a): all counterpart foreign and U.S. patent applications and patents to 2.7(a) and 2.7(b), including those listed in Appendix A; and
  - (d) **Licensed Patent Rights** shall *not* include 2.7(b) or 2.7(c) to the extent that they contain one or more claims directed to new matter which is not the subject matter disclosed in 2.7(a).

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- 2.8 “**Licensed Processes**” means processes, which in the course of being practiced, would be within the scope of one or more claims of the **Licensed Patent Rights** that have not expired or been abandoned or revoked or held unpatentable, invalid or unenforceable by an unappealed or unappealable judgment of a court of competent jurisdiction.
- 2.9 “**Licensed Products**” means tangible materials, which in the course of manufacture, use, sale, or importation, would be within the scope of one or more claims of the **Licensed Patent Rights** that have not expired or been abandoned or revoked or held unpatentable, invalid or unenforceable by an unappealed or unappealable judgment of a court of competent jurisdiction.
- 2.10 “**Licensed Territory**” means the geographical area identified in Appendix B.
- 2.11 “**Net Sales**” means the total gross receipts for sales of **Licensed Products** or practice of **Licensed Processes** by or on behalf of the **Licensee** or its sublicensees, and from leasing, renting, or otherwise making **Licensed Products** available to others without sale or other dispositions, whether invoiced or not, less returns and allowances, packing costs, insurance costs, freight out, taxes or excise duties imposed on the transaction (if separately invoiced), and trade, wholesaler and quantity discounts, chargebacks and rebates and cash discounts in amounts customary in the trade to the extent actually granted. No deductions shall be made for commissions paid to individuals, whether they are with in dependent sales agencies or regularly employed by the **Licensee**, or sublicensees and on its payroll, or for the cost of collections. Net Sales does not include (a) any sale or transfers between or among **Licensee** and its **Affiliate(s)** or sublicensees; provided that the subsequent sale of such **Licensed Product** to a third-party end-user shall be included in Net Sales; (b) donations to non-profit institutions or government agencies or (c) use or practice for test marketing promotional use or demonstration purposes, or for clinical trials or indigent patient programs.
- 2.12 “**Practical Application**” means to manufacture in the case of a composition or product, to practice in the case of a process or method, or to operate in the case of a machine or system; and in each case, under these conditions as to establish that the invention is being utilized and that its benefits are to the extent permitted by law or **Government** regulations available to the public on reasonable terms.

3. **GRANT OF RIGHTS**

- 3.1 The **IC** hereby grants, and the **Licensee** accepts, subject to the terms and conditions of this **Agreement**, a nonexclusive license under the **Licensed Patent Rights** in the **Licensed Territory** to make and have made, research, have researched, to use and have used, to sell and have sold, to offer to sell, and to import any **Licensed Products** in the **Licensed Fields of Use** and to practice and have practiced any **Licensed Processes** in the **Licensed Fields of Use**.

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- 3.2 If the license granted to **Licensee** in Paragraph 3.1 is held to be invalid or otherwise ineffective with respect to any of the **Licensed Patent Rights** on account of any failure by the **IC** to obtain the consent of a co-owner of such **Licensed Patent Rights** to the grant of such license, the **IC** hereby covenants not to enforce such **Licensed Patent Rights** against **Licensee** or any of **Licensee's** permitted sublicensees based on any making or having made, research, having researched, use or having used, sale or having sold, offering to sell, or importation of any **Licensed Products** in the **Licensed Fields of Use** or practice or having practiced any **Licensed Processes** in the **Licensed Fields of Use** by or on behalf of **Licensee** or any of **Licensee's** permitted sublicensees, for so long as this **Agreement** remains in force, and the **IC** further covenants not to consent or agree to, and not to grant or transfer any right permitting or enabling the undertaking of, any such enforcement by any third party.
- 3.3 **Licensee** understands that, as of the date of this **Agreement**, **NIH** and/or **IC**, on the one hand, and the **Co-Owner**, on the other hand, are or may be in discussions regarding a proposed inter-institutional agreement (the "**Proposed IIA**") between them pursuant to which they may enter into certain agreements regarding the licensing and administration of the **Licensed Patent Rights**. **Licensee** agrees, if requested by **NIH** and/or **IC**, to reasonably negotiate appropriate amendments to this **Agreement** to conform with the **Proposed IIA**, if and when executed, and, whether or not the **Proposed IIA** is executed, shall agree to pay any portion of **Licensee's** reimbursement obligations under Paragraphs 6.9 and 6.10 directly to **Co-Owner** as directed by the **IC**, provided that **Licensee's** license hereunder shall remain in force and **Licensee** shall be treated on par with (or more favorably than) other non-exclusive licensees of the **Licensed Patent Rights**.
- 3.4 This **Agreement** confers no license or rights by implication, estoppel, or otherwise under any patent applications or patents of the **IC** other than the **Licensed Patent Rights** regardless of whether these patents are predominant or subordinate to the **Licensed Patent Rights**.

#### 4. SUBLICENSING

- 4.1 The **Licensee** shall notify the **IC** in writing of its intent to sublicense, after which **Licensee** may enter into sublicensing agreements under the **Licensed Patent Rights** only when it concurrently licenses proprietary or in-licensed intellectual property rights controlled by **Licensee** in connection with **Licensed Products** or **Licensed Processes**. For the avoidance of doubt, the **Licensee** does not have the right to solely sublicense the **Licensed Patent Rights**.

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- 4.2 The **Licensee** agrees that any sublicenses granted by it shall provide that the obligations to the **IC** of paragraphs 5.1, 5.2, 8.1, 10.1, 10.2, 12.5, and 13.7-13.9 of this **Agreement** shall be binding upon the sublicensee as if it were a party to this **Agreement**. The **Licensee** further agrees to attach copies of these Paragraphs to all sub license agreements.
- 4.3 Any sublicenses granted by the **Licensee** shall provide for the termination of the sub license, or the conversion to a license directly between the sublicensees and the **IC**, at the option of the sublicensee, upon termination of this **Agreement** under Article 13. This conversion is subject to the **IC** approval and contingent upon acceptance by the sublicensee of the remaining provisions of this **Agreement**.
- 4.4 The **Licensee** agrees to forward to the **IC** a complete copy of each fully executed sublicense agreement postmarked within [\*\*] of the execution of the agreement, which copy may be reasonably redacted as to confidential business information that is not required to enable the **IC** to confirm the compliance of the a sublicense agreement with the requirements of this **Agreement**. To the extent permitted by law, the **IC** agrees to maintain each sublicense agreement in confidence.
5. STATUTORY AND NIH REQUIREMENTS AND RESERVED GOVERNMENT RIGHTS
- 5.1 Prior to the **First Commercial Sale**, the **Licensee** agrees to provide the **IC** with reasonable quantities of **Licensed Products** or materials made through the **Licensed Processes** for the **IC**'s non-clinical research use.
- 5.2 The **Licensee** agrees that products used or sold in the United States embodying **Licensed Products** or produced through use of **Licensed Processes** shall be manufactured substantially in the United States, unless a written waiver is obtained in advance from the **IC**.
6. ROYALTIES AND REIMBURSEMENT
- 6.1 The **Licensee** agrees to pay the **IC** a noncreditable, nonrefundable license issue royalty as set forth in Appendix C.
- 6.2 The **Licensee** agrees to pay the **IC** a minimum annual royalty as set forth in Appendix C.
- 6.3 The **Licensee** agrees to pay the **IC** earned royalties as set forth in Appendix C.
- 6.4 The **Licensee** agrees to pay the **IC** benchmark royalties as set forth in Appendix C.
- 6.5 The **Licensee** agrees to pay the **IC** sublicensing royalties as set forth in Appendix C.

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- 6.6 A patent or patent application licensed under this Agreement shall cease to fall within the **Licensed Patent Rights** for the purpose of computing earned royalty payments in any given country on the earliest of the dates that:
- (a) the application has been abandoned and not continued;
  - (b) the patent expires or irrevocably lapses; or
  - (c) the patent has been held to be invalid or unenforceable by an unappealed or unappealable decision of a court of competent jurisdiction or administrative agency.
- 6.7 No multiple royalties shall be payable because any **Licensed Products** or **Licensed Processes** are covered by more than one of the **Licensed Patent Rights**.
- 6.8 On sales of **Licensed Products** by the **Licensee** to sublicensees or on sales made in other than an arms-length transaction, the value of the **Net Sales** attributed under this Article 6 to this transaction shall be that which is received in a subsequent arms-length transaction from an end-user of the **Licensed Products**.
- 6.9 With regard to unreimbursed expenses associated with the preparation filing, prosecution, and maintenance of all patent applications and patents included within the **Licensed Patent Rights** and paid by the **IC** and the **Co-Owner** prior to the effective date of this **Agreement**, the **Licensee** shall pay the **Co-Owner**, as an additional royalty, within **[\*\*]** of the **Co-Owner's** submission of a statement (which will include summaries of patent prosecution invoices) and request for payment to the **Licensee**, an amount equivalent to the unreimbursed patent expenses previously paid by the **IC** and the **Co-Owner** divided by the aggregate number of commercial licensees of the **Licensed Patent Rights**, that are obligated to reimburse patent costs under similar such provision on the date of the Invoice. Notwithstanding, **Licensee** shall not be required to pay more than **[\*\*]** of such expenses. Such expenses are estimated to total **[\*\*]** Euro (**[\*\*]** US Dollars with an exchange rate of 1.07 US Dollar to Euro) as of January 18, 2017, and accordingly the **Licensee's** payment obligation as of January 18, 2017 is estimated to be **[\*\*]** Euro (**[\*\*]** US Dollars). The conversion to US Dollars, if applicable, shall be made using the exchange rate published on the day the statement is received by **Licensee**. Unless otherwise indicated in **Co-Owner's** request for payment, **Licensee** shall wire its payment obligation under Paragraph 6.9 to the following bank account:

AIM SECTEUR LUCRATIF INSTITUT DE MYOLOGIE  
BNP PARIBAS  
PARIS ASSOCIATIONS (02837)  
IBAN: **[\*\*]** BIC: **[\*\*]**  
Tax ID Number: **[\*\*]**

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Payment under 6.9 shall not be due before the execution of an Inter-Institutional **Agreement** between the **IC** and the **Co-Owner**.

- 6.10 With regard to unreimbursed expenses associated with the preparation, filing, prosecution, and maintenance of all patent applications and patents included within the **Licensed Patent Rights** and paid by the **IC** and the **Co-Owner** on or after the effective date of this **Agreement**, the **IC**, at its sole option, may require the **Licensee**:
- (a) to pay the **Co-Owner** on an annual basis, within [\*\*] of the **Co-Owner's** submission of a statement (which will include summaries of patent prosecution invoices) and request for payment, a royalty amount equivalent to these unreimbursed expenses paid during the previous calendar year(s) divided by the aggregate number of commercial licensees of the **Licensed Patent Rights**, that are obligated to reimburse patent costs under similar such provision on the date of the Invoice;
  - (b) to pay these unreimbursed expenses directly to the law firm employed by the **IC** or the **Co-Owner**, as applicable, to handle these functions divided by the aggregate number of commercial licensees of the **Licensed Patent Rights**, that are obligated to reimburse patent costs under similar such provision on the date of the Invoice. However, in this event, the **IC** or the **Co-Owner**, as applicable, and not the **Licensee** shall be the client of the law firm; or
  - (c) under exceptional circumstances, the **Licensee** may be given the right to assume responsibility for the preparation, filing, prosecution, or maintenance of any patent application or patent included with the **Licensed Patent Rights**. In that event, the **Licensee** shall directly pay the attorneys or agents engaged to prepare, file, prosecute, or maintain these patent applications or patents and shall provide the **IC** with copies of each invoice associated with these services as well as **Co-Owner** documentation that these invoices have been paid.

Notwithstanding the foregoing, **Licensee** shall not be required to pay more than [\*\*] of the amounts set forth in clauses (a) and (b) above.

Payment under 6.10 shall not be due before the execution of an Inter-Institutional Agreement between the **IC** and the **Co-Owner**.

- 6.11 The **Licensee** may elect to surrender its rights in any country of the **Licensed Territory** under any of the **Licensed Patent Rights** upon [\*\*] written notice to the **IC** and owe no payment obligation under Paragraph 6.3, 6.4 or 6.10 in that country after the effective date of the written notice.

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7. PATENT FILING, PROSECUTION, AND MAINTENANCE

The **IC** agrees that the **Co-Owner** will take responsibility for the preparation, filing, prosecution, and maintenance of any and all patent applications or patents included in the **Licensed Patent Rights**, and that the will furthermore be responsible for submitting to the **Licensee** all statements regarding payments of patent expenses as per Paragraphs 6.9 and 6.10.

8. RECORD KEEPING

8.1 The **Licensee** agrees to keep accurate and correct records of **Licensed Products** made, used, sold, or imported and **Licensed Processes** practiced under this **Agreement** appropriate to determine the amount of royalties due the **IC**. These records shall be retained for at least [\*\*] following a given reporting period and shall be available during normal business hours for inspection, at the expense of the **IC**, by an accountant or other designated auditor selected by the **IC** for the sole purpose of verifying reports and royalty payments hereunder. The accountant or auditor shall only disclose to the **IC** information relating to the accuracy of reports and royalty payments made under this **Agreement**. If an inspection shows an underreporting or underpayment in excess of [\*\*] percent ([\*\*]%) for any twelve (12) month period, then the **Licensee** shall reimburse the **IC** for the cost of the inspection at the time the **Licensee** pays the unreported royalties, including any additional royalties as required by Paragraph 9.8. All royalty payments required under this Paragraph shall be due within [\*\*] of the date the **IC** provides the **Licensee** notice of the payment due.

9. REPORTS ON PROGRESS, BENCHMARKS, SALES, AND PAYMENTS

- 9.1 Prior to signing this **Agreement**, the **Licensee** has provided the **IC** with the **Commercial Development Plan** in Appendix E, under which the **Licensee** intends to bring the subject matter of the **Licensed Patent Rights** to the point of **Practical Application**. This **Commercial Development Plan** is hereby incorporated by reference into this **Agreement**. Based on this plan, performance **Benchmarks** are determined as specified in Appendix D.
- 9.2 The **Licensee** shall provide written annual reports on its product development progress or efforts to commercialize under the **Commercial Development Plan** for each of the **Licensed Fields of Use** within [\*\*] after December 31 of each calendar year. These progress reports shall include, but not be limited to: progress on research and development, status of applications for regulatory approvals, manufacture and status of sublicensing, marketing, importing, and sales during the preceding calendar year, as well as, plans for the present calendar year. The **IC** also encourages these reports to include information on any of the **Licensee's** public service activities that relate to the **Licensed Patent Rights**. If reported progress differs from that projected in the **Commercial Development Plan** and **Benchmarks**, the **Licensee** shall explain the reasons for such

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differences. In any annual report, the **Licensee** may propose amendments to the **Commercial Development Plan**, acceptance of which by the **IC** may not be denied unreasonably. The **Licensee** agrees to provide any additional information reasonably required by the **IC** to evaluate the **Licensee's** performance under this **Agreement**. The **Licensee** may amend the **Benchmarks** at any time upon written approval by the **IC**. The **IC** shall not unreasonably withhold approval of any request of the **Licensee** to extend the time periods of this schedule if the request is supported by a reasonable showing by the **Licensee** of diligence in its performance under the **Commercial Development Plan** and toward bringing the **Licensed Products** to the point of **Practical Application**.

- 9.3 The **Licensee** shall report to the **IC** the dates for achieving **Benchmarks** specified in Appendix D and the **First Commercial Sale** in each country in the **Licensed Territory** within [\*\*] of such occurrences.
- 9.4 The **Licensee** shall submit to the **IC**, within [\*\*] after each calendar [\*\*], a royalty report, as described in the example in Appendix F, setting forth for the preceding [\*\*] period, to the extent possible, an estimate of the amount of the **Licensed Products** sold or **Licensed Processes** practiced by or on behalf of the **Licensee** in each country within the **Licensed Territory**, the Net Sales and the amount of royalty accordingly due. With each royalty report, the **Licensee** shall submit payment of earned royalties due. If no earned royalties are due to the **IC** for any reporting period, the written report shall so state. The royalty report shall be certified as correct by an authorized officer of the **Licensee** and shall include a detailed listing of all deductions made under Paragraph 2.11 to determine **Net Sales** made under Article 6 to determine royalties due.
- 9.5 The **Licensee** agrees to forward [\*\*] to the **IC** a copy of these reports received by the **Licensee** from its sublicensees during the preceding [\*\*] period as shall be pertinent to a royalty accounting to the **IC** by the **Licensee** for activities under the sublicense.
- 9.6 Royalties due under Article 6 shall be paid in U.S. dollars and payment options are listed in Appendix G. For conversion of foreign currency to U.S. dollars, the conversion rate shall be the New York foreign exchange rate quoted in *The Wall Street Journal* on the day that the payment is due, and any loss of exchange, value, taxes, or other expenses incurred in the transfer or conversion to U.S. dollars shall be paid entirely by the **Licensee**. The royalty report required by Paragraph 9.4 shall be mailed to the **IC** at its address for Agreement Notices indicated on the Signature Page.
- 9.7 The **Licensee** shall be solely responsible for determining if any tax on royalty income is owed outside the United States and shall pay this tax and be responsible for all filings with appropriate agencies of foreign governments.

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- 9.8 Additional royalties may be assessed by the **IC** on any payment that is more than **[\*\*]** overdue at the rate of **[\*\*]** percent (**[\*\*]**%) per month. This **[\*\*]** percent (**[\*\*]**%) per month rate may be applied retroactively from the original due date until the date of receipt by the **IC** of the overdue payment and additional royalties. The payment of any additional royalties shall not prevent the **IC** from exercising any other rights it may have as a consequence of the lateness of any payment.
- 9.9 All plans and reports required by this Article 9 and marked “confidential” by the **Licensee** shall, to the extent permitted by law, be treated by the **IC** as commercial and financial information obtained from a person and as privileged and confidential, and any proposed disclosure of these records by the **IC** under the Freedom of Information Act (FOIA), 5 U.S.C. §552 shall be subject to the predisclosure notification requirements of 45 CFR §5.65(d).

10. PERFORMANCE

- 10.1 The **Licensee** shall use its reasonable commercial efforts to bring the **Licensed Products** and **Licensed Processes** to **Practical Application**. **Licensee** shall be deemed to have used “reasonable commercial efforts” if it has adhered to the **Commercial Development Plan** in Appendix E and performance of the **Benchmarks** in Appendix D. The efforts of a sublicensee or an Affiliate shall be considered the efforts of the **Licensee**.
- 10.2 The **Licensee** agrees, after its **First Commercial Sale**, to make reasonable quantities of **Licensed Products** or materials produced through the use of **Licensed Processes** available to patient assistance programs if commercially practicable.
- 10.3 The **Licensee** agrees, after its **First Commercial Sale**, and as part of its marketing and product promotion, to develop educational materials (e.g., brochures, website, etc.) directed to patients and physicians detailing the **Licensed Products** or medical aspects of the prophylactic and therapeutic uses of the **Licensed Products**, if commercially practicable and consistent with applicable regulations.
- 10.4 The **Licensee** agrees to supply, to the Mailing Address for **Agreement** Notices indicated on the Signature Page, the Office of Technology Transfer, **NIH** with a reasonable amount of inert samples of the **Licensed Products** or **Licensed Processes** or their packaging for educational and display purposes only.

11. INFRINGEMENT AND PATENT ENFORCEMENT

- 11.1 The **IC** and the **Licensee** agree to notify each other promptly of each infringement or possible infringement of the **Licensed Patent Rights**, as well as, any facts which may affect the validity, scope, or enforceability of the **Licensed Patent Rights** of which either Party becomes aware.

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11.2 In the event that a declaratory judgment action alleging invalidity of any of the **Licensed Patent Rights** shall be brought against the **IC**, the **IC** agrees to notify the **Licensee** that an action alleging invalidity has been brought. The **IC** does not represent that it shall commence legal action to defend against a declaratory action alleging invalidity. The **Licensee** shall take no action to compel the **Government** either to initiate or to join in any declaratory judgment action. Should the **Government** be made a party to any suit by motion or any other action of the **Licensee**, the **Licensee** shall reimburse the **Government** for any costs, expenses, or fees, which the **Government** incurs as a result of the motion or other action. Upon the **Licensee's** payment of all costs incurred by the **Government** as a result of the **Licensee's** joinder motion or other action, these actions by the **Licensee** shall not be considered a default in the performance of any material obligation under this **Agreement**.

12. NEGATION OF WARRANTIES AND INDEMNIFICATION

12.1 The **IC** offers no warranties other than those specified in Article 1.

12.2 The **IC** does not warrant the validity of the **Licensed Patent Rights** and makes no representations whatsoever with regard to the scope of the **Licensed Patent Rights**, or that the **Licensed Patent Rights** may be exploited without infringing other patents or other intellectual property rights of third parties.

12.3 THE **IC** MAKES NO WARRANTIES, EXPRESSED OR IMPLIED, OF MERCHANTABILITY OR FITNESS FOR AP ARTICULAR PURPOSE OF ANY SUBJECT MATIER DEFINED BY THE CLAIMS OF THE **LICENSED PATENT RIGHTS** OR TANGIBLE MATERIALS RELATED THERETO.

12.4 The **IC** does not represent that it shall commence legal actions against third parties infringing the **Licensed Patent Rights**.

12.5 The **Licensee** shall indemnify and hold the **IC**, its employees, students, fellows, agents, and consultants harmless from and against all liability, demands, damages, expenses, and losses, including but not limited to death, personal injury, illness, or property damage in connection with or arising out of:

- (a) the use by or on behalf of the **Licensee**, its sublicensees, its directors, employees, or third parties of any **Licensed Patent Rights**; or
- (b) the design, manufacture, distribution, or use of any **Licensed Products, Licensed Processes** or materials by the **Licensee**, or other products or processes developed in connection with or arising out of the **Licensed Patent Rights**.

12.6 The **Licensee** agrees to maintain a liability insurance program consistent with so und business practice.

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13. TERM, TERMINATION, AND MODIFICATION OF RIGHTS

- 13.1 This **Agreement** is effective when signed by all parties, unless the provisions of Paragraph 14.15 are not fulfilled, and shall extend on a **Licensed Product-by-Licensed Product** and country-by-country basis to the expiration of the last to expire of the **Licensed Patent Rights** covering the **Licensed Product** in such country unless sooner terminated as provided in this Article 13.
- 13.2 In the event that the **Licensee** is in default in the performance of any material obligations under this **Agreement**, including but not limited to the obligations listed in Paragraph 13.5, and if the default has not been remedied within [\*\*] after the date of notice in writing of the default, the **IC** may terminate this **Agreement** by written notice and pursue outstanding royalties owed through procedures provided by the Federal Debt Collection Act.
- 13.3 In the event that the **Licensee** becomes insolvent, files a petition in bankruptcy, has such a petition filed against it, determines to file a petition in bankruptcy, or receives notice of a third party's intention to file an involuntary petition in bankruptcy, the **Licensee** shall immediately notify the **IC** in writing.
- 13.4 The **Licensee** shall have a unilateral right to terminate this **Agreement** in any country or territory by giving the **IC** sixty (60) days written notice to that effect.
- 13.5 Subject to the cure provisions paragraphs 13.2 and 13.6, the **IC** shall specifically have the right to terminate or modify, at its option, this **Agreement**, if the **IC** reasonably determines that the **Licensee**:
- (a) is not executing the **Commercial Development Plan** submitted with its request for a license and the **Licensee** cannot otherwise demonstrate to the **IC's** satisfaction that the **Licensee** has taken, or can be expected to take within a reasonable time, effective steps to achieve **Practical Application** of the **Licensed Products** or **Licensed Processes**;
  - (b) has not achieved the **Benchmarks** as may be modified under Paragraph 9.2;
  - (c) has willfully made a false statement of, or willfully omitted, a material fact in the license application or in any report required by this **Agreement**;
  - (d) has committed a material breach of a covenant or agreement contained in this **Agreement**;
  - (e) is not keeping **Licensed Products** or **Licensed Processes** reasonably available to the public after commercial use commences;
  - (f) cannot reasonably satisfy unmet health and safety needs; or

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- (g) cannot reasonably justify a failure to comply with the domestic production requirement of Paragraph 5.2, unless waived.
- 13.6 In making the determination referenced in Paragraph 13.5, the **IC** shall take into account the normal course of such commercial development programs conducted with sound and reasonable business practices and judgment and the annual reports submitted by the **Licensee** under Paragraph 9.2. Prior to invoking termination or modification of this **Agreement** under Paragraph 13.5, the **IC** shall give written notice to the **Licensee** providing the **Licensee** specific notice of, and a **[\*\*]** opportunity to respond to the **IC's** concerns as to the items reference d in 13.5(a)-13.5(g). If the **Licensee** fails to alleviate the **IC's** concerns as to the items referenced in 13.5(a)-13.5(g) or fails to initiate connective action to the **IC's** satisfaction, the **IC** may terminate this **Agreement**. Notwithstanding the above, with respect to items 13.5(e) and 13.5(f), the **IC** may only terminate the license with respect to the specific **Licensed Product** which gave rise to the **IC's** concern regarding such items; this **Agreement** will otherwise remain in full force and effect.
- 13.7 The **IC** reserves the right according to 35 U.S.C. §209(d)(3) to terminate or modify this **Agreement** if it is reasonably determined that the action is necessary to meet the requirements for public use specified by federal regulations issued after the date of the license and these requirements are not reasonably satisfied by the **Licensee**.
- 13.8 Within **[\*\*]** of receipt of written notice of the **IC's** unilateral decision to modify or terminate this **Agreement**, the **Licensee** may, consistent with the provisions of 37 CFR §404.11, appeal the decision by written submission to the designated **IC** official. The decision of the designated **IC** official shall be the final agency decision. The **Licensee** may thereafter exercise any and all administrative or judicial remedies that may be available.
- 13.9 Within **[\*\*]** of expiration or termination of this **Agreement** under this Article 13, a final report shall be submitted by the **Licensee**. Any royalty payments, including those incurred but not yet paid (such as the full minimum annual royalty), and those related to patent expense, due to the **IC** shall become immediately due and payable upon termination or expiration. If terminated under this Article 13, sublicensees may elect to convert their sublicenses to direct licenses with the **IC** pursuant to Paragraph 4.3. Unless otherwise specifically provided for under this **Agreement**, upon termination or expiration of this **Agreement**, the **Licensee** shall return all **Licensed Products** or other materials included within the **Licensed Patent Rights** to the **IC** or provide the **IC** with written certification of the destruction thereof to the extent the same are covered by **Licensed Patent Rights** at such time. The **Licensee** may not be granted additional **IC** licenses if the final reporting requirement is not fulfilled.

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14. GENERAL PROVISIONS

- 14.1 Neither party may waive or release any of its rights or interests in this **Agreement** except in writing. The failure of the **Government** to assert a right hereunder or to insist upon compliance with any term or condition of this **Agreement** shall not constitute a waiver of that right by the **Government** or excuse a similar subsequent failure to perform any of these terms or conditions by the **Licensee**.
- 14.2 This **Agreement** constitutes the entire agreement between the Parties relating to the subject matter of the **Licensed Patent Rights, Licensed Products and Licensed Processes**, and all prior negotiations, representations, agreements, and understandings are merged into, extinguished by, and completely expressed by this **Agreement**.
- 14.3 The provisions of this **Agreement** are severable, and in the event that any provision of this **Agreement** shall be determined to be invalid or unenforceable under any controlling body of law, this determination shall not in any way affect the validity or enforceability of the remaining provisions of this **Agreement**.
- 14.4 If either party desires a modification to this **Agreement**, the parties shall, upon reasonable notice of the proposed modification by the party desiring the change, confer in good faith to determine the desirability of the modification. No modification shall be effective until a written amendment is signed by the signatories to this **Agreement** or their designees.
- 14.5 The construction, validity, performance, and effect of this **Agreement** shall be governed by Federal law as applied by the Federal courts in the District of Columbia.
- 14.6 All **Agreement** notices required or permitted by this **Agreement** shall be given by prepaid, first class, registered or certified mail or by an express/overnight delivery service provided by a commercial carrier, properly addressed to the other party at the address designated on the Signature Page, or to any other address as may be designated in writing by such other party. **Agreement** notices shall be considered timely if such notices are received on or before the established deadline date or sent on or before the deadline date as verifiable by U.S. Postal Service postmark or dated receipt from a commercial carrier. Parties should request a legibly dated U.S. Postal Service postmark or obtain a dated receipt from a commercial carrier or the U.S. Postal Service. Private metered postmarks shall not be acceptable as proof of timely mailing.
- 14.7 This **Agreement** shall not be assigned or otherwise transferred (including any transfer by legal process or by operation of law, and any transfer in bankruptcy or insolvency, or in any other compulsory procedure or order of court), except to the **Licensee's Affiliate(s)** or in connection with a sale or transfer of all or

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substantially all of **Licensee's** business or assets relating to the subject matter of this **Agreement**, whether by merger, sale of assets or otherwise, without the prior written consent of the **IC**. The parties agree that the identity of the parties is material to the formation of this **Agreement** and that the obligations under this **Agreement** are nondelegable, other than to Affiliates or in connection with the merger, sale, or transfer referred to above. In the event that the **IC** approves a proposed assignment, the **Licensee** shall pay the **IC**, as an additional royalty, [\*\*] percent ([\*\*]%) of the fair market value of consideration received specifically related to the assignment of this **Agreement**, as calculated in good faith by the **Licensee**, taking into consideration the relative value of the **Licensed Patent Rights** in the context of all other intellectual property associated with any such assignment, within [\*\*] of the assignment.

- 14.8 The **Licensee** agrees in its use of any **IC**-supplied materials to comply with all applicable statutes, regulations, and guidelines, including the **NIH** and the **HHS** regulations and guidelines. The **Licensee** agrees not to use the materials for research involving human subjects or clinical trials in the United States without complying with 21 CFR Part 50 and 45 CFR Part 46. The **Licensee** agrees not to use the materials for research involving human subjects or clinical trials outside of the United States without notifying the **IC**, in writing, of the research or trials and complying with the applicable regulations of the appropriate national control authorities. Written notification to the **IC** of research involving human subjects or clinical trials outside of the United States shall be given no later than [\*\*] prior to commencement of the research or trials.
- 14.9 The **Licensee** acknowledges that it is subject to and agrees to abide by the United States laws and regulations (including the Export Administration Act of 1979 and Arms Export Control Act) controlling the export of technical data, computer software, laboratory prototypes, biological materials, and other commodities. The transfer of these items may require a license from the appropriate agency of the **Government** or written assurances by the **Licensee** that it shall not export these items to certain foreign countries without prior approval of the agency. The **IC** neither represents that a license is or is not required or that, if required, it shall be issued.
- 14.10 The **Licensee** agrees to mark the **Licensed Products** or their packaging sold in the United States with all applicable U.S. patent numbers and similarly to indicate "Patent Pending" status. All **Licensed Products** manufactured in, shipped to, or sold in other countries shall be marked in a manner to preserve the **IC** patent rights in those countries.
- 14.11 By entering into this **Agreement**, the **IC** does not directly or indirectly endorse any product or service provided, or to be provided, by the **Licensee** whether directly or indirectly related to this **Agreement**. The **Licensee** shall not state or imply that this **Agreement** is an endorsement by the **Government**, the **IC**, any

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other **Government** organizational unit, or any **Government** employee. Additionally, the **Licensee** shall not use the names of the **IC**, **NIH**, **FDA** or **HHS** or the **Government** or their employees in any advertising, promotional, or sales literature without the prior written approval of the **IC**.

- 14.12 The Parties agree to attempt to settle amicably any controversy or claim arising under this **Agreement** or a breach of this **Agreement**, except for appeals of modifications or termination decisions provided for in Article 13. The **Licensee** agrees first to appeal any unsettled claims or controversies to the designated the **IC** official, or designee, whose decision shall be considered the final agency decision. Thereafter, the **Licensee** may exercise any administrative or judicial remedies that may be available.
- 14.13 Nothing relating to the grant of a license, nor the grant itself, shall be construed to confer upon any person any immunity from or defenses under the antitrust laws or from a charge of patent misuse, and the acquisition and use of rights pursuant to 37 CFR Part 404 shall not be immunized from the operation of state or Federal law by reason of the source of the grant.
- 14.14 Paragraphs 8.1, 9.7-9.9, 12.1-12.5, 13.8, 13.9, 14.12 and 14.14 of this **Agreement** shall survive termination of this **Agreement**.
- 14.15 The terms and conditions of this **Agreement** shall, at the **IC**'s sole option, be considered by the **IC** to be withdrawn from the **Licensee**'s consideration and the terms and conditions of this **Agreement**, and the **Agreement** itself to be null and void, unless this **Agreement** is executed by the **Licensee** and a fully executed original is received by the **IC** within [\*\*] from the date of the **IC** signature found at the Signature Page.

SIGNATURES BEGIN ON NEXT PAGE

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[Final] [Torus Therapeutics] [02-02-2017]

SIGNATURE PAGE

For the IC:

/s/ Alan H. Deutch

Name: Alan H. Deutch, Ph.D  
Title: Director  
Office: Office of Technology Transfer and Development (OTTAD)  
NHLBI  
National Institute of Health

2017.02.02

Date:

Mailing Address or Email Address for **Agreement** notices and reports:

License Compliance and Administration  
Monitoring & Enforcement  
Office of Technology Transfer  
National Institutes of Health  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852-3803 U.S.A.

E-Mail: [LicenseNotices\\_Reports@mail.nih.gov](mailto:LicenseNotices_Reports@mail.nih.gov)

For the **Licensee** (Upon, information and belief, the undersigned expressly certifies or affirms that the contents of any statements of the **Licensee** made or referred to in this document are truthful and accurate.):

By:

/s/ Jason Rhodes

Signature of Authorized Official

Date: February 2, 2017

Jason Rhodes

Printed Name

Chairman

Title

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I. Official and Mailing Address for **Agreement** notices:

Jason Rhodes \_\_\_\_\_

Name

Name:

Chairman \_\_\_\_\_

Title:

Mailing Address

Torus Therapeutics, Inc. \_\_\_\_\_

400 Technology Square, 10th Floor \_\_\_\_\_

Cambridge, MA 02139 \_\_\_\_\_

\_\_\_\_\_

Email Address:    [\*\*] \_\_\_\_\_

Phone: \_\_\_\_\_

Fax: \_\_\_\_\_

II. Official and Mailing Address for Financial notices (the **Licensee's** contact person for royalty payments)

Jason Rhodes \_\_\_\_\_

Name

Chairman \_\_\_\_\_

Title

Mailing Address:

Torus Therapeutics, Inc. \_\_\_\_\_

400 Technology Square, 10<sup>th</sup> Floor \_\_\_\_\_

Cambridge, MA 02139 \_\_\_\_\_

\_\_\_\_\_

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---

Email Address: [\*\*]  
Phone: 857-201-2700  
Fax: \_\_\_\_\_

Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31U.S.C. §§1001 (criminal liability including fine(s) and/or imprisonment).

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**APPENDIX C - ROYALTIES**

Royalties:

- I. The **Licensee** agrees to pay to the **IC** a non-creditable, nonrefundable license issue royalty in the amount of [\*\*] (\$[\*\*]) US dollars.
- II. The **Licensee** agrees to pay to the **IC** a nonrefundable minimum annual royalty in the amount of [\*\*] Dollars (\$[\*\*]) as follows:
  - (a) The first minimum annual royalty is due within [\*\*] of the first January 1 following the effective date of this **Agreement**. For the sake of clarity, if the Effective date of this **Agreement** is January 15, 2017, then the first minimum annual royalty shall be due within [\*\*] of January 1, 2018; and
  - (b) Subsequent minimum annual royalty payments are due and payable on January 1 of each calendar year and may be credited against any earned royalties due for sales made in that year.
- III. The **Licensee** agrees to pay the **IC** earned royalties of [\*\*] percent ([\*\*]%) on Net Sales by or on behalf of **Licensee** or its sub licensees on a **Licensed Product-by-Licensed Product** and country-by- country basis until the expiration of the last-to-expire **Licensed Patent Rights** covering such **Licensed Product** in such country. **Licensee** may deduct up to [\*\*] percent ([\*\*]%) of third party payments made by **Licensee**, **Licensee's** Affiliates, or Sublicensee to a third party for **Licensed Product** and/or **Licensed Processes** from the respective royalty due the **IC**, excluding payments made to University of Massachusetts Medical School for licensed technology under agreements that exist as of the effective date of this **Agreement** or for payments made to the **NIH/IC** for closely related technology on which Robert Kotin is an inventor and for which the intellectual property filing is in process as of the effective date of this **Agreement**. However, the royalty payments may not be reduced by more than [\*\*]% of what would have been owed to the **IC** without such allowed reduction, which means that the earned royalties cannot be reduced below [\*\*]%
- IV. The **Licensee** agrees to pay the **IC** one-time **Benchmark** royalties per **Licensed Product** within [\*\*] of achieving each **Benchmark**:
  - (a) [\*\*] \$[\*\*]
  - (b) [\*\*] \$[\*\*]
  - (c) [\*\*] \$[\*\*]
  - (d) [\*\*] \$[\*\*]
  - (e) [\*\*] \$[\*\*]

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Total one-time **Benchmark** payments per **Licensed Product** developed or commercialized under the **Agreement**, regardless of the number of indications in which such **Licensed Product** might be pursued or approved, shall not exceed \$350,000. One-time milestones are not payable for **Licensed Products** that encode a transgene with substantially similar protein function as a **Licensed Product** for which one-time **Benchmark** payments have already been made.

- V. The **Licensee** agrees to pay the **IC** additional sublicensing royalties of **[\*\*]** percent (**[\*\*]**%) of consideration (Sublicense Consideration) received for the granting of each sublicense under Section 4.4 that is reasonably allocable, as determined in good faith by the **Licensee**, to the grant of a sublicense of the rights granted to the **Licensee** under the **Licensed Patent Rights** within **[\*\*]** of the execution of such sublicense. Sub license Consideration shall not include: (a) license maintenance fees, (b) payments made in consideration for the issuance of equity or debt securities of **Licensee**, (c) payments made for the reimbursement of, or committed to, research and development or reimbursement of patent prosecution, defense, enforcement and maintenance costs, (d) milestone or benchmark payments or (e) royalties or other profit sharing.

15A-084-2017; L-1242017/0

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**PUBLIC HEALTH SERVICE**

Amendment

This **Agreement** is based on the model Amendment Agreement adopted by the U.S. Public Health Service (“**PHS**”) Technology Transfer Policy Board for use by components of the National Institutes of Health (“**NIH**”), the Centers for Disease Control and Prevention (“**CDC**”), and the Food and Drug Administration (“**FDA**”), which are agencies of the **PHS** within the Department of Health and Human Services (“**HHS**”).

This Cover Page identifies the Parties to this **Agreement**:

The U.S. Department of Health and Human Services, as represented by  
NHLBI

an Institute or Center (hereinafter referred to as the “**IC**”) of the  
NIH

and

GenerationBio,  
hereinafter referred to as the “**Licensee**”,  
having offices at 301 Binney Street, 4<sup>th</sup> Floor, Cambridge, MA 02142  
created and operating under the laws of Delaware.  
**Tax ID No.:** 814301284

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**First AMENDMENT TO L-124-2017/0**

This is the First amendment (“**First Amendment**”) of the agreement by and between the **IC** and **Licensee** having an effective date of February 02, 2017 and having **IC** Reference Number L-124-2017/0 (“**Agreement**”). This **First Amendment**, having **IC** Reference Number L-124-2017/1 includes, in addition to the amendments made below, 1) a Signature Page, and 2) Attachment 1 (Royalty Payment Information).

WHEREAS, the **IC** and the **Licensee** desire that the **Agreement** be amended a first time as set forth below in order to 1) clarify the dates of patent costs reimbursement, 2) clarify that the nonexclusive license hereunder is granted to **Licensee** by all owners of the Licensed Intellectual Property, and 3) add a new clause related to public health.

NOW, THEREFORE, in consideration of the mutual covenants and promises contained herein, the **IC** and the **Licensee**, intending to be bound, hereby mutually agree to the following:

1) Replace Paragraph 3.1 with the following:

The **IC** and the **Co-Owner** hereby grant and the **Licensee** accepts, subject to the terms and conditions of this **Agreement**, a nonexclusive license under the **Licensed Patent Rights** in the **Licensed Territory** to make and have made, research, have researched, to use and have used, to sell and have sold, to offer to sell, and to import any **Licensed Products** in the **Licensed Fields of Use** and to practice and have practiced any **Licensed Processes** in the **Licensed Fields of Use**. The **IC** represents that it and the **Co-Owner** have entered into an Inter-Institutional Agreement dated June 24, 2019, under which the **IC** has the authority to grant licenses under **Co-Owner’s** ownership rights in the **Licensed Patent Rights**.

2) Replace Paragraph 6.9 with the following:

With regard to unreimbursed expenses associated with the preparation, filing, prosecution, and maintenance of all patent applications and patents included within the **Licensed Patent Rights** and paid by the **Co-Owner** prior to the date listed on the **Co-Owner’s** first request for payment, the **Licensee** shall pay the **Co-Owner**, as an additional royalty, within [\*\*] of the **Co-Owner’s** submission of a request for payment (which will include summaries of patent prosecution invoices) to the **Licensee**, an amount equivalent to the unreimbursed patent expenses previously paid by the **Co-Owner**, divided by the aggregate number of commercial licensees of the **Licensed Patent Rights** that are obligated to reimburse patent expenses under similar such provision, on the date of the request for payment. Notwithstanding, **Licensee** shall not be required to pay more than [\*\*] of such total patent expenses. The payment shall be made in Euro or in US Dollars. The conversion to US Dollars, if applicable, shall be made using the exchange rate published on the day the statement is received by **Licensee**.

Unless otherwise indicated in **Co-Owner’s** request for payment, **Licensee** shall wire its payment obligation under Paragraph 6.9 to the following bank account:

AIM SECTEUR LUCRATIF INSTITUT DE MYOLOGIE  
BNP PARIBAS  
PARIS ASSOCIATIONS (02837) IBAN: [\*\*]  
BIC: [\*\*]  
Tax ID Number: [\*\*]

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3) Replace Paragraph 6.10 with the following:

With regard to reasonable unreimbursed expenses (such that do not include expenses incurred due to unwarranted extensive delays caused by gross negligence of the **Co-Owner**) associated with the preparation, filing, prosecution, and maintenance of all patent applications and patents included within the **Licensed Patent Rights** and paid by the **Co-Owner** on or after the date of the Co-Owner's first request for payment referenced in Paragraph 6.9, the **IC**, at its sole option, may require the **Licensee**

to pay the **Co-Owner** on an annual basis, within [\*\*] of the **Co-Owner's** submission of a request for payment (which will include summaries of patent prosecution invoices), a royalty amount equivalent to these unreimbursed expenses paid during the previous year divided by the aggregate number of commercial licensees of the **Licensed Patent Rights** that are obligated to reimburse patent expenses under a similar such provision;

The payment shall be made in Euro or in US Dollars. The conversion to US Dollars, if applicable, shall be made using the exchange rate published on the day the request for payment is received by **Licensee**.

under exceptional circumstances, the **Licensee** may be given the right to assume responsibility for the preparation, filing, prosecution, or maintenance of any patent application or patent included with the **Licensed Patent Rights**. In that event, the **Licensee** shall directly pay the attorneys or agents engaged to prepare, file, prosecute, or maintain these patent applications or patents and shall provide the **IC** with copies of each invoice associated with these services as well as documentation that these invoices have been paid.

Notwithstanding the foregoing, the **Licensee's** payment obligation under paragraph 6.10 shall not exceed [\*\*] of the total patent expenses for the year.

4) In Article 10: "Performance" add Paragraph 10.5 as follows:

For gene therapy products for the treatment of rare diseases **Licensee** shall make commercially reasonable efforts:

- (i) to pursue broad international patient accessibility for products commercialized under the license such that the conditions of commercialization of the products under the licence shall not constitute a material obstacle to the ability of patients suffering rare diseases to have reasonable access to such product for their own treatment, taking into account any existing and applicable regulatory and reimbursement systems and any other applicable legal and regulatory regimes on a relevant national, international or regional level, and as the case may be, by voluntarily joining accredited programs such as Patient Assistance Program (PAP) or global equivalents thereof, if it is reasonably feasible to do so;

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- (ii) in the event of early termination of the **Agreement**, **Licensee** shall negotiate in good faith with the **Co-Owner**, or the existing and future licensees of the **Licensed Patent Rights**, a license to intellectual property rights that (a) claim the relevant **Licensed Product** or the use of the relevant **Licensed Product**, (b) were generated by **Licensee** as part of its development of the relevant **Licensed Product**, (c) are owned by **Licensee**, and (d) but for having a license under which the manufacture, use, sale, offer for sale or import of the relevant **Licensed Product** by the **Co-Owner** or such existing or future licensee would infringe such intellectual property rights, on market terms and conditions negotiated in a bona fide manner;
  - (iii) to reasonably seek the grant of a conditional (EU) or accelerated (US) approval for eligible patients, in keeping with applicable legal and regulatory regimes; and
  - (iv) to reasonably accommodate requests for pre-approval compassionate access to **Licensed Products** provided that such access is:
    - (a) feasible, (b) in keeping with applicable legal and regulatory regimes, and (c) **Licensee** retains control over the **Licensed Products** and their dissemination.
- 5) In the event any provision(s) of the **Agreement** is/are inconsistent with Attachment 1, such provision(s) is/are hereby amended to the extent required to avoid such inconsistency and to give effect to the shipping and payment information in such Attachment 1.
- 6) All terms and conditions of the **Agreement** not herein amended remain binding and in effect.
- 7) The terms and conditions of this **First Amendment** shall, at the **IC's** sole option, be considered by the **IC** to be withdrawn from the **Licensee's** consideration and the terms and conditions of this **First Amendment**, and the **First Amendment** itself, to be null and void, unless this **First Amendment** is executed by the **Licensee** and a fully executed original is received by the **IC** within [\*\*] from the date of the **IC's** signature found at the Signature Page.
- 8) This **First Amendment** is effective on the date it is executed by all parties.

**SIGNATURES BEGIN ON NEXT PAGE**

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SIGNATURE PAGE

In Witness Whereof, the parties have executed this *First Amendment* on the dates set forth below. Any communication or notice to be given shall be forwarded to the respective addresses listed below.

For the **IC**:

/s/ Vincent A. Kolesnitchenko, Ph.D.  
Vincent Kolesnitchenko, Ph.D. on behalf of Cecilia Pazman, Ph.D.  
Acting Director  
Office of Technology Transfer and Development  
National Heart, Lung, and Blood Institute  
National Institutes of Health

2019.07.10  
Date

Mailing Address or E-mail Address for **Agreement** notices and reports:

License Compliance and Administration  
Monitoring & Enforcement  
Office of Technology Transfer  
National Institutes of Health  
6011 Executive Boulevard, Suite 325  
Rockville, Maryland 20852-3804 U.S.A.

E-mail: LicenseNotices\_Reports@mail.nih.gov

For the **Licensee** (Upon information and belief, the undersigned expressly certifies or affirms that the contents of any statements of the **Licensee** made or referred to in this document are truthful and accurate.):

/s/ Geoffrey McDonough  
Signature of Authorized Official

July 1, 2019  
Date

Name: Geoffrey McDonough  
Title: Chief Executive Officer

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I. Official and Mailing Address for **Agreement** notices:

Jennifer Elliot \_\_\_\_\_

Name

Chief Legal Officer \_\_\_\_\_

Title

Mailing Address:

301 Binney St., Suite 401 \_\_\_\_\_

Cambridge, MA 02142 \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

Email Address: [\*\*] \_\_\_\_\_

Phone: [\*\*] \_\_\_\_\_

Fax: \_\_\_\_\_

II. Official and Mailing Address for Financial notices (the **Licensee's** contact person for royalty payments):

Thomas Graney \_\_\_\_\_

Name

Chief Financial Officer \_\_\_\_\_

Title

Mailing Address:

301 Binney St., Suite 401 \_\_\_\_\_

Cambridge, MA 02142 \_\_\_\_\_

\_\_\_\_\_

\_\_\_\_\_

Email Address: [\*\*] \_\_\_\_\_

Phone: \_\_\_\_\_

Fax: \_\_\_\_\_

Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31 U.S.C. §§3801-3812 (civil liability) and 18 U.S.C. §1001 (criminal liability including fine(s) or imprisonment).

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Subsidiaries of the Registrant

| <u>Entity</u>                         | <u>Jurisdiction of Incorporation</u> |
|---------------------------------------|--------------------------------------|
| Generation Bio Securities Corporation | Massachusetts                        |