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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 September 30, 2023

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 November 3, 2023 there were 66,078,017 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 potential achievement of milestones and receipt of payments under our collaboration with ModernaTX, Inc.;
- our ability to enter into additional collaborations with third parties or obtain additional funding;
- our ability to find one or more third parties to assume our lease or sublease the property in Waltham, MA;
- 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 and obtain and maintain regulatory approvals for any product candidates we may develop;
- our estimates regarding the potential addressable patient populations for our programs;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our expectations regarding our ability to obtain and maintain intellectual property protection;
- our intellectual property position;
- our ability to identify additional products, product candidates or technologies with significant commercial potential that are consistent with our objectives;
- the impact of government laws and regulations;
- our competitive position and our expectations regarding developments and projections relating to our competitors and any competing therapies that are or become available; and
- developments and expectations relating to our competitors and our industry.

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)

	September 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 93,360	$ 93,171
Marketable securities	197,665	185,920
Tenant receivable	660	395
Prepaid expenses and other current assets	6,124	7,530
Total current assets	297,809	287,016
Property and equipment, net	22,376	22,215
Operating lease right-of-use assets	54,414	59,208
Restricted cash	5,692	5,692
Deferred offering costs	433	434
Other long-term assets	768	1,699
Total assets	$ 381,492	$ 376,264
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,650	$ 662
Accrued expenses and other current liabilities	9,474	11,402
Deferred revenue	11,974	—
Operating lease liability	8,721	7,086
Total current liabilities	32,819	19,150
Deferred revenue, net of current portion	45,766	—
Operating lease liability, net of current portion	70,993	74,621
Total liabilities	149,578	93,771
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 September 30, 2023 and December 31, 2022	—	—
Common stock, $0.0001 par value; 150,000,000 shares authorized at September 30, 2023 and December 31, 2022; 65,927,956 and 59,505,437 shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively	7	6
Additional paid-in capital	768,102	727,335
Accumulated other comprehensive loss	(4)	(83)
Accumulated deficit	(536,191)	(444,765)
Total stockholders' equity	231,914	282,493
Total liabilities and stockholders' equity	$ 381,492	$ 376,264

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 September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Revenues:				
Collaboration revenue	$ 2,146	$ —	$ 3,026	$ —
Operating expenses:				
Research and development	21,862	21,192	65,694	75,111
General and administrative	11,641	11,477	37,474	31,383
Total operating expenses	33,503	32,669	103,168	106,494
Loss from operations	(31,357)	(32,669)	(100,142)	(106,494)
Other income:				
Other income and interest income, net	3,091	1,363	8,716	2,260
Net loss and net loss attributable to common stockholders	$ (28,266)	$ (31,306)	$ (91,426)	$ (104,234)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.43)	$ (0.53)	$ (1.43)	$ (1.81)
Weighted average common shares outstanding, basic and diluted	65,907,000	58,872,334	63,951,508	57,679,635
Comprehensive loss:				
Net loss	$ (28,266)	$ (31,306)	$ (91,426)	$ (104,234)
Other comprehensive loss:				
Unrealized gains (losses) on marketable securities	19	37	79	(302)
Comprehensive loss	$ (28,247)	$ (31,269)	$ (91,347)	$ (104,536)

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

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

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	Three Months Ended September 30, 2023					
Balances at June 30, 2023	65,784,250	$ 7	$ 762,228	$ (23)	$ (507,925)	$ 254,287
Vesting of restricted common stock	143,706	—	(130)	—	—	(130)
Stock-based compensation expense	—	—	6,004	—	—	6,004
Unrealized gains on marketable securities	—	—	—	19	—	19
Net loss	—	—	—	—	(28,266)	(28,266)
Balances at September 30, 2023	65,927,956	$ 7	$ 768,102	$ (4)	$ (536,191)	$ 231,914

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	Three Months Ended September 30, 2022					
Balances at June 30, 2022	57,597,141	$ 6	$ 706,261	$ (339)	$ (381,054)	$ 324,874
Issuance of common stock from public ATM offering, net of commissions and offering costs of $315	1,379,887	—	9,617	—	—	9,617
Issuance of common stock upon exercise of stock options	15,026	—	58	—	—	58
Vesting of restricted common stock	280,404	—	(394)	—	—	(394)
Issuance of common stock under other equity plans	2,711		15	—	—	15
Stock-based compensation expense	—	—	5,848	—	—	5,848
Unrealized gains on marketable securities	—	—	—	37	—	37
Net loss	—	—	—	—	(31,306)	(31,306)
Balances at September 30, 2022	59,275,169	$ 6	$ 721,405	$ (302)	$ (412,360)	$ 308,749

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

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

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	Nine Months Ended September 30, 2023					
Balances at December 31, 2022	59,505,437	$ 6	$ 727,335	$ (83)	$ (444,765)	$ 282,493
Sale of common stock in connection with the Moderna Share Purchase Agreement	5,859,375	1	22,555	—	—	22,556
Vesting of restricted common stock	455,463	—	(448)	—	—	(448)
Issuance of common stock under other equity plans	107,681	—	367	—	—	367
Stock-based compensation expense	—	—	18,293	—	—	18,293
Unrealized gains on marketable securities	—	—	—	79	—	79
Net loss	—	—	—	—	(91,426)	(91,426)
Balances at September 30, 2023	65,927,956	$ 7	$ 768,102	$ (4)	$ (536,191)	$ 231,914

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
	Nine Months Ended September 30, 2022					
Balances at December 31, 2021	56,969,618	$ 6	$ 689,866	$ —	$ (308,126)	$ 381,746
Issuance of common stock from public ATM offering, net of commissions and offering costs of $404	1,795,524	—	12,338	—	—	12,338
Issuance of common stock upon exercise of stock options	147,400	—	611	—	—	611
Vesting of restricted common stock	294,767	—	(394)	—	—	(394)
Issuance of common stock under other equity plans	67,860	—	379	—	—	379
Stock-based compensation expense	—	—	18,605	—	—	18,605
Unrealized losses on marketable securities	—	—	—	(302)	—	(302)
Net loss	—	—	—	—	(104,234)	(104,234)
Balances at September 30, 2022	59,275,169	$ 6	$ 721,405	$ (302)	$ (412,360)	$ 308,749

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)

	Nine Months Ended September 30,	
	2023	2022
Cash flows from operating activities:		
Net loss	$ (91,426)	$ (104,234)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	18,293	18,605
Depreciation and amortization expense	3,938	3,784
Amortization (accretion) of premium (discount) on marketable securities, net	(6,699)	(984)
Loss on sale of property and equipment	24	28
Loss on impairment of property and equipment	—	5,395
Changes in operating assets and liabilities:		
Tenant receivable	(265)	(395)
Prepaid expenses and other current assets	1,408	(4,939)
Operating lease right-of-use assets	4,794	4,017
Other noncurrent assets	931	(1,151)
Accounts payable	1,386	(444)
Accrued expenses and other current liabilities	(2,633)	(1,663)
Deferred revenue	44,474	—
Operating lease liability	(1,992)	2,536
Net cash used in operating activities	(27,767)	(79,445)
Cash flows from investing activities:		
Purchases of property and equipment	(2,817)	(8,119)
Purchases of marketable securities	(295,967)	(221,000)
Maturities of marketable securities	291,000	55,000
Net cash used in investing activities	(7,784)	(174,119)
Cash flows from financing activities:		
Payment of share issuance costs	(179)	(50)
Proceeds from sale of common stock in connection with the Moderna Share Purchase Agreement	36,000	—
Proceeds from issuance of common stock from public ATM offering, net of commissions and offering costs	—	12,360
Proceeds from exercise of stock options and other types of equity, net	367	990
Tax withholding payments related to net share settlements of restricted stock units	(448)	(394)
Net cash provided by financing activities	35,740	12,906
Net increase (decrease) in cash, cash equivalents and restricted cash	189	(240,658)
Cash, cash equivalents and restricted cash at beginning of period	98,863	380,837
Cash, cash equivalents and restricted cash at end of period	$ 99,052	$ 140,179
Supplemental disclosure of noncash investing and financing information:		
Purchases of property and equipment included in accounts payable and accrued expenses	$ 1,330	$ 165
Unrealized gains (losses) on marketable securities	$ 79	$ (302)

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

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 Co. 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 novel immune-quiet DNA, or iqDNA; our unique 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 iqDNA. iqDNA is an optimized variant of our closed-ended DNA, or ceDNA, that has displayed a superior tolerability profile compared to previous ceDNA constructs and that we have chosen as our cargo for our lead program in hemophilia A as well as for all other programs. 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 cell types 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, 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.

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 sale of instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), sales of convertible preferred stock (which converted into common stock in 2020), and sales of common stock in underwritten public offerings, "at-the-market" offerings, and in a private placement, as well as payments pursuant to our collaboration with ModernaTX, Inc., or Moderna. We have incurred recurring losses, including net losses of $91.4 million for the nine months ended September 30, 2023 and $104.2 million for the nine months ended September 30, 2022. As of September 30, 2023, we had an accumulated deficit of $536.2 million. We expect to continue to generate operating losses in the foreseeable future. As of November 9, 2023, 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 additional 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 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, 2022 was derived from audited financial statements but does not include all disclosures required by GAAP. The accompanying unaudited financial statements as of September 30, 2023 and for the three and nine months ended September 30, 2023 and 2022 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 September 30, 2023, the results of operations for the three and nine months ended September 30, 2023 and 2022, and cash flows for the nine months ended September 30, 2023 and 2022 have been made. The results of operations for the three and nine months ended September 30, 2023 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2023 or any other period.

Our significant accounting policies are described in Note 2 of the Notes to Consolidated Financial Statements included in our Annual Report on Form 10-K that was most recently filed with the SEC.

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 national depository institutions in which our cash, cash equivalents, and marketable securities are held. We maintain our cash equivalents in money market funds that invest in U.S. treasury securities. We have adopted an investment policy that limits the amounts that we may invest in the securities of a single issuer with the exclusion of the U.S. government. We have not experienced any credit losses.

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.

Revenue Recognition

We enter into collaboration agreements that are within the scope of ASC Topic 606, “Revenue from Contracts with Customers”, or ASC 606, under which we license rights to certain of our potential product candidates and perform research and development services. The terms of these contracts typically include payment of the following: non-refundable, upfront fees; reimbursement of research and development costs; development, regulatory, and commercial milestone payments; royalties on net sales of licensed products, and a premium or discount on the sale of our common stock.

Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration that the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for contracts determined to be within the scope of ASC 606, we perform the following five steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when, or as, we satisfy each performance obligation. We only apply the five-step model to contracts when it is probable that we will collect consideration we are entitled to in exchange for the goods or services we transfer to the customer.

The promised goods or services in our arrangements typically consist of license rights to our intellectual property and research and development services. We provide options to additional items in the contract, which will be accounted for as separate contracts if and when the other party elects to exercise such options, unless the option provides a material right to such party. We evaluate the other party's options for material rights, or options to acquire additional goods or services for free or at a discount. If the other party's options are determined to represent a material right, the material right is recognized as a separate performance obligation at the outset of the contract. Performance obligations are promised goods or services in a contract to transfer a distinct good or service to the other party and are considered distinct when (i) the other party can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised goods or services are distinct, we consider factors such as the stage of development of the underlying intellectual property, the capabilities of the other party to develop the intellectual property on its own or whether the required expertise is readily available and whether the goods or services are integral or dependent to other goods or services in the contract.

We estimate the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration. At the inception of each contract that includes variable consideration, we evaluate the number of potential payments and the likelihood that the payments will be received. We utilize either the most likely amount method or expected amount method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration that is included in the transaction price may be constrained and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period.

Our contracts include development, regulatory, and commercial milestone payments that will be assessed under the most likely amount method and constrained if it is probable that a significant revenue reversal would occur. Milestone payments that are not within our control or the counterparty's control, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. At the end of each reporting period, we re-evaluate the probability of achievement of such development, regulatory, and commercial milestones and any related constraint, and if necessary, we adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenues in the period of adjustment. To date, we have not recognized any consideration related to the achievement of development, regulatory, or commercial milestone revenue resulting from our collaboration contracts.

For contracts that include sales-based royalties, including milestone payments based on the level of sales, and where the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any consideration related to sales-based royalty revenue resulting from our collaboration contract.

We allocate the transaction price based on the estimated stand-alone selling price of each of the performance obligations. We must develop assumptions that require judgment to determine the stand-alone selling price for each performance obligation identified in the contract. We utilize key assumptions to determine the stand-alone selling price for service obligations, which may include other comparable transactions, pricing considered in negotiating the transaction and the estimated costs. Additionally, in determining the standalone selling price for material rights, we utilize comparable transactions, clinical trial success probabilities, and estimates of option exercise likelihood. Variable consideration is

allocated specifically to one or more performance obligations in a contract when the terms of the variable consideration relate to the satisfaction of the performance obligation and the resulting amounts allocated are consistent with the amounts we would expect to receive for the satisfaction of each performance obligation.

The consideration allocated to each performance obligation is recognized as revenue when control is transferred for the related goods or services. For performance obligations that consist of licenses and other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition.

Upfront payments and fees are recorded as deferred revenue upon receipt or when due until we perform our obligations. Amounts are recorded as accounts receivable when our right to consideration is unconditional.

3. Marketable Securities and Fair Value Measurements

The following tables present our marketable securities by security type:

	As of September 30, 2023			
(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury securities	$ 197,669	$ 8	$ (12)	$ 197,665

	As of December 31, 2022			
(in thousands)	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury securities	$ 186,003	$ 13	$ (96)	$ 185,920

As of September 30, 2023 and December 31, 2022, our marketable securities consisted of investments that mature within one year of their purchase date.

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 September 30, 2023 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 35,109	$ —	$ —	$ 35,109
Marketable securities:				
U.S. treasury securities	—	197,665	—	197,665
Totals	$ 35,109	$ 197,665	$ —	$ 232,774

	Fair Value Measurements at December 31, 2022 Using:			
(in thousands)	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 77,010	$ —	$ —	$ 77,010
Marketable securities:				
U.S. treasury securities	—	185,920	—	185,920
Totals	$ 77,010	$ 185,920	$ —	$ 262,930

4. Collaboration and License Agreement

Moderna Collaboration and License Agreement

In March 2023, we entered into a Collaboration and License Agreement, or the Collaboration Agreement, with Moderna to collaborate on developing treatments for certain diseases by targeting delivery of nucleic acids to liver cells and certain cells outside of the liver.

Under the Collaboration Agreement, the parties have agreed to collaborate on preclinical research programs relating to lipid nanoparticle, or LNP, delivery systems and nucleic acid payloads, with each party obtaining certain rights to intellectual property used in and arising out of such research programs. Each party will be solely responsible for its own clinical development and commercialization of products under the Collaboration Agreement. Moderna will reimburse us for the internal and external costs incurred by us in conducting the research programs, to the extent consistent with such research plans and budgets.

Moderna has exclusive options, upon payment of option exercise fees, to obtain worldwide, exclusive, sublicensable licenses under specified company intellectual property to develop, manufacture and commercialize (a) products comprising LNP delivery systems and nucleic acid payloads that are directed to (i) up to two liver targets, (ii) up to two non-liver targets and (iii) a third liver or non-liver target and (b) Exclusive Targets, which are Independent Program Products (as defined below) that include messenger RNA, or mRNA, that are directed to gene and protein targets in any of certain agreed-upon immune cell types, referred to as the Cell Target Types. Subject to the exclusivity obligations described below, each party has granted to the other a worldwide, non-exclusive, sublicensable license under certain LNP-related intellectual property arising out of the non-liver ctLNP program, or the Joint Collaboration ctLNP Intellectual Property, to develop, manufacture and commercialize products comprising LNP delivery systems and nucleic acid payloads directed to gene and protein targets in any of the Cell Target Types, or Independent Program Products.

Each party is obligated to use commercially reasonable efforts to complete the activities assigned to it under the research plans, and Moderna is further obligated to use commercially reasonable efforts to develop, seek regulatory approval for and commercialize at least one product directed to each target for which Moderna exercises its exclusive license option in at least one indication in the United States and in specified European countries.

We have agreed not to, directly or indirectly, alone or with, for or through any third party, develop, manufacture, commercialize or exploit (a) products containing mRNA that are directed to any of the Cell Target Types, during an agreed-upon exclusivity period, which may be extended by payment of extension fees, (b) products directed to any liver target or non-liver target during the option periods for those targets, (c) products directed to any liver target or non-liver target for which Moderna has exercised its exclusive license option or (d) products containing mRNA that are directed to any Exclusive Target for which Moderna has exercised its exclusive license option.

Under the terms of the Collaboration Agreement, in April 2023, Moderna made an upfront payment to us of $40.0 million, and paid us $7.5 million in prepaid research funding. In addition, we are eligible to receive up to $1.8 billion in milestone payments upon the achievement of specified development, regulatory, commercial, and sales milestone events, research term extension fees and exclusivity extension fees. Subject to reductions in specified circumstances, we will also be entitled to receive tiered royalties: (i) ranging from high-single-digits to low-double-digits on sales of licensed products that are directed to any liver target or non-liver target with respect to which Moderna has exercised its exclusive license option, and (ii) in the single digits on sales of Independent Program Products, including the exclusively licensed Independent Program Products directed to the Exclusive Targets. In consideration for the non-exclusive license granted by Moderna to us under the Joint Collaboration ctLNP Intellectual Property, we have agreed to pay Moderna tiered royalties in the single digits on sales of Independent Program Products that include mRNA, subject to reductions in specified circumstances. Royalties will be paid by each party, on a licensed product-by-licensed product and country-by-country basis, until the latest to occur of: (i) expiration of the last-to-expire of specified licensed patent rights; (ii) expiration of regulatory exclusivity; or (iii) ten years after the first commercial sale of the applicable licensed product.

In addition, in connection with the execution of the Collaboration Agreement, we entered into a Share Purchase Agreement, or the Share Purchase Agreement, with Moderna, pursuant to which we issued and sold 5,859,375 shares of

our common stock to Moderna, at a price of $6.14 per share, for an aggregate purchase price of $36.0 million, which closed concurrently with the execution of the Collaboration Agreement. Under the Share Purchase Agreement, Moderna has the right, subject to certain terms and conditions, to purchase up to 3.06% of the outstanding shares of our common stock (on a post-closing basis) in connection with a future equity financing of at least $25.0 million by us.

Moderna Agreement Assessment

We assessed the promised goods and services under the Collaboration Agreement, in accordance with ASC 606. At inception, the Collaboration Agreement included one combined performance obligation, which includes the license to the ctLNP technology to target indications outside of the liver and the related research services to develop such technology, as the two items are not distinct in context of the contract. The Collaboration Agreement also provides Moderna with options to receive additional research services and options to receive exclusive licenses. The options to receive exclusive licenses allow Moderna to develop and commercialize product candidates that utilize our ctLNP and ceDNA technology for targets within the liver, as well as utilizing the ctLNP technology to be developed as part of the Collaboration Agreement and our ceDNA technology for targets outside the liver. These options are considered to be a priced at a discount to its standalone selling price and therefore are considered to be material rights.

The initial transaction price included a $40.0 million upfront fee, premium paid over the fair value of the common stock of $13.3 million in connection with shares issued and sold to Moderna under the Share Purchase Agreement, and estimated revenue associated with the payment for research services, including $7.5 million in prepaid research services. We utilized the expected amount method to determine the amount of reimbursement for these activities. We utilized the most likely amount method to determine the amount of consideration to include in the transaction price related to any variable consideration related to exclusivity fees, and milestones, and the royalty payments are constrained based on the royalty constraint. No amounts are included in the transaction price related to these elements.

We initially allocated the transaction price to each unit of account as follows:

Performance Obligations (in thousands)	Standalone Selling Price	Transaction Price Allocated
ctLNP technology and research license	$ 52,500	$ 42,576
First liver program commercialization option license	7,000	5,677
Second liver program commercialization option license	7,000	5,677
First non-liver program commercialization option license	11,700	9,488
Second non-liver program commercialization option license	11,700	9,488
Third liver or non-liver program commercialization option license	6,150	4,987
Total	$ 96,050	$ 77,893

The transaction price was allocated to each unit of account based on the relative estimated standalone selling prices, over which management has applied significant judgment, of each element. We developed the estimated standalone selling price for combined performance obligation and each of the options to receive licenses primarily based on the probability-weighted present value of expected future cash flows associated with each license related to each specific program and an estimate of the costs to provide services including a reasonable return. In developing such estimate, we also considered applicable market conditions and relevant entity-specific factors, including those factors contemplated in negotiating the agreement, the probability of success and the time needed to commercialize a product candidate pursuant to the associated license.

On a quarterly basis, we measure proportional performance of the combined performance obligation over time using an input method based on cost incurred relative to the total estimated costs by determining the proportion of effort incurred as a percentage of total effort we expect to expend. This ratio is then applied to the transaction price allocated to the combined performance obligation and each of the options to receive licenses. Any changes to these estimates will be recognized in the period in which they change as a cumulative catch up. All allocated consideration for the material rights is deferred until such time that Moderna exercises its options or the right to exercise the options expires. Upon exercise,

we will determine the appropriate revenue recognition methodology and any other implications on the accounting treatment for the arrangement.

The following table provides a summary of the transaction price allocated to each unit of account, in addition to revenue activity during the period:

Performance Obligations	Transaction Price Allocated	Revenue Recognized During		Deferred Revenue
(in thousands)	As of September 30, 2023	Three Months Ended September 30, 2023	Nine Months Ended September 30, 2023	As of September 30, 2023
ctLNP technology and research license	$ 40,979	$ 2,146	$ 3,026	$ 30,188
First liver program commercialization option license	5,464	—	—	4,429
Second liver program commercialization option license	5,464	—	—	4,429
First non-liver program commercialization option license	9,132	—	—	7,402
Second non-liver program commercialization option license	9,132	—	—	7,402
Third liver or non-liver program commercialization option license	4,799	—	—	3,890
Total	$ 74,970	$ 2,146	$ 3,026	$ 57,740

5. Property and equipment, net

Property and equipment, net consisted of the following:

(in thousands)	September 30, 2023	December 31, 2022
Laboratory equipment	$ 14,317	$ 13,619
Computer equipment and software	1,356	1,189
Furniture and fixtures	1,256	1,146
Leasehold improvements	20,854	20,786
Construction in progress	3,027	13
	40,810	36,753
Less: Accumulated depreciation and amortization	(18,434)	(14,538)
Total	$ 22,376	$ 22,215

Depreciation and amortization expense for the three and nine months ended September 30, 2023 was $1.3 million and $3.9 million, respectively. Depreciation and amortization expense for the three and nine months ended September 30, 2022 was $1.4 million and $3.8 million, respectively.

In July 2021, we entered into a lease agreement to build out a current Good Manufacturing Practice-, or cGMP-, compliant manufacturing facility in Waltham, Massachusetts in order to scale ceDNA manufacturing utilizing RES for clinical and initial commercial supply. We decided to transition from building out the cGMP compliant manufacturing facility to utilizing an external cleanroom facility after achieving increased scalability of the RES development process in the second half of 2022. Consequently, we are seeking one or more third parties to assume our lease or sublease the property. The balance of construction in progress at September 30, 2023, was comprised primarily of the capitalization of construction costs to renovate the property for alternative use.

6. Accrued Expenses

Accrued expenses and other current liabilities consisted of the following:

(in thousands)	September 30, 2023	December 31, 2022
Accrued employee compensation and benefits	$ 5,809	$ 7,970
Accrued external research and development expenses	1,786	1,959
Accrued professional fees	815	1,047
Property and equipment	694	—
Other	370	426
Total	$ 9,474	$ 11,402

7. Equity

As of September 30, 2023, 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 August 2021, we entered into an “at-the-market” sales agreement pursuant to which we may, from time to time, sell shares of our common stock having an aggregate offering price of up to $250.0 million. As of November 9, 2023, the issuance date of these condensed consolidated financial statements, we have issued and sold 1,795,524 shares of our common stock pursuant to this sales agreement resulting in net proceeds of $12.3 million.

In March 2023, in connection with the Share Purchase Agreement with Moderna, we issued and sold 5,859,375 shares of our common stock to Moderna at a price of $6.14 per share for an aggregate purchase price of $36.0 million. For additional information, refer to Note 4, Collaboration and License Agreements.

Each share of common stock entitles the holder to one vote on all matters submitted to a vote of our stockholders. Holders of common stock 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 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, 2022 and 2023, 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, from 12,154,517 shares to 14,433,745 shares, and from 14,433,745 shares to 16,813,962 shares, respectively. 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 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 our IPO, fair value of common stock is based on quoted market prices.

As of September 30, 2023, 1,457,015 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 nine months ended September 30, 2023, we granted time-based options to certain employees for the purchase of an aggregate of 1,765,301 shares of common stock with a weighted average grant date fair value of $3.67 per share that vest over a weighted average period of approximately four years.

Restricted stock units

During the nine months ended September 30, 2023, we issued 848,530 restricted stock units with a fair value of $3.9 million 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 authorized for issuance under the 2020 ESPP automatically increases on the first day of each fiscal year, beginning with the fiscal year that commenced 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, 2022, and 2023, the number of shares of common stock authorized for issuance under the 2020 ESPP was increased from 481,231 shares to 950,931 shares, from 950,931 shares to 1,520,738 shares, and from 1,520,738 shares to 2,115,792 shares, respectively. As of September 30, 2023, 1,826,149 shares remained available for issuance under the 2020 ESPP.

Stock-based compensation

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(in thousands)	2023	2022	2023	2022
Research and development expenses	$ 2,874	$ 3,046	$ 8,608	$ 9,674
General and administrative expenses	3,130	2,802	9,685	8,931
Total	$ 6,004	$ 5,848	$ 18,293	$ 18,605

As of September 30, 2023, total unrecognized compensation cost related to unvested time-based stock options and restricted stock units was $31.7 million, with $27.1 million expected to be recognized over a weighted average period of 2.2 years and $4.6 million expected to be recognized over a weighted average period of 2.4 years, respectively.

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 contribute a portion of their annual compensation on a pre-tax and/or after-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 the 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 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 nine months ended September 30, 2023.

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:

	September 30,	
	2023	2022
Unvested restricted stock units	1,179,349	1,170,433
Stock options to purchase common stock	9,676,685	8,786,200
Total	10,856,034	9,956,633

11. Related Parties

In March 2023, we entered into the Collaboration Agreement with Moderna. In connection with the Share Purchase Agreement, we issued and sold 5,859,375 shares of our common stock to Moderna, which resulted in Moderna becoming the beneficial owner of 8.9% of our outstanding common stock and a related party.

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 is meant to provide material information relevant to an assessment of the financial condition and results of operations of our company, including an evaluation of the amounts and uncertainties of cash flows from operations and from outside resources, so as to allow investors to better view our company from management's perspective. It 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, in our Annual Report and in the other documents filed with the SEC, 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 novel immune-quiet DNA, or iqDNA; our unique 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 iqDNA. iqDNA is an optimized variant of our closed-ended DNA, or ceDNA, that has displayed a superior tolerability profile compared to previous ceDNA constructs and that we have chosen as our cargo for our lead program in hemophilia A as well as for all other programs. 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 cell types 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.

We are advancing a broad portfolio of programs, including programs for rare and prevalent diseases of the liver. 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 that result from mutations in a single gene, and which can be treated by delivering our iqDNA cargo to cells of the liver, called hepatocytes. We are focusing on indications like hemophilia A, which is our lead program, that have well-established biomarkers and clear clinical and regulatory pathways. We are at the preclinical research stage and are currently optimizing our ctLNP platform to improve hepatocyte delivery to treat hemophilia A and other rare monogenic diseases. Our ctLNP platform is comprised of three components: a base stealth LNP that has demonstrated the ability to avoid off-target clearance by the liver and spleen, bioconjugation chemistry that attaches biological targeting ligands to the stealth LNP, and biological targeting ligands that are designed to drive highly selective delivery of the ctLNP to the target cell or tissue type. We plan to expand our portfolio to include programs based on cell-targeted delivery of iqDNA to immune cells, tumors, retina, skeletal muscle, and to the central nervous system by developing discrete ctLNPs, each with a discrete targeting ligand engineered to reach a specific target cell type or tissue.

In addition, we believe that our non-viral genetic medicine platform may be used to develop therapies that deliver antibody genes to direct the liver to produce antibody therapies from patients' own cells for years at a time from a single dose in a process we refer to as endogenous therapeutic antibody production, or ETAP. We plan to advance ETAP programs across multiple therapeutic areas, including prevalent diseases.

In July 2021, we entered into a lease agreement to build out a current Good Manufacturing Practice-, or cGMP-, compliant manufacturing facility, or the Seyon Facility, in Waltham, Massachusetts in order to scale ceDNA manufacturing utilizing RES for clinical and initial commercial supply. We decided to transition from building out the cGMP compliant manufacturing facility to utilizing an external cleanroom facility after achieving increased scalability of the RES development process in the second half of 2022. We have entered into an agreement with an external cleanroom facility at which we expect to manufacture cGMP-compliant clinical and initial commercial supply of iqDNA using RES that will

allow us to retain control over personnel, quality, infrastructure and process. Additionally, we may enter into agreements with contract manufacturing organizations, or CMOs, to provide further manufacturing capacity.

In March 2023, we entered into a Collaboration and License Agreement, or the Collaboration Agreement, with ModernaTX, Inc., or Moderna, to collaborate on developing treatments for certain diseases by targeting delivery of nucleic acids to liver cells and certain cells outside of the liver. Under the Collaboration Agreement, the parties have agreed to collaborate on preclinical research programs relating to lipid nanoparticle, or LNP, delivery systems and nucleic acid payloads, with each party obtaining certain rights to intellectual property used in and arising out of such research programs.

The research programs will be conducted pursuant to research plans and associated research budgets established by governance committees formed by the parties. Moderna will reimburse us for the internal and external costs we incur in conducting the research programs, to the extent consistent with such research plans and budgets. Each party will be solely responsible for its own clinical development and commercialization of products under the Collaboration Agreement.

In addition, Moderna has exclusive options, upon payment of option exercise fees, to obtain worldwide, exclusive, sublicensable licenses under certain of our specified intellectual property to develop, manufacture and commercialize (a) products comprising LNP delivery systems and nucleic acid payloads that are directed to (i) up to two liver targets, (ii) up to two agreed-upon non-liver targets and (iii) a third liver or non-liver target and (b) Independent Program Products, which are products comprising LNP delivery systems that include mRNA that are directed to gene and protein targets in any of the agreed-upon immune cell types, or Cell Targets Types.

Under the terms of the Collaboration Agreement, in April 2023, Moderna made an upfront payment to us of $40.0 million, and paid us $7.5 million in prepaid research funding. In addition, we are eligible to receive up to $1.8 billion in milestone payments upon the achievement of specified development, regulatory, commercial, and sales milestone events, research term extension fees and exclusivity extension fees. Subject to reduction in specified circumstances, we will also be entitled to receive tiered royalties: (i) ranging from high-single-digits to low-double-digits on sales of licensed products that are directed to the liver targets and non-liver targets with respect to which Moderna has exercised its exclusive license options, and (ii) in the single digits on sales of Independent Program Products, including the exclusively licensed Independent Program Products. In consideration for the non-exclusive license granted by Moderna to us under the LNP-related intellectual property arising out of the research program focused on the discovery and development of ctLNPs directed to agreed-upon immune cell types, we have agreed to pay Moderna tiered royalties ranging from low-single-digits to mid-single-digits on sales of Independent Program Products that include mRNA, subject to reductions in specified circumstances.

In connection with the Collaboration Agreement, we entered into a Share Purchase Agreement with Moderna, pursuant to which we issued and sold 5,859,375 shares of our common stock to Moderna, at a price of $6.14 per share, for an aggregate purchase price of $36.0 million. In addition, under the Share Purchase Agreement, Moderna has the right, subject to certain terms and conditions, to purchase up to 3.06% of the outstanding shares of our common stock (on a post-closing basis) in connection with a future equity financing of at least $25.0 million by us. For additional information on our collaboration with Moderna and the accounting thereunder, refer to Note 4, Collaboration and License Agreements.

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. We expect that any revenue recognized for the next several years will be derived primarily from our current collaboration with Moderna and any additional collaborations that we may enter into in the future. Historically, we have funded our operations with proceeds from the sale of instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), sales of convertible preferred stock (which converted into common stock in 2020), and sales of common stock in underwritten public offerings, “at-the-market” offerings, and in a private placement, as well as payments pursuant to our collaboration with Moderna. 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. In August 2021, we entered into an "at-the-market" sales agreement pursuant to which we may, from time to time, sell shares of our common stock having an aggregate offering price of up to $250.0 million. As of November 9, 2023, we have issued and sold 1,795,524 shares of our common stock pursuant to this sales agreement resulting in net proceeds of $12.3 million. In March 2023, in connection with the Share Purchase Agreement entered into with Moderna, we issued and sold 5,859,375 shares of our common stock to Moderna at a price of $6.14 per share for an aggregate purchase price of $36.0 million.

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 nine months ended September 30, 2023 and 2022, we reported net losses of $91.4 million and $104.2 million, respectively. As of September 30, 2023, we had an accumulated deficit of $536.2 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:

- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- continue our current research programs and conduct additional research programs, including pursuant to our collaboration with Moderna;
- expand the capabilities of our proprietary non-viral genetic medicine platform;
- 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;
- establish additional manufacturing sources and secure supply chain capacity sufficient to provide necessary quantities of any product candidates we may develop for clinical or commercial use;
- hire additional clinical, regulatory and scientific personnel;
- advance any product candidates we identify into preclinical and clinical development;
- seek marketing approvals for any product candidates that successfully complete clinical trials; and
- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval.

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, including our collaboration with Moderna. 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 2026. We have based our estimates as to how long we expect we will be able to fund our operations on assumptions that may prove to be wrong. We could use our available capital resources sooner than we currently expect, in which case we would be required to obtain additional financing, which may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. See "—Liquidity and Capital Resources."

Components of Our Results of Operations

Collaboration revenue

Our revenue consists of collaboration revenue, including amounts recognized for payments for licenses, research funding and milestone payments earned under our collaboration and license agreements.

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, contractors and CROs, and regulatory agency fees;
- the cost of developing and scaling our manufacturing process and capabilities and manufacturing drug substance and drug product for use in our research and preclinical studies, including under agreements with third parties, such as consultants, contractors and contract development organizations, or CDOs;
- 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, CDOs and CROs in connection with our research, preclinical and manufacturing activities. We do not allocate our research and development costs to specific programs because costs are deployed across multiple programs and our platform and, as such, are not separately classified. We expect that our research and development expenses will increase substantially as we advance our programs into clinical development and expand our discovery, research and preclinical activities in the near term and in the future. At this time, we cannot accurately estimate or know the nature, timing and

costs of the efforts that will be necessary to complete the preclinical and clinical development of any product candidates we may develop. The successful development of any of our product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with product development, including the following:

- the timing and progress of preclinical studies, including investigational new drug, or IND , -enabling studies;
- the number and scope of preclinical and clinical programs we decide to pursue;
- raising additional funds necessary to complete preclinical and clinical development of our product candidates;
- the timing of 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 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;
- our ability to scale RES to produce clinical and initial commercial supply;
- our ability to establish arrangements with third-party manufacturers for preclinical and clinical supply;
- the availability of specialty raw materials for use in production of our product candidates;
- 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 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;
- our ability to maintain a continued acceptable safety, tolerability and efficacy profile of our product candidates following approval; and
- the terms and timing of any existing or future collaboration, license or other arrangement, including the terms and timing of any achievement of milestones and the receipt of payments thereunder.

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 substantial 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 and interest income, net

Other income and interest income, net consists of interest income earned on our invested cash balances and other miscellaneous income unrelated to our core operations.

Results of Operations

Comparison of the three and nine months ended September 30, 2023 and 2022

The following table summarizes our results of operations for the three and nine months ended September 30, 2023 and 2022:

(in thousands)	Three Months Ended September 30, 2023	2022	Change 2023 vs 2022	Nine Months Ended June 30, 2023	2022	Change 2023 vs 2022
Revenue:						
Collaboration revenue	$ 2,146	$ —	$ 2,146	$ 3,026	$ —	$ 3,026
Operating expenses:						
Research and development	21,862	21,192	670	65,694	75,111	(9,417)
General and administrative	11,641	11,477	164	37,474	31,383	6,091
Total operating expenses	33,503	32,669	834	103,168	106,494	(3,326)
Loss from operations	(31,357)	(32,669)	1,312	(100,142)	(106,494)	6,352
Other income:						
Other income and interest income, net	3,091	1,363	1,728	8,716	2,260	6,456
Net loss	$ (28,266)	$ (31,306)	$ 3,040	$ (91,426)	$ (104,234)	$ 12,808

Collaboration revenue

During the three and nine months ended September 30, 2023, we recognized $2.1 million and $3.0 million, respectively, in collaboration revenue under our Collaboration Agreement with Moderna. For additional information on our collaboration with Moderna and the accounting thereunder, refer to Note 4, Collaboration and License Agreements.

Research and development expenses

The following table summarizes our research and development expenses for the three and nine months ended September 30, 2023 and 2022:

(in thousands)	Three Months Ended September 30, 2023	2022	Change 2023 vs 2022	Nine Months Ended September 30, 2023	2022	Change 2023 vs 2022
Personnel-related	$ 6,940	$ 7,258	$ (318)	$ 21,032	$ 22,427	$ (1,395)
Preclinical and manufacturing	4,905	3,339	1,566	15,111	11,513	3,598
Facilities-related	3,434	3,964	(530)	10,326	18,826	(8,500)
Stock-based compensation	2,874	3,046	(172)	8,608	9,674	(1,066)
Lab supplies	1,463	1,180	283	3,318	4,111	(793)
Consulting and professional services	486	562	(76)	1,504	2,733	(1,229)
Other	1,760	1,843	(83)	5,795	5,827	(32)
Total research and development expenses	$ 21,862	$ 21,192	$ 670	$ 65,694	$ 75,111	$ (9,417)

Research and development expenses were $21.9 million for the three months ended September 30, 2023, compared to $21.2 million for the three months ended September 30, 2022. The increase in preclinical and manufacturing costs of $1.6 million was driven primarily by increased preclinical activities. This increase was offset primarily by a decrease in facilities-related costs of $0.5 million, primarily driven by our decision to transition from building out the Seyon Facility to utilizing an external cleanroom facility.

Research and development expenses were $65.7 million for the nine months ended September 30, 2023, compared to $75.1 million for the nine months ended September 30, 2022. The decrease in facilities-related costs of $8.5 million was primarily driven by our decision to transition from building out the Seyon Facility to utilizing an external cleanroom

facility. The decreases in personnel-related and stock-based compensation costs of $1.4 million and $1.1 million, respectively, were driven by a slight decrease in headcount. These decreases were offset by an increase in preclinical and manufacturing costs of $3.6 million, driven primarily by increased preclinical activities.

General and administrative expenses

The following table summarizes our general and administrative expenses for the three and nine months ended September 30, 2023 and 2022:

(in thousands)	Three Months Ended September 30, 2023	2022	Change 2023 vs 2022	Nine Months Ended September 30, 2023	2022	Change 2023 vs 2022
Personnel-related	$ 3,646	$ 3,822	$ (176)	$ 12,195	$ 11,592	$ 603
Stock-based compensation	3,130	2,802	328	9,685	8,931	754
Professional and consultant fees	1,794	1,565	229	6,409	5,720	689
Facilities-related	2,495	2,730	(235)	7,443	3,528	3,915
Other	576	558	18	1,742	1,612	130
Total general and administrative expenses	$ 11,641	$ 11,477	$ 164	$ 37,474	$ 31,383	$ 6,091

General and administrative expenses were $11.6 million for the three months ended September 30, 2023, compared to $11.5 million for the three months ended September 30, 2022. The increase in stock-based compensation costs of $0.3 million was primarily a result of a slight increase in headcount to support our general and administrative function.

General and administrative expenses were $37.5 million for the nine months ended September 30, 2023, compared to $31.4 million for the nine months ended September 30, 2022. The increase in facilities-related costs of $3.9 million was primarily driven by rent expense related to the Seyon Facility after our decision to transition from building out the Seyon Facility to utilizing an external cleanroom facility. The increases in stock-based compensation costs and personnel-related costs of $0.8 million and $0.6 million, respectively, were primarily a result of a slight increase in headcount to support our general and administrative function.

Other income and interest income, net

Other income and interest income, net for the three and nine months ended September 30, 2023 was $3.1 million and $8.7 million, as compared to $1.4 million and $2.3 million for the three and nine months ended September 30, 2022. The increase in other income and interest income, net during the three and nine months ended September 30, 2023 was primarily due to an increase of interest earned on our invested cash balances.

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. We expect that any revenue recognized for the next several years will be derived primarily from our current collaboration with Moderna and any additional collaborations that we may enter into in the future. Through September 30, 2023, we have recognized $3.0 million in collaboration revenue under the Collaboration Agreement with Moderna. Historically, we have funded our operations with proceeds from the sale of instruments convertible into convertible preferred stock (which converted into convertible preferred stock in 2017), sales of convertible preferred stock (which converted into common stock in 2020), and sales of common stock in underwritten public offerings, “at-the-market” offerings, and in a private placement, as well as payments pursuant to our collaboration with Moderna. 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 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. In August 2021, we entered into an "at-the-market" sales agreement pursuant to which we may, from time to time, sell shares of our common stock having an aggregate offering price of up to $250.0 million. As of November 9, 2023, the issuance date of the condensed consolidated financial statements, we have issued and sold 1,795,524 shares of our common stock pursuant to this sales agreement resulting in net proceeds of $12.3 million. In March 2023, in connection with the Share Purchase Agreement with Moderna, we issued and sold 5,859,375 shares of our common stock to Moderna at a price of $6.14 per share for an aggregate purchase price of $36.0 million. As of September 30, 2023, we had cash, cash equivalents and marketable securities of $291.0 million.

Cash flows

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

	Nine Months Ended September 30,	
(in thousands)	**2023**	**2022**
Net cash used in operating activities	$ (27,767)	$ (79,445)
Net cash used in investing activities	(7,784)	(174,119)
Net cash provided by financing activities	35,740	12,906
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 189	$ (240,658)

Operating activities

During the nine months ended September 30, 2023, operating activities used $27.8 million of cash, primarily resulting from our net loss of $91.4 million, offset by the net changes in our operating assets and liabilities of $48.1 million and the net of non-cash charges of $15.6 million. Net changes in our operating assets and liabilities for the nine months ended September 30, 2023 consisted of a $44.5 million increase of deferred revenue, a $4.8 million decrease in operating lease right-of-use assets, a $1.4 million decrease in prepaid expenses and other current assets, a $0.9 million decrease of other noncurrent assets offset by a $0.3 million increase in tenant receivable, a $1.2 million decrease of accrued expense and other current liabilities and accounts payable and a $2.0 million decrease in operating lease liability.

During the nine months ended September 30, 2022, operating activities used $79.4 million of cash, primarily resulting from our net loss of $104.2 million, offset by the net of non-cash charges of $26.8 million and cash used in the changes in our operating assets and liabilities of $2.0 million. Net cash used by changes in our operating assets and liabilities for the nine months ended September 30, 2022 consisted of a $1.2 million increase of other noncurrent assets, a $2.5 million increase in operating lease liability, a $2.1 million decrease of accrued expense and other current liabilities and accounts payable, a $4.9 million increase in prepaid expenses and other current assets, a $4.0 million decrease in operating lease right-of-use assets and a $0.4 million increase in tenant receivable.

Changes in accounts payable, accrued expenses and other current liabilities, prepaid expenses, and other long-term assets in the periods were generally due to growth in our business and the timing of vendor invoicing and payments.

Investing activities

During the nine months ended September 30, 2023, net cash used in investing activities was $7.8 million, primarily due to purchases of marketable securities of $296.0 million and property and equipment of $2.8 million during the period, offset by $291.0 million in maturities of marketable securities. During the nine months ended September 30, 2022, net cash used in investing activities was $174.1 million, due to an increase in purchases of marketable securities of $221.0 million and property and equipment of $8.2 million during the period, offset by $55.0 million in maturities of marketable securities. Property and equipment purchases during the nine months ended September 30, 2023 and 2022 were primarily related to leasehold improvements and lab equipment for our facility in Cambridge, Massachusetts.

Financing activities

During the nine months ended September 30, 2023, net cash provided by financing activities was $35.7 million, consisting primarily of net proceeds from the sale and issuance of our common stock to Moderna. During the nine months ended September 30, 2022, net cash provided by financing activities was $12.9 million, consisting primarily of net proceeds from the issuance of common stock pursuant to our "at-the-market" sales agreement of $12.4 million and $1.0 million in proceeds from the exercise of common stock options and other types of equity, net during the period, offset by tax withholding payments relating to net share settlements of restricted stock units.

Funding requirements

We expect our expenses to increase substantially in connection with our ongoing activities, particularly as we advance 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;
- our research and development costs and the amounts we receive as reimbursement and milestone payments under our collaboration with Moderna;
- the costs, timing and outcome of regulatory review of any product candidates we may develop;
- the cost and timing of completion of commercial-scale manufacturing activities, including the costs and resources required to manufacture our drug substance and drug product using external cleanroom facilities and/or CMOs;
- 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 2026. 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. Although we may receive potential future payments under our collaboration with Moderna, 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 various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ significantly from these estimates under different assumptions or conditions.

We define our critical accounting policies as those accounting principles generally accepted in the United States that require us to make subjective estimates and judgments about matters that are uncertain and are likely to have a material impact on our financial condition and results of operations as well as the specific manner in which we apply those principles. Management has determined that our most critical accounting policies are those relating to accrued research and development expenses, stock-based compensation, and revenue recognition.

Except for the addition of Revenue Recognition, during nine months ended September 30, 2023, 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.

Revenue Recognition

We enter into collaboration agreements that are within the scope of Accounting Standards Codification Topic 606, “Revenue from Contracts with Customers”, or ASC 606, under which we license rights to certain of our potential product candidates and perform research and development services.

Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for contracts determined to be within the scope of ASC 606, we perform the following five steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations, including whether they are distinct in the context

of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when, or as, we satisfy each performance obligation. We only apply the five-step model to contracts when it is probable that we will collect consideration we are entitled to in exchange for the goods or services we transfer to the customer. For a further discussion, please refer to Note 2, Revenue Recognition.

Item 3. Quantitative and Qualitative Disclosures about Market Risks.

Interest Rate Market Risk

We are exposed to market risk related to changes in interest rates. We had marketable securities of $197.7 million as of September 30, 2023. During the nine months ended September 30, 2023, we recognized $8.5 million in interest earned on our invested cash balances and we did not record any impairment charges to our marketable securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because a majority of our investments are in short-term securities. Interest rate changes would result in a change in the net fair value of these financial instruments due to the difference between the current market interest rate and the market interest rate at the date of purchase of the financial instrument. We currently do not seek to hedge this exposure to fluctuations in interest rates. We have not been exposed to, nor do we anticipate being exposed to, material risks due to changes in interest rates.

Counterparty Credit Risk

Our investment portfolio is subject to counterparty credit risk due to potential changes in the credit ratings of the issuers. A downgrade in the credit rating of an issuer of a debt security or further deterioration of the credit markets could result in a decline in the fair value of the debt instruments. Our investment guidelines prohibit investment in auction rate securities and we do not believe we have any direct exposure to losses relating from mortgage-based securities or derivatives related thereto such as credit-default swaps.

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 September 30, 2023. 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 September 30, 2023, 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

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 three months ended September 30, 2023 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.

We hold a portion of our cash and cash equivalents that we use to meet our working capital and operating expense needs in deposit accounts that could be adversely affected if the financial institutions holding such funds fail.

We hold a portion of cash and cash equivalents that we use to meet our working capital and operating expense needs in deposit accounts. The balance held in these accounts may exceed the Federal Deposit Insurance Corporation, or FDIC, standard deposit insurance limit of $250,000. If a financial institution in which we hold such funds fails or is subject to significant adverse conditions in the financial or credit markets, we could be subject to a risk of loss of all or a portion of such uninsured funds or be subject to a delay in accessing all or a portion of such uninsured funds. Any such loss or lack of access to these funds could adversely impact our short-term liquidity and ability to meet our operating expense obligations.

For example, on March 10, 2023, Silicon Valley Bank, or SVB, and Signature Bank were closed by state regulators and the FDIC was appointed receiver for each bank. The FDIC created successor bridge banks and all deposits of SVB and Signature Bank were transferred to the bridge banks under a systemic risk exception approved by the United States Department of the Treasury, the Federal Reserve and the FDIC. While we believe that we are not exposed to significant credit risk due to the financial strength of the national depository institutions in which our cash, cash equivalents, and marketable securities are held, if the financial institutions in which we hold funds for working capital and operating expenses were to fail, we cannot provide any assurances that such governmental agencies would take action to protect our uninsured deposits in a similar manner.

We also maintain investment accounts in which we hold our investments and, if access to the funds we use for working capital and operating expenses is impaired, we may not be able to open new operating accounts or to sell investments or transfer funds from our investment accounts to new operating accounts on a timely basis sufficient to meet our operating expense obligations.

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

In March 2023, we entered into the Collaboration Agreement with Moderna, to collaborate on developing treatments for certain diseases by targeting delivery of nucleic acids to liver cells and certain cells outside of the liver utilizing our ctLNP proprietary platform. We may seek in the future additional third-party collaborators for the research, development and commercialization of certain of the product candidates we may develop. However, we have agreed to certain exclusivity provisions that limit our ability to develop, manufacture, commercialize or exploit certain products we develop pursuant to the Collaboration Agreement or against certain targets set forth in the Collaboration Agreement and, as a result, could limit our ability to into additional third-party collaborations. We will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of any product candidates we may seek to develop with them. For example, while Moderna has agreed to use commercially reasonable efforts to complete the activities assigned to it under the research plans set forth in the Collaboration Agreement, we cannot control the amount or timing of resources that they dedicate to these activities. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. We cannot predict the success of our collaboration with Moderna or any other collaboration that we may enter into.

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;

- collaborators may not pursue development and commercialization of any product candidates we may develop or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the collaborator's strategic focus or available funding or external factors such as an acquisition that diverts resources or creates competing priorities;

- collaborators may delay programs, preclinical studies or clinical trials, provide insufficient funding for programs, preclinical studies or clinical trials, stop a preclinical study or clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;

- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with any product candidates we may develop if the collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;

- collaborators may be acquired by a third party having competitive products or different priorities;

- collaborators with marketing and distribution rights to one or more medicines may not commit sufficient resources to the marketing and distribution of such medicine or medicines;

- collaborators may not properly obtain, maintain, enforce or defend our intellectual property or proprietary rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our proprietary information or expose us to potential litigation;

- disputes may arise between the collaborators and us that result in the delay or termination of the research, development, or commercialization of our medicines or any product candidates we may develop or that result in costly litigation or arbitration that diverts management attention and resources;

- we may lose certain valuable rights under certain circumstances, including if we undergo a change of control;

- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates we may develop; and

- collaboration agreements, including the Collaboration Agreement with Moderna, may not lead to development or commercialization of product candidates in the most efficient manner or at all. If a present or future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our product development or commercialization program under such collaboration could be delayed, diminished or terminated.

If our collaborations do not result in the successful development and commercialization of product candidates, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed, and we may need additional resources to develop product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to find a suitable replacement collaborator or attract new collaborators, and our development programs may be delayed or the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in the risk factors discussed in Part I, Item 1A in our Annual Report apply to the activities of our collaborators.

We may in the future decide to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of any product candidates we may develop. These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that

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

Our current collaboration with Moderna, future acquisitions or strategic alliances could disrupt our business and harm our financial condition and results of operations.

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

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

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

Item 5. Other Information.

Director and Officer Trading Arrangements

None of our directors or officers adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K) during the quarterly period covered by this Quarterly Report.

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: November 9, 2023

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

Date: November 9, 2023

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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a15(f) and 15d-15(f)) 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) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) 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

(d) 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: November 9, 2023

/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)) and internal control over financial reporting (as defined in Exchange Act Rules 13a15(f) and 15d-15(f)) 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) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

(c) 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

(d) 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: November 9, 2023

/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 September 30, 2023 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)
November 9, 2023

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 September 30, 2023 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)
November 9, 2023