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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-Q

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended June 30, 2021

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to_____

Commission File Number: 001-39319

GENERATION BIO CO.
(Exact name of registrant as specified in its charter)

Delaware **(State or other jurisdiction of incorporation or organization)**	**81-4301284** **(I.R.S. Employer Identification Number)**
301 Binney Street **Cambridge, Massachusetts** **(Address of principal executive offices)**	**02142** **(Zip Code)**

(617) 655-7500
(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 Par Value	**GBIO**	**Nasdaq Global Select Market**

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a 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 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of July 30, 2021 there were 56,780,584 shares of Common Stock, $0.0001 par value per share, outstanding.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q, or this Quarterly Report, of Generation Bio Co., contains forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act and Section 21E of the Securities Exchange Act of 1934, as amended, that involve substantial risks and uncertainties. All statements, other than statements of historical fact, contained in this Quarterly Report, 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," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "will," "would," or the negative of these words or other 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 Quarterly Report 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 our existing cash, cash equivalents and marketable securities will be sufficient to fund our operating expenses and capital expenditure requirements;
- the timing of and our ability to complete the build-out and regulatory agency review of our manufacturing facility;
- our ability to operate our manufacturing facility and manufacture for clinical and commercial supply out of the facility;
- the potential advantages of our non-viral genetic medicine platform;
- 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;
- our estimates regarding the potential addressable patient populations for our programs;
- the impact of the COVID-19 pandemic and our response to the pandemic;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our expectations regarding our ability to obtain, maintain, protect, defend and enforce our 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;
- 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; and

- our ability to maintain and establish collaborations or obtain additional funding.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and stockholders should not place undue reliance on our forward-looking statements. Actual results or events could 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 Quarterly Report, particularly in the "Risk Factors" section in this Quarterly Report and our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Moreover, we operate in a competitive and rapidly changing environment. 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.

Stockholders should read this Quarterly Report and the documents that we file with the SEC with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report are made as of the date of this Quarterly Report, 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.

Except where the context otherwise requires or where otherwise indicated, the terms "we," "us," "our," "our company," "the company," and "our business" in this Quarterly Report refer to Generation Bio Co. and its consolidated subsidiary.

Generation Bio Co.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements (unaudited)

Generation Bio Co.
Condensed Consolidated Balance Sheets
(In thousands, except share and per share amounts)
(Unaudited)

	June 30, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 392,072	$ 62,889
Marketable securities	33,121	199,438
Prepaid expenses and other current assets	7,548	5,408
Total current assets	432,741	267,735
Property and equipment, net	23,609	23,781
Operating lease right-of-use assets	31,988	—
Restricted cash	2,051	2,051
Deferred offering costs	—	336
Other long-term assets	403	252
Total assets	$ 490,792	$ 294,155
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 589	$ 267
Accrued expenses and other current liabilities	9,055	10,953
Operating lease liability	4,287	—
Total current liabilities	13,931	11,220
Operating lease liability, net of current portion	43,459	—
Deferred rent, net of current portion	—	14,922
Total liabilities	57,390	26,142
Commitments and contingencies (Note 9)		
Stockholders' equity:		
Preferred stock, $0.0001 par value; 5,000,000 shares authorized and no shares issued or outstanding at June 30, 2021 and December 31, 2020	—	—
Common stock, $0.0001 par value; 150,000,000 shares authorized at June 30, 2021 and December 31, 2020; 56,732,310 and 46,970,012 shares issued at June 30, 2021 and December 31, 2020, respectively; 56,431,513 and 46,291,877 shares outstanding at June 30, 2021 and December 31, 2020, respectively	6	5
Additional paid-in capital	678,720	456,974
Accumulated other comprehensive income	4	9
Accumulated deficit	(245,328)	(188,975)
Total stockholders' equity	433,402	268,013
Total liabilities and stockholders' equity	$ 490,792	$ 294,155

The accompanying notes are an integral part of these condensed consolidated financial statements.

Generation Bio Co.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(In thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	$ 22,656	$ 13,456	$ 41,409	$ 26,850
General and administrative	8,186	4,308	15,088	8,950
Total operating expenses	30,842	17,764	56,497	35,800
Loss from operations	(30,842)	(17,764)	(56,497)	(35,800)
Other income:				
Interest income	51	33	144	352
Net loss and net loss attributable to common stockholders	$ (30,791)	$ (17,731)	$ (56,353)	$ (35,448)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.55)	$ (1.50)	$ (1.01)	$ (4.10)
Weighted average common shares outstanding, basic and diluted	56,318,025	11,801,704	55,843,348	8,648,358
Comprehensive loss:				
Net loss	$ (30,791)	$ (17,731)	$ (56,353)	$ (35,448)
Other comprehensive loss:				
Unrealized losses on marketable securities	(6)	(4)	(5)	(4)
Comprehensive loss	$ (30,797)	$ (17,735)	$ (56,358)	$ (35,452)

The accompanying notes are an integral part of these condensed consolidated financial statements.

Generation Bio Co.
Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)
(In thousands, except share amounts)
(Unaudited)

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
	Three Months Ended June 30, 2021							
Balances at March 31, 2021	—	$ —	56,094,529	$ 6	$ 673,257	$ 10	$ (214,537)	$ 458,736
Issuance of common stock upon exercise of stock options	—	—	155,942	—	731	—	—	731
Vesting of restricted common stock	—	—	165,531	—	—	—	—	—
Issuance of common stock under other equity plans	—	—	15,511	—	355	—	—	355
Stock-based compensation expense	—	—	—	—	4,377	—	—	4,377
Unrealized losses on marketable securities	—	—	—	—	—	(6)	—	(6)
Net loss	—	—	—	—	—	—	(30,791)	(30,791)
Balances at June 30, 2021	—	$ —	56,431,513	$ 6	$ 678,720	$ 4	$ (245,328)	$ 433,402

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
	Three Months Ended June 30, 2020							
Balances at March 31, 2020	46,361,960	$ 224,425	5,570,652	$ 1	$ 11,517	$ —	$ (126,169)	$ (114,651)
Conversion of convertible preferred stock into common stock upon initial public offering	(46,361,960)	(224,425)	27,094,085	3	224,422	—	—	224,425
Issuance of common stock upon initial public offering, net of issuance costs of $3,185			12,105,263	1	210,714	—	—	210,715
Issuance of common stock upon exercise of stock options	—	—	5,434	—	31	—	—	31
Vesting of restricted common stock	—	—	252,590	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	1,368	—	—	1,368
Unrealized losses on marketable securities	—	—	—	—	—	(4)	—	(4)
Net loss	—	—	—	—	—	—	(17,731)	(17,731)
Balances at June 30, 2020	—	$ —	45,028,024	$ 5	$ 448,052	$ (4)	$ (143,900)	$ 304,153

The accompanying notes are an integral part of these condensed consolidated financial statements.

Generation Bio Co.
Condensed Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)
(In thousands, except share amounts)
(Unaudited)

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount	Shares	Amount				
	Six Months Ended June 30, 2021							
Balances at December 31, 2020	—	$ —	46,291,877	$ 5	$ 456,974	$ 9	$ (188,975)	$ 268,013
Issuance of common stock upon public offering, net of issuance costs of $590	—	—	9,200,000	1	211,285	—	—	211,286
Issuance of common stock upon exercise of stock options	—	—	543,520	—	2,250	—	—	2,250
Vesting of restricted common stock	—	—	380,605	—	—	—	—	—
Issuance of common stock under other equity plans	—	—	15,511	—	355	—	—	355
Stock-based compensation expense	—	—	—	—	7,856	—	—	7,856
Unrealized losses on marketable securities	—	—	—	—	—	(5)	—	(5)
Net loss	—	—	—	—	—	—	(56,353)	(56,353)
Balances at June 30, 2021	—	$ —	56,431,513	$ 6	$ 678,720	$ 4	$ (245,328)	$ 433,402

	Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive (Loss)	Accumulated Deficit	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount				
	Six Months Ended June 30, 2020							
Balances at December 31, 2019	26,425,664	$ 115,593	5,270,889	$ 1	$ 9,859	$ —	$ (108,452)	$ (98,592)
Issuance of Series C convertible preferred stock, net of issuance costs of $2,640	19,936,296	108,832	—	—	—	—		—
Conversion of convertible preferred stock into common stock upon initial public offering	(46,361,960)	(224,425)	27,094,085	3	224,422	—		224,425
Issuance of common stock upon initial public offering, net of issuance costs of $3,185	—	—	12,105,263	1	210,714	—		210,715
Issuance of common stock upon exercise of stock options	—	—	40,263	—	185	—	—	185
Vesting of restricted common stock	—	—	517,524	—	—	—	—	—
Stock-based compensation expense	—	—	—	—	2,872	—	—	2,872
Unrealized losses on marketable securities	—	—	—	—	—	(4)	—	(4)
Net loss	—	—	—	—	—	—	(35,448)	(35,448)
Balances at June 30, 2020	—	$ —	45,028,024	$ 5	$ 448,052	$ (4)	$ (143,900)	$ 304,153

The accompanying notes are an integral part of these condensed consolidated financial statements.

Generation Bio Co.
Condensed Consolidated Statements of Cash Flows
(In thousands)
(Unaudited)

	Six Months Ended June 30,	
	2021	2020
Cash flows from operating activities:		
Net loss	$ (56,353)	$ (35,448)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	7,856	2,872
Depreciation and amortization expense	2,194	1,502
Amortization (accretion) of premium (discount) on marketable securities, net	412	—
Changes in operating assets and liabilities:		
Tenant receivable	—	448
Prepaid expenses and other current assets	(2,140)	(2,514)
Other noncurrent assets	(584)	(151)
Accounts payable	322	799
Accrued expenses and other current liabilities	(171)	(304)
Deferred rent	—	(468)
Net cash used in operating activities	(48,464)	(33,264)
Cash flows from investing activities:		
Purchases of property and equipment	(2,183)	(2,223)
Purchases of marketable securities	—	(129,399)
Maturities of marketable securities	165,900	—
Net cash provided by (used in) investing activities	163,717	(131,622)
Cash flows from financing activities:		
Proceeds from issuance of convertible preferred stock, net of issuance costs incurred and paid in current period	—	108,854
Proceeds from public offering of common stock, net of underwriting discounts and commissions	211,876	213,900
Payment of offering costs	(550)	(1,493)
Proceeds from exercise of stock options	2,604	185
Net cash provided by financing activities	213,930	321,446
Net increase in cash, cash equivalents and restricted cash	329,183	156,560
Cash, cash equivalents and restricted cash at beginning of period	64,940	17,183
Cash, cash equivalents and restricted cash at end of period	$ 394,123	$ 173,743
Supplemental disclosure of noncash investing and financing information:		
Conversion of convertible preferred stock to common stock	$ —	$ 224,425
Purchases of property and equipment included in accounts payable and accrued expenses	$ 333	$ 900
Offering costs included in accounts payable and accrued expenses	$ 118	$ 1,692

The accompanying notes are an integral part of these condensed consolidated financial statements.

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

1. Nature of the Business and Basis of Presentation

Generation Bio Co., or Generation Bio, was incorporated on October 21, 2016 as Torus Therapeutics, Inc. and subsequently changed its name to Generation Bio Co. Generation Bio and its consolidated subsidiary, or the company, we, our or us, are innovating genetic medicines to provide durable, redosable treatments for potentially hundreds of millions of patients living with rare and prevalent diseases. Our non-viral genetic medicine platform incorporates our high-capacity DNA construct called closed-ended DNA, or ceDNA; our cell-targeted lipid nanoparticle delivery system, or ctLNP; and our highly scalable capsid-free manufacturing process that uses our proprietary cell-free rapid enzymatic synthesis, or RES, to produce ceDNA. Using our approach, we are developing novel genetic medicines 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 genetic medicines to be redosable, which may enable individualized patient titration to reach the desired therapeutic expression and to maintain efficacy throughout a patient's life. We are headquartered in Cambridge, Massachusetts.

We are 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, uncertainties regarding the timing and ability to complete the build-out of our manufacturing facility, 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 our development efforts are successful, it is uncertain when, if ever, we will realize significant revenue from product sales.

In June 2020, we completed our initial public offering, or IPO, pursuant to which we issued and sold 12,105,263 shares of our common stock, including 1,578,947 shares pursuant to the full exercise of the underwriters' option to purchase additional shares resulting in net proceeds of $210.7 million, after deducting underwriting discounts and commissions and other offering expenses. Upon the closing of the IPO, all of our outstanding convertible preferred stock automatically converted into shares of common stock. In January 2021, we issued and sold 9,200,000 shares of our common stock, including 1,200,000 shares pursuant to the full exercise of the underwriters' option to purchase additional shares, in a follow-on public offering, resulting in net proceeds of $211.3 million after deducting underwriting discounts and commissions and other offering expenses.

The accompanying condensed 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. Since inception, we have funded our operations with proceeds from the sales of instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), the sale of convertible preferred stock (which converted into common stock in 2020), and most recently, with proceeds from the sale of common stock in underwritten public offerings. We have incurred recurring losses, including net losses of $56.4 million for the six months ended June 30, 2021 and $35.4 million for the six months ended June 30, 2020. As of June 30, 2021, we had an accumulated deficit of $245.3 million. We expect to continue to generate operating losses in the foreseeable future. As of August 11, 2021, the issuance date of these condensed consolidated financial statements, we expect that our cash, cash equivalents and marketable securities will be sufficient to fund our operating expenses and capital expenditure requirements for at least 12 months.

We will need to obtain additional funding through public or private equity offerings, debt financings, collaborations, strategic alliances and/or licensing arrangements. We may not be able to obtain financing on acceptable terms, or at all, and we 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 our stockholders. Arrangements with collaborators or others may require us to relinquish rights to certain of our technologies or programs. If we are unable to obtain funding, we could

Generation Bio Co.
Notes to Condensed Consolidated Financial Statements
(Unaudited)

be forced to delay, reduce or eliminate some or all of our research and development programs, pipeline expansion or commercialization efforts, which could adversely affect our business prospects. Although management will continue to pursue these plans, there is no assurance that we will be successful in obtaining sufficient funding on terms acceptable to us to fund continuing operations when needed or at all.

The accompanying condensed consolidated financial statements reflect the operations of Generation Bio and our wholly owned subsidiary, Generation Bio Securities Corporation. Intercompany balances and transactions have been eliminated in consolidation. The accompanying condensed consolidated financial statements have been prepared in conformity with generally accepted accounting principles in the United States of America, or GAAP. Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification, or ASC, and Accounting Standards Update, or ASU, of the Financial Accounting Standards Board, or 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 expenses during the reporting periods. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, the accrual of research and development expenses and stock-based compensation expense. We base our estimates on historical experience, known trends and other market-specific or other relevant factors that we believe 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 interim financial information

The condensed consolidated balance sheet as of December 31, 2020 was derived from audited financial statements but does not include all disclosures required by GAAP. The accompanying unaudited financial statements as of June 30, 2021 and for the three and six months ended June 30, 2021 and 2020 have been prepared by us pursuant to the rules and regulations of the Securities and Exchange Commission, or SEC, for interim financial statements. Certain information and footnote disclosures normally included in financial statements prepared in accordance with GAAP have been condensed or omitted pursuant to such rules and regulations. These financial statements should be read in conjunction with our audited financial statements included in our Annual Report on Form 10-K that was most recently filed with the SEC. In the opinion of management, all adjustments, consisting only of normal recurring adjustments necessary for a fair presentation of our financial position as of June 30, 2021, the results of operations for the three and six months ended June 30, 2021 and 2020, and cash flows for the six months ended June 30, 2021 and 2020 have been made. The results of operations for the three and six months ended June 30, 2021 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2021 or any other period.

Concentrations of credit risk and of significant suppliers

Financial instruments that potentially expose us to concentrations of credit risk consist primarily of cash, cash equivalents, and marketable securities. We believe that we are not exposed to significant credit risk due to the financial strength of the depository institutions in which our cash and cash equivalents are held. We maintain our cash equivalents in money market funds that invest in U.S. treasury securities. Our marketable securities as of June 30, 2021 consisted of U.S. government treasury securities. We have adopted an investment policy that limits the amounts that we may invest in the securities of single issuer with the exclusion of the U.S. government. We have not experienced any credit losses.

Generation Bio Co.
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(Unaudited)

We are dependent on a small number of third-party suppliers for our drug substance and drug product. In particular, we rely, 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 for our programs. These programs could be adversely affected by a significant interruption in the supply process.

Marketable securities

Our marketable securities that consist of debt securities as of June 30, 2021 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. Effective January 1, 2021, we adopted ASU 2016-13, Financial Instruments – Credit Losses (Topic 326), using the effective date method. As we have never recorded any other-than-temporary-impairment adjustments to our available-for-sale debt securities prior to January 1, 2021, no transition provisions are applicable to our condensed consolidated financial statements and related disclosures.

We assess our available-for-sale debt securities under the available-for-sale debt security impairment model in Topic 326 as of each reporting date in order to determine if a portion of any decline in fair value below carrying value recognized on our available-for-sale debt securities is the result of a credit loss. We record credit losses in the condensed consolidated statements of operations and comprehensive loss as credit loss expense, which is limited to the difference between the fair value and the amortized cost of the security. To date, we have not recorded any credit losses on our available-for-sale debt securities.

Leases

We determine whether a contract is, or contains, a lease at inception. We classify each of our leases as operating or financing considering factors such as the length of the lease term, the present value of the lease payments, the nature of the asset being leased, and the potential for ownership of the asset to transfer during the lease term. Leases with terms greater than one-year are recognized on the condensed consolidated balance sheets as right-of-use assets and lease liabilities and are measured at the present value of the fixed payments due over the expected lease term less the present value of any incentives, rebates or abatements we expect to receive from the lessor. Options to extend a lease are included in the expected lease term if exercise of the option is deemed reasonably certain. Costs determined to be variable and not based on an index or rate are not included in the measurement of the lease liability and are expensed as incurred. The interest rate implicit in lease contracts is typically not readily determinable. As such, we utilize the appropriate incremental borrowing rate, which is the rate incurred to borrow on a collateralized basis an amount equal to the lease payments over a similar term and in a similar economic environment. To estimate our incremental borrowing rate, a credit rating applicable to our company is estimated using a synthetic credit rating analysis since we do not currently have a rating agency-based credit rating. We record expense to recognize fixed lease payments on a straight-line basis over the expected lease term. We have elected the practical expedient not to separate lease and non-lease components for real estate leases.

Recently adopted accounting pronouncements

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842). The 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 provided 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 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 was permitted for all entities. In November 2019, the FASB issued ASU 2019-10, which deferred the effective date for

Generation Bio Co.
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nonpublic entities to annual reporting periods beginning after December 15, 2020, and interim periods within fiscal years beginning after December 15, 2021. In June 2020, the FASB issued ASU 2020-05, which granted a one-year effective-date delay for nonpublic entities to annual reporting periods beginning after December 15, 2021 and to interim periods within fiscal years beginning after December 15, 2022. Early adoption continues to be allowed. We early adopted 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. We elected 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. Please read Note 5 for additional disclosures related to accounting for leases under this new standard. The adoption of the new standard has had a material impact on our condensed consolidated balance sheet as the standard requires us to measure and recognize a right-of-use asset and lease liability. As most leases do not provide an implicit rate, our incremental borrowing rate was determined based on the information available at the date of adoption to measure our lease liability. Costs determined to be variable and not based on an index or rate were not included in the measurement of the lease liability. We recognized a lease liability and related right-of-use asset on our condensed consolidated balance sheet of approximately $49.7 million and $33.4 million, net of deferred rent, respectively, as of January 1, 2021, which are presented as separate line items on the condensed consolidated balance sheet as of June 30, 2021. The adoption of the standard did not have a material impact on our condensed consolidated statement of operations and comprehensive loss and did not require a cumulative adjustment to accumulated deficit on our condensed consolidated statement of stockholders' equity as of June 30, 2021.

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 was 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. We adopted ASU 2016-13 on January 1, 2021 and the adoption did not have a material impact on our condensed consolidated financial statements and related disclosures.

In December 2019, the FASB issued ASU No. 2019-12, Income Taxes – Simplifying the Accounting for Income Taxes (Topic 740). The amendments in this update simplify the accounting for income taxes by removing certain exceptions to the general principles as well as clarifying and amending existing guidance to improve consistent application. For public entities, the guidance was effective for annual reporting periods beginning after December 15, 2020 and for interim periods within those fiscal years. For nonpublic entities, the guidance is effective for annual reporting periods beginning after December 15, 2021 and to interim periods within fiscal years beginning after December 15, 2022. Early adoption is permitted for all entities. Depending on the amendment, adoption may be applied on the retrospective, modified retrospective or prospective basis. We early adopted the amendments as of January 1, 2021 on a prospective basis. The amendments did not have a significant impact on our condensed consolidated financial statements and related disclosures.

Recently issued accounting pronouncements

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies that we adopt as of the specified effective date. As an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, we 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, we could adopt the new or revised standard at the time nonpublic companies adopt the new or revised standard. However, as of June 30, 2021, the market value of our common stock that was held by non-affiliates exceeded $700.0 million, and as a result, we will no longer qualify as an emerging growth company as of December 31,

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2021. Therefore, as of that date, we will no longer be able to take advantage of the extended transition period for adopting new or revised accounting standards.

3. Marketable Securities and Fair Value Measurements

The following tables present our marketable securities by security type:

	As of June 30, 2021			
(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
U.S. treasury securities	$ 33,117	$ 4	$ —	$ 33,121

	As of December 31, 2020			
(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury securities	$ 129,446	$ 9	$ (4)	$ 129,451
Commercial paper	52,441	—	—	52,441
Corporate debt securities	17,542	4	—	17,546
Total	$ 199,429	$ 13	$ (4)	$ 199,438

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As of June 30, 2021 and December 31, 2020, marketable securities consisted of investments that mature within one year.

The following tables present our assets that are measured at fair value on a recurring basis and indicate the level within the fair value hierarchy of the valuation techniques that we utilized to determine such fair value:

	Fair Value Measurements at June 30, 2021 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 226,378	$ —	$ —	$ 226,378
Marketable securities:				
U.S. treasury securities	—	33,121	—	33,121

	Fair Value Measurements at December 31, 2020 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 63,827	$ —	$ —	$ 63,827
Marketable securities:				
U.S. treasury securities	—	129,451	—	129,451
Commercial paper	—	52,441	—	52,441
Corporate debt securities	—	17,546	—	17,546
Total	$ 63,827	$ 199,438	$ —	$ 263,265

Money market funds were valued based on quoted market prices, which represent a Level 1 measurement within the fair value hierarchy. Our marketable securities, which have consisted of U.S. treasury securities, commercial paper and corporate debt securities were valued using quoted prices in active markets for similar securities, which represent a Level 2 measurement within the fair value hierarchy.

4. Accrued Expenses

Accrued expenses and other current liabilities consisted of the following:

(in thousands)	June 30, 2021	December 31, 2020
Accrued employee compensation and benefits	$ 3,751	$ 6,150
Accrued external research and development expenses	3,028	1,772
Accrued professional fees	1,058	940
Deferred rent	—	1,389
Other	1,218	702
Total	$ 9,055	$ 10,953

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5. Leases

We lease our office and laboratory space under a noncancelable operating lease that was entered into in August 2018, amended in July 2019 and June 2020, and expires in 2029. We have an option to extend the lease term for one additional term of five years at the greater of the then-current base rent or the then-current fair market value. Exercise of this option was not determined to be reasonably certain and thus was not considered in determining the operating lease liability on the condensed consolidated balance sheet as of June 30, 2021. We posted a letter of credit in the amount of approximately $2.1 million as a security deposit. The letter of credit is subject to increase if we were to sublease any portion of the leased premises. This lease does not include any restrictions or covenants that had to be accounted for under the lease guidance.

The following table presents our costs included in operating expenses related to the operating lease:

(in thousands)	For the Three Months Ended June 30, 2021	For the Six Months Ended June 30, 2021
Operating lease cost	$ 1,504	$ 3,009
Variable lease cost	446	892
Total	$ 1,950	$ 3,901

Net cash paid for the amounts included in the measurement of the operating lease liability on the condensed consolidated balance sheet and operating activities in our condensed consolidated statement of cash flow was $3.6 million for the period ending June 30, 2021. The weighted-average remaining lease term and weighted-average incremental borrowing rate for all leases as of June 30, 2021 was 7.8 years and 6.5%, respectively.

Future lease payments for our noncancelable operating lease as of June 30, 2021 and a reconciliation to the carrying amount of the operating lease liability presented in the condensed consolidated balance sheet as of June 30, 2021 is as follows:

Year Ending December 31,	(in thousands)
2021 (remaining 6 months)	$ 3,681
2022	7,267
2023	7,441
2024	7,679
2025	7,881
Thereafter	27,570
Total undiscounted payments due under operating leases	61,519
Less imputed interest	(13,773)
Total	$ 47,746
Current operating lease liability	$ 4,287
Non-current operating lease liability	43,459
Total	$ 47,746

6. Convertible Preferred Stock

Prior to the IPO, we had issued Series A convertible preferred stock, or Series A, Series B convertible preferred stock, or Series B, and Series C convertible preferred stock, or Series C. Collectively the Series A, Series B and Series C are referred to as the Convertible Preferred Stock.

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On January 9, 2020, we issued and sold 19,936,296 shares of Series C at a price of $5.5914 per share for gross proceeds of $111.5 million. We incurred issuance costs in connection with this transaction of $2.6 million.

Upon issuance of each class of Convertible Preferred Stock, we assessed the embedded conversion and liquidation features of the shares and determined that such features did not require us to separately account for these features. We also concluded that no beneficial conversion feature existed on the issuance date of each class of Convertible Preferred Stock.

Upon the closing of the IPO in June 2020, our Convertible Preferred Stock automatically converted into 27,094,085 shares of common stock.

7. Equity

As of June 30, 2021, our amended and restated certificate of incorporation authorizes us to issue 150,000,000 shares of common stock, par value $0.0001 per share, and 5,000,000 shares of preferred stock, par value $0.0001 per share, all of which preferred stock is undesignated.

In January 2021, we issued and sold 9,200,000 shares of our common stock, including 1,200,000 shares pursuant to the full exercise of the underwriters' option to purchase additional shares, in a follow-on public offering, resulting in net proceeds of $211.3 million after deducting underwriting discounts and commissions and other offering expenses.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of our stockholders. Common stockholders are not entitled to receive dividends, unless declared by the board of directors.

8. Stock-Based Compensation

Stock incentive plans

Our 2017 Stock Incentive Plan, or the 2017 Plan, provided for us to grant incentive stock options or nonstatutory stock options, restricted stock, restricted stock units and other equity awards to employees, non-employees, and directors. In January 2020, the number of shares of common stock authorized for issuance under the 2017 Plan was increased from 8,407,405 shares to 10,275,717 shares.

In May 2020, our board of directors adopted, and in June 2020, our stockholders approved, the 2020 Stock Incentive Plan, or the 2020 Plan and, together with the 2017 Plan, the Plans, which became effective on June 11, 2020. 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. The number of shares of common stock reserved for issuance under the 2020 Plan is the sum of (1) 2,547,698 shares; plus (2) the number of shares (up to a maximum of 7,173,014 shares) as was equal to the sum of (x) the number of shares of common stock reserved for issuance under the 2017 Plan that remained available for grant under the 2017 Plan on June 11, 2020 and (y) the number of shares of 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 lesser of (i) 4% of the number of shares of common stock outstanding on such date, and (ii) an amount determined by the board of directors. In January 2021, the number of shares of common stock authorized for issuance under the 2020 Plan was increased from 10,275,717 shares to 12,154,517 shares. Upon the effectiveness of the 2020 Plan, we ceased granting additional awards under the 2017 Plan.

The Plans are 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 Plans are determined at the

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discretion of the board of directors, or its committee if so delegated. Stock options granted under the Plans 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 of the date of grant. Prior to our IPO, fair value of common stock was determined by the board of directors. Subsequent to the IPO, fair value of common stock is based on quoted market prices.

As of June 30, 2021, 3,069,610 shares remained available for future issuance under the 2020 Plan. Shares subject to outstanding awards granted under the Plans that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by us at their original issuance price pursuant to a contractual repurchase right will be available for future awards under the 2020 Plan.

Grant of stock options

During the six months ended June 30, 2021, we granted service-based options to certain employees, non-employees, and directors for the purchase of 1,633,789 shares of common stock with a weighted average grant date fair value of $20.05 per share that vest over a weighted average period of approximately four years.

Employee stock purchase plan

In May 2020, our board of directors adopted, and in June 2020, our stockholders approved, the 2020 Employee Stock Purchase Plan, or the 2020 ESPP, which became effective June 11, 2020. The 2020 ESPP is administered by our board of directors or by a committee appointed by the board of directors. The number of shares of 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) 1,302,157 shares of common stock, (2) 1% of the number of shares of common stock outstanding on such date, and (3) an amount determined by the board of directors. In January 2021, the number of shares of common stock authorized for issuance under the 2020 ESPP was increased from 481,231 shares to 950,931 shares. During the six months ended June 30, 2021, we issued 15,511 shares of common stock under the 2020 ESPP and 935,420 shares remain available for issuance as of June 30, 2021.

Stock-based compensation

Stock-based compensation expense was classified in the condensed consolidated statements of operations and comprehensive loss as follows:

(in thousands)	Three Months Ended June 30, 2021	Three Months Ended June 30, 2020	Six Months Ended June 30, 2021	Six Months Ended June 30, 2020
Research and development expenses	$ 2,376	$ 936	$ 4,274	$ 1,763
General and administrative expenses	2,001	432	3,582	1,109
Total	$ 4,377	$ 1,368	$ 7,856	$ 2,872

As of June 30, 2021, total unrecognized compensation cost related to unvested stock-based awards was $51.0 million, which is expected to be recognized over a weighted average period of 2.8 years. Additionally, as of June 30, 2021, we had unrecognized compensation cost related to unvested stock-based awards with performance-based vesting conditions for which performance has not been deemed probable of $1.9 million.

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9. Commitments and Contingencies

401(k) Plan

We have a defined-contribution plan under Section 401(k) of the Internal Revenue Code of 1986, as amended, or 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. In September 2020, we adopted a match program, beginning on January 1, 2021, for employee contributions to the 401(k) Plan up to a maximum of four percent of the employee's salary, subject to the maximums established under U.S. Internal Revenue Code of 1986, as amended.

Indemnification agreements

In the ordinary course of business, we 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, we have entered into indemnification agreements with members of our board of directors and our executive officers that will require us, 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 we could be required to make under these indemnification agreements is, in many cases, unlimited. We have not incurred any material costs as a result of such indemnifications and are not currently aware of any indemnification claims.

Legal proceedings

We, from time to time, may be party to litigation arising in the ordinary course of business. We were not subject to any material legal proceedings during the six months ended June 30, 2021.

10. Net Loss per Share

We have 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. We 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:

	June 30,	
	2021	2020
Unvested restricted common stock	300,797	1,209,072
Unvested restricted common stock units	16,400	—
Stock options to purchase common stock	6,008,215	5,439,546
	6,325,412	6,648,618

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11. Subsequent Event

In July 2021, we entered into a 12-year operating lease agreement to build out an approximately 104,000 square foot current Good Manufacturing Practice-, or cGMP-, compliant manufacturing facility in Waltham, Massachusetts to scale ceDNA manufacturing utilizing RES for cGMP-compliant clinical and initial commercial supply. In addition, the new facility will house expanded capacity for research production and process development activities. Subject to certain conditions, the lease is anticipated to commence in June 2022, and monthly rent payments are expected to begin in September 2022. In connection with the lease, we provided a security deposit of $3.6 million in the form of a letter of credit. We will pay an initial monthly base rent of approximately $0.4 million that will increase annually, up to an estimated monthly base rent of $0.8 million. We have an option to extend the lease term for two additional terms of five years at the then-current fair market value. We are obligated to pay operating costs, taxes and utilities applicable to the facility. We will be responsible for costs of constructing interior improvements within the facility that exceed a construction allowance of $26.0 million provided by the landlord.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q, or Quarterly Report, and our consolidated financial statements and related notes appearing in our most recently filed Annual Report on Form 10-K, or Annual Report, with the Securities and Exchange Commission, or SEC. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, 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 Quarterly Report and in our Annual Report, 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 innovating genetic medicines to provide durable, redosable treatments for potentially hundreds of millions of patients living with rare and prevalent diseases. Our non-viral genetic medicine platform incorporates our high-capacity DNA construct called closed-ended DNA, or ceDNA; our cell-targeted lipid nanoparticle delivery system, or ctLNP; and our highly scalable capsid-free manufacturing process that uses our proprietary cell-free rapid enzymatic synthesis, or RES, to produce ceDNA. Using our approach, we are developing novel genetic medicines 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 genetic medicines 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. The combination of the expected multi-year durability of a single dose of ceDNA, tissue-specific delivery and manufacturing capacity may enable dosing for hundreds of millions of patients living with prevalent diseases.

We are advancing a broad and expansive portfolio of programs, including rare and prevalent diseases of the liver and retina. We are focused on diseases with significant unmet need for which our non-viral genetic medicine platform may substantially improve clinical efficacy relative to current gene therapy approaches. We are initially prioritizing 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 to include rare and prevalent diseases of the skeletal muscle, the central nervous system, or CNS, and oncology by developing discrete ctLNPs, each engineered to reach a different tissue.

Additionally, we believe our non-viral genetic medicine 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 endogenous therapeutic antibody production, or ETAP, programs across multiple therapeutic areas. Data from an *in vivo* study conducted as part of our research collaboration with Vir Biotechnology, Inc. showed that ceDNA delivered via LNP enabled mice to generate persistent anti-spike protein human antibody concentrations with a peak level of 8µg/ml, which corresponds to a level that may be therapeutically relevant in humans. Furthermore, endogenously produced antibodies in the serum of ceDNA-treated mice retained binding and functional activity, neutralizing SARS-CoV-2 *ex vivo* at the same level as recombinantly produced monoclonal antibodies.

Our most advanced liver disease programs are in hemophilia A and phenylketonuria, or PKU, which are in the preclinical stage of development, and our most advanced retina disease programs are in Leber's Congenital Amaurosis 10, or LCA10, and Stargardt disease, which are in the lead optimization stage of development. In the preclinical stage of development, we are conducting additional *in vivo* studies to identify development candidates and are assessing these candidates in investigational new drug, or IND, -enabling studies. We plan to submit an IND application for our hemophilia A program in 2023, and expect to report Factor VIII expression data in non-human primates by year-end. In the lead optimization stage, we are seeking to identify ceDNA constructs that provide disease relevant expression in an animal model.

In parallel with our platform development, we are developing the constructs and manufacturing capacity to rapidly advance new disease programs in a tissue or therapeutic area once human proof of concept is established. We have developed novel, next-generation rapid enzymatic approach to manufacture ceDNA that does not rely on Sf9 cells. Instead, the process uses enzymes to convert plasmid DNA into ceDNA, similar to the current high-capacity methods used to manufacture

messenger RNA, or mRNA vaccines. In comparison to Sf9 manufacturing, RES has consistently yielded highly pure ceDNA, reduced ceDNA variability, and shortened the ceDNA production cycle time from 28 days to one day. We expect that scaling RES may enable us to manufacture our potential drug candidates in a cost-effective manner and to expand access to patients with prevalent diseases, requiring hundreds of millions of doses, on a sustainable basis. We plan to transition all of our portfolio programs to RES.

Additionally, to realize the full potential of RES, in July 2021, we entered into a lease agreement with Zinc II PropCo 2020, LLC to build out an approximately 104,000 square foot current Good Manufacturing Practice-, or cGMP-, compliant manufacturing facility in Waltham, Massachusetts. The facility, expected to be operational in 2023, will be designed to scale ceDNA manufacturing utilizing RES for cGMP-compliant clinical and initial commercial supply. In addition, the new facility will house expanded capacity for research production and process development activities. We plan to invest up to $45 million in the new manufacturing facility over the next two years, and we believe this investment will allow us to realize the potential of RES, maximizing the value of our platform and accelerating the development of subsequent programs. We plan to continue to rely on contract manufacturing organizations during and after construction to provide redundancy and secure additional ceDNA supply.

Since our inception in October 2016, we have focused substantially all of our resources on building our non-viral genetic medicine 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 (which converted into common stock in 2020) and, most recently, with proceeds from the sale of common stock in our public offerings. In June 2020, we completed our initial public offering, or IPO, pursuant to which we issued and sold 12,105,263 shares of our common stock, including 1,578,947 shares sold by us pursuant to the full exercise of the underwriters' option to purchase additional shares. We received net proceeds of $210.7 million, after deducting underwriting discounts and commissions and other offering expenses. In January 2021, we issued and sold 9,200,000 shares of our common stock, including 1,200,000 shares sold by us pursuant to the full exercise of the underwriters' option to purchase additional shares, in a follow-on public offering, resulting in net proceeds of $211.3 million after deducting underwriting discounts and commissions and other offering expenses.

Historically, 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 six months ended June 30, 2021, we reported net losses of $56.4 million, and for the six months ended June 30, 2020, we reported net losses of $35.4 million. As of June 30, 2021, we had an accumulated deficit of $245.3 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 proprietary non-viral genetic medicine 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;

- build out our manufacturing facility and scale RES to produce clinical and initial commercial supply;
- 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, we expect to continue 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 our existing cash, cash equivalents and marketable securities, will enable us to fund our operating expenses and capital expenditures into 2024. 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.”

COVID-19

In March 2020, COVID-19 was declared a global pandemic by the World Health Organization and to date, the COVID-19 pandemic continues to present a substantial public health and economic challenge around the world. The length of time and full extent to which the COVID-19 pandemic may directly or indirectly impact our business, results of operations and financial condition will depend on future developments that are highly uncertain, subject to change and difficult to predict. We, our contract development and manufacturing organizations, or CDMOs, and our contract research organizations, or CROs, experienced temporary reductions in the capacity to undertake research-scale production and to execute some preclinical studies. While these operations have since normalized, we, together with our CDMOs and CROs, are closely monitoring the impact of the COVID-19 pandemic on these operations.

We also plan to continue to closely monitor the ongoing impact of the COVID-19 pandemic on our employees and our other business operations. In an effort to provide a safe work environment for our employees, we had, among other things, limited employees in our office and lab facilities to those where on-site presence is needed for their job activities, increased the cadence of sanitization of our office and lab facilities, implemented various social distancing measures in our offices and labs including replacing all in-person meetings with virtual interactions, and provided personal protective equipment for our employees present in our office and lab facilities. Currently, we continue to monitor the impact and effects of the COVID-19 pandemic and our response to it, and, in accordance with updated federal and state guidelines, we have relaxed some of our COVID-19 related restrictions and are permitting on-site presence for a limited number of additional

employees. We expect to continue to take actions as may be required or recommended by government authorities or as we determine are in the best interests of our employees and other business partners in light of the pandemic.

Components of Our Results of Operations

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 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 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 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 the submission and acceptance of IND applications 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;
- the availability of specialty raw materials for use in production of our product candidates;
- our ability to build out our manufacturing facility and scale RES to produce clinical and initial commercial supply;
- our ability to establish arrangements with third-party manufacturers for preclinical and 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 continue to 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

Interest income

Interest income consists of interest earned on our invested cash balances.

Results of Operations

Comparison of the three months ended June 30, 2021 and 2020

The following table summarizes our results of operations for the three months ended June 30, 2021 and 2020:

(in thousands)	Three Months Ended June 30, 2021	2020	Change
Operating expenses:			
Research and development	$ 22,656	$ 13,456	$ 9,200
General and administrative	8,186	4,308	3,878
Total operating expenses	30,842	17,764	13,078
Loss from operations	(30,842)	(17,764)	(13,078)
Other income:			
Interest income	51	33	18
Net loss	$ (30,791)	$ (17,731)	$ (13,060)

Research and development expenses

The following table summarizes our research and development expenses for the three months ended June 30, 2021 and 2020:

(in thousands)	Three Months Ended June 30, 2021	2020	Change
Preclinical and manufacturing	$ 7,809	$ 3,291	$ 4,518
Personnel-related	5,767	4,293	1,474
Facilities	2,520	2,308	212
Stock-based compensation	2,376	936	1,440
Lab supplies	1,805	572	1,233
Consulting and professional services	645	1,147	(502)
Other	1,734	909	825
Total research and development expenses	$ 22,656	$ 13,456	$ 9,200

Research and development expenses were $22.7 million for the three months ended June 30, 2021, compared to $13.5 million for the three months ended June 30, 2020. The increase in preclinical and manufacturing costs and lab supplies of $4.5 million and $1.2 million, respectively, was primarily due to increased preclinical activity as we continue our efforts to advance our two lead programs into IND-enabling studies. The increases of $1.5 million in personnel-related costs and $1.4 million in stock-based compensation costs were primarily due to increased headcount in our research and development function.

General and administrative expenses

The following table summarizes our general and administrative expenses for the three months ended June 30, 2021 and 2020:

(in thousands)	Three Months Ended June 30, 2021	2020	Change
Personnel-related	$ 3,254	$ 1,829	$ 1,425
Professional and consultant fees	2,183	1,528	655
Stock-based compensation	2,001	432	1,569
Facilities	236	372	(136)
Other	512	147	365
Total general and administrative expenses	$ 8,186	$ 4,308	$ 3,878

General and administrative expenses were $8.2 million for the three months ended June 30, 2021, compared to $4.3 million for the three months ended June 30, 2020. The increases in stock-based compensation costs and personnel-related costs of $1.6 million and $1.4 million, respectively, were primarily a result of an increase in headcount in our general and administrative function.

Other income

Other income for each of the three months ended June 30, 2021 and June 30, 2020 was $0.1 million. Other income consisted primarily of interest earned on invested cash balances.

Comparison of the six months ended June 30, 2021 and 2020

The following table summarizes our results of operations for the six months ended June 30, 2021 and 2020:

	Six Months Ended June 30,		
(in thousands)	**2021**	**2020**	**Change**
Operating expenses:			
Research and development	$ 41,409	$ 26,850	$ 14,559
General and administrative	15,088	8,950	6,138
Total operating expenses	56,497	35,800	20,697
Loss from operations	(56,497)	(35,800)	(20,697)
Other income:			
Interest income	144	352	(208)
Net loss	$ (56,353)	$ (35,448)	$ (20,905)

Research and development expenses

The following table summarizes our research and development expenses for the six months ended June 30, 2021 and 2020:

	Six Months Ended June 30,		
(in thousands)	**2021**	**2020**	**Change**
Preclinical and manufacturing	$ 13,638	$ 6,763	$ 6,875
Personnel-related	11,155	8,112	3,043
Facilities	4,740	4,744	(4)
Stock-based compensation	4,274	1,763	2,511
Lab supplies	3,435	1,850	1,585
Consulting and professional services	1,103	2,138	(1,035)
Other	3,064	1,480	1,584
Total research and development expenses	$ 41,409	$ 26,850	$ 14,559

Research and development expenses were $41.4 million for the six months ended June 30, 2021, compared to $26.9 million for the six months ended June 30, 2020. The increase in preclinical and manufacturing costs and lab supplies of $6.9 million and $1.6 million, respectively, was primarily due to increased preclinical activity as we continue our efforts to advance our two lead programs into IND-enabling studies. The increases of $3.0 million in personnel-related costs and $2.5 million in stock-based compensation costs were primarily due to increased headcount in our research and development function.

General and administrative expenses

The following table summarizes our general and administrative expenses for the six months ended June 30, 2021 and 2020:

(in thousands)	Six Months Ended June 30, 2021	2020	Change
Personnel related	$ 6,292	$ 3,395	$ 2,897
Stock-based compensation	3,582	1,109	2,473
Professional and consultant fees	3,599	3,388	211
Facilities	682	741	(59)
Other	933	317	616
Total general and administrative expenses	$ 15,088	$ 8,950	$ 6,138

General and administrative expenses were $15.1 million for the six months ended June 30, 2021, compared to $9.0 million for the six months ended June 30, 2020. The increases in personnel-related costs and stock-based compensation costs of $2.9 million and $2.5 million, respectively, were primarily a result of an increase in headcount in our general and administrative function.

Other income

Other income for the six months ended June 30, 2021 was $0.1 million compared to $0.4 million for the six months ended June 30, 2020. Other income consisted primarily of interest earned on invested cash balances. The decrease in interest income from the six months ended June 30, 2020 to the six months ended June 30, 2021 was primarily due to lower invested balances and lower interest rates during 2021.

Liquidity and Capital Resources

Since our inception, 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. We have not yet commercialized any product candidates and we do not expect to generate revenue from sales of any product candidates for several years, if at all. To date, we have funded our operations with proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), the sale of convertible preferred stock (which converted into common stock in 2020) and with proceeds from the sale of common stock in our public offerings. In June 2020, we completed our IPO, pursuant to which we issued and sold 12,105,263 shares of our common stock, including 1,578,947 shares sold by us pursuant to the full exercise of the underwriters' option to purchase additional shares. We received net proceeds of $210.7 million, after deducting underwriting discounts and commissions and other offering expenses. In January 2021, we issued and sold 9,200,000 shares of our common stock, including 1,200,000 shares sold by us pursuant to the full exercise of the underwriters' option to purchase additional shares, in a follow-on public offering, resulting in net proceeds of $211.3 million, after deducting underwriting discounts and commissions and other offering expenses. As of June 30, 2021, we had cash, cash equivalents and marketable securities of $425.2 million.

Cash flows

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

(in thousands)	Six Months Ended June 30, 2021	2020
Cash used in operating activities	$ (48,464)	$ (33,264)
Cash provided by (used in) investing activities	163,717	(131,622)
Cash provided by financing activities	213,930	321,446
Net increase in cash, cash equivalents and restricted cash	$ 329,183	$ 156,560

Operating activities

During the six months ended June 30, 2021, operating activities used $48.5 million of cash, primarily resulting from our net loss of $56.4 million and changes in our operating assets and liabilities of $2.6 million, both partially offset by non-cash charges of $10.5 million. Net cash used by changes in our operating assets and liabilities for the six months ended June 30, 2021 consisted primarily of a $2.1 million increase in prepaid expenses and other current assets and a $0.6 million increase in other noncurrent assets.

During the six months ended June 30, 2020, operating activities used $33.3 million of cash, primarily resulting from our net loss of $35.4 million and changes in our operating assets and liabilities of $2.2 million, both partially offset by non-cash charges of $4.4 million. Net cash used by changes in our operating assets and liabilities for the six months ended June 30, 2020 consisted primarily of a $2.5 million increase in prepaid expenses and other current assets and a $0.5 million decrease in deferred rent, partially offset by a decrease of $0.4 million in tenant receivable and an increase of $0.5 million in accounts payable and accrued expenses and other current liabilities.

Changes in accounts payable, accrued expenses and other current liabilities and prepaid expenses in the periods were generally due to growth in our business and the timing of vendor invoicing and payments. Changes in operating lease right-of-use-assets, operating lease liability, and deferred rent were primarily related to our adoption of the new lease accounting standard on January 1, 2021.

Investing activities

During the six months ended June 30, 2021, net cash provided by investing activities was $163.7 million, due to the maturities of marketable securities of $165.9 million, partially offset by a $2.2 million increase in purchases of property and equipment during the period. During the six months ended June 30, 2020, net cash used in investing activities was $131.6 million, due to the purchases of marketable securities and property and equipment during the period.

Property and equipment purchases during the six months ended June 30, 2021 and 2020 were primarily related to leasehold improvements and lab equipment for our facility in Cambridge, Massachusetts.

Financing activities

During the six months ended June 30, 2021, net cash provided by financing activities was $213.9 million, consisting primarily of proceeds from our follow-on public offering of common stock of $211.9 million, net of underwriting discounts and commissions, and proceeds of $2.6 million from the exercise of common stock options, partially offset by the payment of $0.6 million of public offering costs. During the six months ended June 30, 2020, net cash provided by financing activities was $321.4 million, consisting primarily of proceeds from our IPO of common stock of $213.9 million, net of discounts and commissions, and proceeds from the sale of our Series C preferred stock of $108.9 million, partially offset by the payment of $1.5 million of public offering costs.

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 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 clinical and commercial-scale manufacturing activities, including the build-out of our manufacturing facility;
- 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 genetic medicine 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 our existing cash, cash equivalents, and marketable securities will enable us to fund our operating expenses and capital expenditures into 2024. 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.

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 the "Risk Factors" section of this Quarterly Report and in our Annual Report for additional risks associated with our substantial capital requirements.

Critical Accounting Policies and Significant Judgments and Estimates

Our condensed consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States of America, or GAAP. The preparation of our condensed consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses and related disclosures and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. We base our estimates on historical experience, known trends and events and on various other factors that we believe to be 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 significantly from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in Note 2 to our condensed consolidated financial statements appearing elsewhere in this Quarterly Report, we believe that the accounting policies related to accrued research and development expenses and stock-based compensation are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements. There have been no material changes to our critical accounting policies and estimates from those disclosed in our financial statements and the related notes included in our Annual Report.

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 SEC.

Recently Issued and Adopted Accounting Pronouncements

A description of recently adopted and recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 2 to our condensed consolidated financial statements included in this Quarterly Report.

Item 3. Quantitative and Qualitative Disclosures about Market Risks.

We are a smaller reporting company, as defined in Rule 12b-2 under the Exchange Act, for this reporting period and are not required to provide the information required under this item.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, under the supervision and with the participation of our President and Chief Executive Officer and our Chief Financial Officer, our principal executive officer and principal financial and accounting officer, respectively, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2021. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and

procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of June 30, 2021, our President and Chief Executive Officer and our Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

During the six months ended June 30, 2021, we implemented certain internal controls as a result of our adoption of the new lease standard on January 1, 2021. There were no other changes in our internal control over financial reporting (as defined in Rules 13a–15(f) and 15d–15(f) under the Exchange Act) during the six months ended June 30, 2021 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1A. Risk Factors.

The following information updates, and should be read in conjunction with, the risk factors discussed in Part I, Item 1A Risk Factors in our Annual Report, which could materially affect our business, financial condition, or future results.

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 $56.4 million and $35.4 million for the six months ended June 30, 2021 and 2020, respectively. As of June 30, 2021, we had an accumulated deficit of $245.3 million. To date, we have funded our operations with the proceeds from instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), the sale of convertible preferred stock (which converted into common stock in 2020) and from the sale of common stock in our public offerings. 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 into preclinical and clinical development;
- expand the capabilities of our proprietary non-viral genetic medicine 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;
- build out and maintain a commercial-scale cGMP-compliant manufacturing facility;
- 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 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.

Risks relating to manufacturing

We intend to build out and operate our own manufacturing facility, which will require significant resources. If we fail to successfully build out our facility on a timely basis or at all or fail to successfully operate our facility, our business will be materially harmed.

In July 2021, we entered into a lease for approximately 104,000 square feet to develop a cGMP-compliant facility in Waltham, Massachusetts at which we intend to operate our own manufacturing facility. The facility requires substantial build-out and there can be no assurance that we will complete such build-out in a timely manner or at all, and the costs of doing so may be greater than we anticipate. Any commercial manufacturing facility we develop will also require FDA approval, which we may never obtain. Even if approved, we would be subject to ongoing periodic unannounced inspection by the FDA and corresponding state agencies to ensure strict compliance with cGMPs and other government regulations.

We also do not yet have sufficient information to reliably estimate the cost of the clinical and commercial manufacturing and processing of any product candidates we may develop at the new facility, and the actual cost to manufacture and process any product candidates we may develop could materially and adversely affect the commercial viability of such product candidates. In addition, the ultimate dose selected for clinical use and commercial supply will affect our ability to scale and our costs per dose. As a result, we may never be able to develop a commercially viable product.

We have limited experience in operating a manufacturing facility and managing the manufacturing process and it may be more difficult or more expensive than expected. Furthermore, we will need to hire additional personnel with such expertise. The manufacture of drugs and biologics is complex and requires significant expertise, including the development of advanced manufacturing techniques and process controls. Manufacturers of drugs and biologics often encounter difficulties in production, particularly in scaling and validating initial production and ensuring the absence of contamination. These difficulties may include those related to production costs and yields, quality control and quality assurance testing, stability of the product, operator error, shortages of qualified personnel, as well as difficulty in compliance with strictly enforced federal, state and foreign regulations. Additionally, we may not be able to achieve clinical or commercial manufacturing on our own to satisfy demands for any of our product candidates, if and when developed.

The application of any new regulatory guidelines or parameters may also adversely affect our ability to manufacture any product candidates we may develop. Furthermore, if contaminants are discovered in our supply of such product candidates or in the manufacturing facility, the facility may need to be closed for an extended period of time to investigate and remedy the contamination, which could delay clinical development of our programs and impair our ability to sell any product candidates we develop commercially. We cannot assure you that any stability or other issues relating to the manufacture of our product candidates will not occur in the future.

In connection with operating our own manufacturing facility, we will assume responsibility for storing and shipping any manufactured materials, and we may fail to manage the logistics of storing and shipping any product candidates we may develop. Storage failures and shipment delays and problems caused by us, our vendors or other factors not in our control, such as weather or global supply chain and shipping challenges, could result in loss of usable product or prevent or delay the delivery of such product candidates to patients. We may also experience manufacturing difficulties due to resource constraints and, as a result, our ability to provide any product candidates we may develop to patients could be jeopardized.

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 genetic medicines. 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 established a cGMP-ready cell (Sf9)-based process to produce ceDNA at the 200-liter scale that we have successfully transferred to a CDMO to supply research material. Additionally, we intend to build out and operate our own manufacturing facility to scale ceDNA manufacturing utilizing RES for additional research material as well as cGMP-compliant clinical and initial commercial supply. We plan to continue partnering with CDMOs during and after construction of our new manufacturing facility to ensure redundancy, secure additional ceDNA supply and provide materials needed for commercial supply.

A number of risk factors common to the manufacturing of biologics and drugs could also cause production issues or interruptions for our genetic medicines, 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, pandemics, 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 or scaling our manufacturing to sufficient levels 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 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 genetic medicine platform and RES are novel, and the combination of novel manufacturing process and constructs with development of the process at larger scale 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 genetic medicine platform and RES are each novel, and the manufacture of products on the basis of our platform and RES is untested at a large scale. Our preclinical studies to date have been completed using a different manufacturing process than RES and we may not achieve the same results when we transition to RES. 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 and the regulatory review and approval process may be more expensive or take longer than for other product candidates that may be produced using manufacturing processes with which such regulatory agencies are more familiar. 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.

Item 6. Exhibits.

Exhibit Number	Description of Exhibit
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) of the Securities Exchange Act, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) of the Securities Exchange Act, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1**	Certification of Principal Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2**	Certification of Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101)

* Filed herewith.

** Furnished herewith.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

GENERATION BIO CO.

Date: August 11, 2021

By: /s/ Geoff McDonough
Geoff McDonough, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

Date: August 11, 2021

By: /s/ Matthew Norkunas
Matthew Norkunas, M.D., M.B.A.
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Geoff McDonough, hereby certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Generation Bio Co.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 11, 2021

/s/ Geoff McDonough

Geoff McDonough, M.D.
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER
PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Matthew Norkunas, hereby certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Generation Bio Co.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

(a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

(b) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

(c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

(a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

(b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 11, 2021

/s/ Matthew Norkunas

Matthew Norkunas, M.D., M.B.A.
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION OF CHIEF EXECUTIVE OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Geoff McDonough, certify pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge, the Quarterly Report on Form 10-Q of Generation Bio Co. for the quarter ended June 30, 2021 fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and the information contained in such Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Generation Bio Co.

/s/ Geoff McDonough

Geoff McDonough, M.D.
President and Chief Executive Officer
(Principal Executive Officer)
August 11, 2021

Exhibit 32.2

CERTIFICATION OF CHIEF FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Matthew Norkunas, certify pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge, the Quarterly Report on Form 10-Q of Generation Bio Co. for the quarter ended June 30, 2021 fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and the information contained in such Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Generation Bio Co.

/s/ Matthew Norkunas

Matthew Norkunas, M.D., M.B.A.
Chief Financial Officer
(Principal Financial and Accounting Officer)
August 11, 2021