
**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, 2024

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)

301 Binney Street
Cambridge, Massachusetts
(Address of principal executive offices)

81-4301284
(I.R.S. Employer
Identification Number)

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	<input type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>	Smaller reporting company	<input checked="" type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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 1, 2024 there were 66,792,822 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, or the Exchange Act, 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:

- 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., or Moderna;
- the potential advantages of our non-viral genetic medicine platforms;
- the initiation, timing, progress and results of our research and development programs and preclinical studies and clinical trials;
- the timing of and our ability to submit applications and obtain and maintain regulatory approvals for any product candidates we may develop;
- our plans to develop and, if approved, subsequently commercialize 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 commercial objectives;
- the impact of government laws and regulations;
- our competitive position and expectations regarding developments and projections relating to our competitors and any competing therapies that are or may become available; and
- our ability to maintain and establish collaborations or obtain additional funding.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and stockholders should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report, particularly in the “Risk Factors”

section in this Quarterly Report and our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission, or SEC, that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Moreover, we operate in a competitive and rapidly changing environment. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures, or investments we may make or enter into.

Stockholders should read this Quarterly Report and the documents that we file with the SEC with the understanding that our actual future results may be materially different from what we expect. The forward-looking statements contained in this Quarterly Report are made as of the date of this Quarterly Report, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

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

Generation Bio Co.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements (unaudited)

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

	<u>September 30,</u> <u>2024</u>	<u>December 31,</u> <u>2023</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 21,657	\$ 66,446
Marketable securities	163,113	197,918
Collaboration receivable	2,212	—
Tenant receivable	—	3,960
Prepaid expenses and other current assets	4,272	4,294
Total current assets	<u>191,254</u>	<u>272,618</u>
Marketable securities, net of current portion	15,042	—
Property and equipment, net	16,010	25,799
Operating lease right-of-use assets	21,220	69,852
Restricted cash	2,152	5,791
Other long-term assets	3,106	698
Total assets	<u>\$ 248,784</u>	<u>\$ 374,758</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 1,659	\$ 2,346
Accrued expenses and other current liabilities	6,197	16,529
Deferred revenue	11,512	12,919
Operating lease liability	10,797	8,120
Total current liabilities	<u>30,165</u>	<u>39,914</u>
Deferred revenue, net of current portion	31,195	41,942
Operating lease liability, net of current portion	82,982	89,774
Total liabilities	<u>144,342</u>	<u>171,630</u>
Commitments and contingencies (Note 10)		
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 5,000,000 shares authorized and no shares issued or outstanding at September 30, 2024 and December 31, 2023	—	—
Common stock, \$0.0001 par value; 150,000,000 shares authorized at September 30, 2024 and December 31, 2023; 66,753,508 and 66,205,550 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively	7	7
Additional paid-in capital	785,779	774,224
Accumulated other comprehensive income	319	274
Accumulated deficit	(681,663)	(571,377)
Total stockholders' equity	<u>104,442</u>	<u>203,128</u>
Total liabilities and stockholders' equity	<u>\$ 248,784</u>	<u>\$ 374,758</u>

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,	
	2024	2023	2024	2023
Revenues:				
Collaboration revenue	\$ 7,554	\$ 2,146	15,704	\$ 3,026
Operating expenses:				
Research and development	15,088	21,862	45,811	65,694
General and administrative	9,181	11,641	29,124	37,474
Loss on lease termination	1,169	—	59,596	—
Total operating expenses	25,438	33,503	134,531	103,168
Loss from operations	(17,884)	(31,357)	(118,827)	(100,142)
Other income:				
Other income and interest income, net	2,571	3,091	8,541	8,716
Net loss	\$ (15,313)	\$ (28,266)	(110,286)	\$ (91,426)
Net loss per share, basic and diluted	\$ (0.23)	\$ (0.43)	(1.66)	\$ (1.43)
Weighted average common shares outstanding, basic and diluted	66,738,468	65,907,000	66,568,326	63,951,508
Comprehensive loss:				
Net loss	\$ (15,313)	\$ (28,266)	(110,286)	\$ (91,426)
Other comprehensive (loss) income:				
Unrealized gains on marketable securities	599	19	45	79
Comprehensive loss	\$ (14,714)	\$ (28,247)	(110,241)	\$ (91,347)

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 Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Three Months Ended September 30, 2024						
Balances at June 30, 2024	66,702,734	\$ 7	\$ 782,030	\$ (280)	\$ (666,350)	\$ 115,407
Issuance of common stock upon exercise of stock options	9,680	—	13	—	—	13
Vesting of restricted common stock	41,094	—	(16)	—	—	(16)
Stock-based compensation expense	—	—	3,752	—	—	3,752
Unrealized gains on marketable securities	—	—	—	599	—	599
Net loss	—	—	—	—	(15,313)	(15,313)
Balances at September 30, 2024	<u>66,753,508</u>	<u>\$ 7</u>	<u>\$ 785,779</u>	<u>\$ 319</u>	<u>\$ (681,663)</u>	<u>\$ 104,442</u>

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (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	<u>65,927,956</u>	<u>\$ 7</u>	<u>\$ 768,102</u>	<u>\$ (4)</u>	<u>\$ (536,191)</u>	<u>\$ 231,914</u>

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 Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Nine Months Ended September 30, 2024						
Balances at December 31, 2023	66,205,550	\$ 7	\$ 774,224	\$ 274	\$ (571,377)	\$ 203,128
Issuance of common stock upon exercise of stock options	22,517	—	31	—	—	31
Vesting of restricted common stock	369,414	—	(172)	—	—	(172)
Issuance of common stock under ESPP	156,027	—	247	—	—	247
Stock-based compensation expense	—	—	11,449	—	—	11,449
Unrealized gains on marketable securities	—	—	—	45	—	45
Net loss	—	—	—	—	(110,286)	(110,286)
Balances at September 30, 2024	<u>66,753,508</u>	<u>\$ 7</u>	<u>\$ 785,779</u>	<u>\$ 319</u>	<u>\$ (681,663)</u>	<u>\$ 104,442</u>

	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (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 ESPP	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	<u>65,927,956</u>	<u>\$ 7</u>	<u>\$ 768,102</u>	<u>\$ (4)</u>	<u>\$ (536,191)</u>	<u>\$ 231,914</u>

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,	
	2024	2023
Cash flows from operating activities:		
Net loss	\$ (110,286)	\$ (91,426)
Adjustments to reconcile net loss to net cash used in operating activities:		
Loss on lease termination	59,085	—
Stock-based compensation expense	11,449	18,293
Depreciation and amortization expense	3,755	3,938
Amortization (accretion) of premium (discount) on marketable securities, net	(6,312)	(6,699)
Other	84	24
Changes in operating assets and liabilities:		
Collaboration receivable	(2,212)	—
Tenant receivable	—	(265)
Prepaid expenses and other current assets	694	1,408
Operating lease right-of-use assets	2,799	4,794
Other noncurrent assets	(2,840)	931
Accounts payable	(79)	1,386
Accrued expenses and other current liabilities	(9,526)	(2,633)
Deferred revenue	(12,154)	44,474
Operating lease liability	(7,236)	(1,992)
Net cash used in operating activities	<u>(72,779)</u>	<u>(27,767)</u>
Cash flows from investing activities:		
Purchases of property and equipment	(2,023)	(2,817)
Proceeds from sale of property and equipment	149	—
Purchases of marketable securities	(115,881)	(295,967)
Maturities of marketable securities	142,000	291,000
Net cash provided by (used in) investing activities	<u>24,245</u>	<u>(7,784)</u>
Cash flows from financing activities:		
Payment of share issuance costs	—	(179)
Proceeds from sale of common stock in connection with the Moderna Share Purchase Agreement	—	36,000
Proceeds from exercise of stock options and ESPP, net	278	367
Tax withholding payments related to net share settlements of restricted stock units	(172)	(448)
Net cash provided by financing activities	<u>106</u>	<u>35,740</u>
Net (decrease) increase in cash, cash equivalents and restricted cash	(48,428)	189
Cash, cash equivalents and restricted cash at beginning of period	72,237	98,863
Cash, cash equivalents and restricted cash at end of period	<u>\$ 23,809</u>	<u>\$ 99,052</u>
Supplemental disclosure of noncash investing and financing information:		
Purchases of property and equipment included in accounts payable and accrued expenses	\$ 47	\$ 1,330
Unrealized gains on marketable securities	\$ 45	\$ 79

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

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

1. Nature of the Business and Basis of Presentation

Generation Bio Co., or Generation Bio, was incorporated on October 21, 2016 as Torus Therapeutics, Inc. and subsequently changed its name to Generation Bio Co. Generation Bio Co. and its consolidated subsidiary, or the company, we, our or us, are innovating non-viral genetic medicines to provide durable, redosable treatments for potentially hundreds of millions of patients living with rare and prevalent diseases. We are developing two distinct and complementary platforms that we believe will enable highly differentiated therapeutic applications. Our first platform is a potent, highly selective cell-targeted lipid nanoparticle, or ctLNP, delivery system for nucleic acids, which is designed to avoid off-target clearance by the liver and spleen, enabling ctLNPs to persist in systemic circulation and allowing for highly selective and potent ligand-driven targeting to specific tissues and cell types. The identification and optimization of new ligands to target new tissues and cell types is an efficient, flexible, and modular process, which we believe will allow us to rapidly expand our portfolio. Our second platform is our novel immune-quiet DNA, or iqDNA, a partially single-stranded DNA, which is a variant of our closed-ended DNA, or ceDNA, designed to enable long-lasting high levels of gene expression from non-integrating episomes, while avoiding innate immune sensors that have long prevented DNA from use in non-viral systems. Underpinning the iqDNA platform is our highly scalable capsid-free manufacturing process that uses our proprietary cell-free rapid enzymatic synthesis, or RES, to produce highly pure iqDNA at scale. 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 collaboration revenue under our collaboration with ModernaTX, Inc., or Moderna. We have incurred recurring losses, including net losses of \$110.3 million for the nine months ended September 30, 2024 and \$91.4 million for the nine months ended September 30, 2023. As of September 30, 2024, we had an accumulated deficit of \$681.7 million. We expect to continue to generate operating losses in the foreseeable future. As of November 6, 2024, 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 measurement of proportional performance of the performance obligation of our collaboration agreements, 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, 2023 was derived from audited financial statements but does not include all disclosures required by GAAP. The accompanying unaudited financial statements as of September 30, 2024 and for the three and nine months ended September 30, 2024 and 2023 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, 2024, the results of operations for the three and nine months ended September 30, 2024 and 2023, and cash flows for the nine months ended September 30, 2024 and 2023 have been made. The results of operations for the three and nine months ended September 30, 2024 are not necessarily indicative of the results of operations that may be expected for the year ending December 31, 2024 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. Updates to our significant accounting policies are discussed below.

Employee Retention Credit

Under the provisions of the extension of the Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, passed by the United States Congress and signed by the President, we are eligible for a refundable Employee Retention Credit, or ERC, subject to certain criteria. ASC 105, Generally Accepted Accounting Principles, describes the decision-making framework when no clear guidance exists in GAAP for a particular transaction. Specifically, ASC 105-10-05-2 instructs companies to look for guidance for a similar transaction within GAAP and apply that guidance by analogy. As such, forms of government assistance, such as the ERC, provided to business entities would not be within the scope of International Accounting Standards 20, or IAS 20, Accounting for Government Grants and Disclosure of Government Assistance, but it may be applied by analogy under ASC 105-10-05-2. We accounted for the ERC as a government grant in accordance with IAS 20 by analogy under ASC 105-10-05-2.

We recognized a \$2.3 million ERC upon completion of an analysis providing reasonable assurance that we met the conditions set forth in the CARES Act and it was reasonably assured that we will receive the employee retention credit. We recorded the ERC in other long-term assets on our condensed consolidated balance sheet as of September 30, 2024 related to labor costs recognized during 2020 and 2021. The ERC was recorded in research and development expenses and general and administrative expenses proportionately in the manner in which the qualified wages and related costs were classified. We have filed for refunds of the ERC and as of the date of this Quarterly Report, we have not received any refunds.

3. Marketable Securities and Fair Value Measurements

The following tables present our marketable securities by security type:

(in thousands)	As of September 30, 2024			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury securities	\$ 177,836	\$ 319	\$ —	\$ 178,155

(in thousands)	As of December 31, 2023			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury securities	\$ 197,644	\$ 274	\$ —	\$ 197,918

The following table summarizes our marketable securities by contractual maturity as of September 30, 2024:

(in thousands)	As of September 30, 2024	
	Amortized Cost	Fair Value
Less than one year	\$ 162,932	\$ 163,113
Greater than one year but less than two years	14,904	15,042
Total	<u>\$ 177,836</u>	<u>\$ 178,155</u>

Our marketable securities as of December 31, 2023 consisted of investments that mature within one year of their purchase date.

We assess our available-for-sale securities under the available-for-sale security impairment model in ASC 326, “Financial Instruments - Credit Losses”, or ASC 326, as of each reporting date in order to determine if a portion of any decline in fair value below carrying value recognized on our available-for-sale securities is the result of a credit loss. We also evaluate our available-for-sale securities for impairment using a variety of factors including our intent to sell the underlying securities prior to maturity and whether it is more likely than not that we would be required to sell the securities before the recovery of their amortized basis. During the nine months ended September 30, 2024 and 2023, we did not recognize any impairment or realized gains or losses on sales of available-for-sale securities, and we did not record an allowance for, or recognize, any expected credit losses.

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:

(in thousands)	Fair Value Measurements at September 30, 2024 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	\$ 5,460	\$ —	\$ —	\$ 5,460
Marketable securities:				
U.S. treasury securities	—	178,155	—	178,155
Totals	\$ 5,460	\$ 178,155	\$ —	\$ 183,615

(in thousands)	Fair Value Measurements at December 31, 2023 Using:			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	\$ 38,210	\$ —	\$ —	\$ 38,210
Marketable securities:				
U.S. treasury securities	—	197,918	—	197,918
Totals	\$ 38,210	\$ 197,918	\$ —	\$ 236,128

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 and resulted in Moderna becoming a related party. 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 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.

During the three months ended September 30, 2024, we recorded additional revenue related to a change in estimate due to revisions in the research plan resulting in a reduction in the estimated timeline of future research services and total estimated costs. The change in estimate resulted in a \$6.8 million decrease in the allocated transaction price and a \$3.8 million increase in collaboration revenue, a \$3.8 million decrease in deferred revenue and a \$0.06 decrease in net loss per share, basic and diluted, for both the three and nine months ended September 30, 2024.

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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	Revenue Recognized During	Deferred Revenue
	As of September 30, 2024	Three Months Ended September 30, 2024	Nine Months Ended September 30, 2024	As of September 30, 2024
(in thousands)				
ctLNP technology and research license	\$ 40,409	\$ 7,554	\$ 15,704	\$ 14,549
First liver program commercialization option license	5,388	—	—	4,526
Second liver program commercialization option license	5,388	—	—	4,526
First non-liver program commercialization option license	9,006	—	—	7,565
Second non-liver program commercialization option license	9,006	—	—	7,565
Third liver or non-liver program commercialization option license	4,734	—	—	3,976
Total	\$ 73,931	\$ 7,554	\$ 15,704	\$ 42,707

5. Property and equipment, net

Property and equipment, net consisted of the following:

(in thousands)	September 30,	December 31,
	2024	2023
Laboratory equipment	\$ 14,543	\$ 14,859
Computer equipment and software	1,422	1,447
Furniture and fixtures	1,293	1,293
Leasehold improvements	20,909	20,865
Construction in progress	112	7,030
	38,279	45,494
Less: Accumulated depreciation and amortization	(22,269)	(19,695)
Total	\$ 16,010	\$ 25,799

Depreciation and amortization expense for the three and nine months ended September 30, 2024 was 1.2 million and \$3.8 million, respectively. Depreciation and amortization expense for the three and nine months ended September 30, 2023 was \$1.3 million and \$3.9 million, respectively.

In July 2021, we entered into a lease agreement for a manufacturing facility in Waltham, Massachusetts, or the Seyon Lease. On January 31, 2024, we notified Waltham CenterPoint I Investment Group, LLC, or Landlord, of termination of the Seyon Lease due to Landlord's breach of its obligations to us under the Seyon Lease and returned possession of the premises to Landlord, effective January 31, 2024. On February 14, 2024, Landlord sent us a notice of the termination of the Seyon Lease due to our non-payment of rent. On February 20, 2024, Landlord served us with a complaint, filed in Massachusetts Superior Court, or the Court, with respect to the Seyon Lease. The complaint sought declaratory judgment that we unlawfully terminated the Seyon Lease and also asserted a claim for breach of contract damages. Following receipt of the complaint, we filed a counterclaim against Landlord asserting breach of contract and violation of Massachusetts General Law Chapter 93A. On October 29, 2024, the Court determined that although we did not have the right to terminate the Seyon Lease, Landlord's subsequent termination was effective, and that we had alleged sufficient facts to state a claim for breach of contract and violation of Massachusetts General Law Chapter 93A by Landlord. We will continue to vigorously defend our rights with respect to this matter. During the nine months ended September 30, 2024, in connection with the termination of the Seyon Lease, we recorded a non-cash charge of \$6.2 million in an impairment of construction in progress. For additional information, refer to Note 7, Leases.

6. Accrued Expenses

Accrued expenses and other current liabilities consisted of the following:

(in thousands)	<u>September 30,</u> <u>2024</u>	<u>December 31,</u> <u>2023</u>
Accrued employee compensation and benefits	\$ 4,505	\$ 13,208
Accrued external research and development expenses	600	1,169
Accrued professional fees	654	908
Property and equipment	33	838
Other	405	406
Total	<u>\$ 6,197</u>	<u>\$ 16,529</u>

In November 2023, following a review of strategic priorities and a determination by our management and board of directors to implement a strategic reorganization, to invest in our ctLNP delivery platform to develop wholly-owned programs for extrahepatic cell types and to develop our iqDNA platform for our lead program in hemophilia A and other programs, we announced a reduction in our workforce of approximately 40%, or RIF, and implemented reductions in operational expenditures including Good Manufacturing Practice readiness and manufacturing expenses. We completed the RIF during the second quarter of 2024.

In connection with the RIF, affected employees were eligible to receive one-time severance benefits, including cash severance, temporary healthcare coverage, to the extent they were eligible for and elected such coverage, and transition support services, subject to each such employee entering into an effective separation agreement, which included a general release of claims against us. We offered a retention bonus to certain affected employees if such employees remained in continuous employment with us through their respective separation dates and executed a general release of claims against us.

Below is a summary of accrued restructuring costs recorded and included in accrued expenses and other current liabilities during the year ended September 30, 2024:

(in thousands)	<u>Severance and Benefits Costs</u>
Balance at December 31, 2023	<u>\$ 5,291</u>
Cash payments	(5,055)
Restructuring expenses	380
Adjustments	(402)
Balance at September 30, 2024	<u>\$ 214</u>

During the nine months ended September 30, 2024, we recorded \$0.4 million of restructuring expenses in our condensed consolidated statements of operation and comprehensive loss all of which was classified as general and administrative expense. We did not recognize any restructuring expense during the nine months ended September 30, 2023.

7. Leases

We lease our office and laboratory space under a noncancelable operating lease that expires in 2029, or the Office and Lab Lease. We have an option to extend the Office and Lab Lease term for one additional term of five years at the greater of the then-current base rent or the then-current fair market value. Exercise of this option was not determined to be reasonably certain and thus was not considered in determining the operating lease liability on the consolidated balance sheet as of September 30, 2024. We posted a letter of credit in the amount of approximately \$2.1 million as a security deposit. The letter of credit is subject to increase if we were to sublease any portion of the leased premises. The Office and Lab Lease does not include any restrictions or covenants that had to be accounted for under the lease guidance.

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Future lease payments for our noncancelable operating leases as of September 30, 2024 and a reconciliation to the carrying amount of the operating lease liabilities presented in the condensed consolidated balance sheet as of September 30, 2024 are as follows:

Three Months Ended September 30,	(in thousands)	
2024 (remaining 3 months)	\$	1,935
2025		7,838
2026		8,059
2027		8,275
2028		8,535
Thereafter		2,834
Total undiscounted payments due under operating leases		37,476
Less imputed interest		(5,673)
Total	\$	31,803
Current operating lease liability	\$	5,707
Non-current operating lease liability		26,096
Total	\$	31,803

The following table presents our costs included in operating expenses related to our noncancelable operating leases:

(in thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Operating lease cost	\$ 1,467	\$ 3,280	\$ 4,401	\$ 9,842
Variable lease cost	528	832	1,575	2,542
Total	\$ 1,995	\$ 4,112	\$ 5,976	\$ 12,384

Net cash paid for the amounts included in the measurement of the operating lease liability on the condensed consolidated balance sheet and operating activities in our consolidated statement of cash flows was \$5.7 million and \$9.6 million for the nine months ended September 30, 2024 and 2023, respectively. The weighted-average remaining lease term and weighted-average incremental borrowing rate for all leases as of September 30, 2024 was approximately 5 years and 7.1%, respectively.

The Seyon Lease commenced in December 2021, when we were granted access to the facility, and monthly rent payments began in September 2022; the total rent payment was expected to be approximately \$104.3 million for the 12-year lease term. We had an option to extend the Seyon Lease term for two additional terms of five years each at the greater of the then-current base rent or the then-current fair market value. Exercise of this option was not determined to be reasonably certain and thus was not considered in determining the operating lease liability. In connection with the Seyon Lease, we provided a security deposit of \$3.6 million in the form of a letter of credit. We paid an initial monthly base rent of approximately \$0.4 million that increased annually, up to a monthly base rent of \$0.6 million. We were obligated to pay operating costs, taxes and utilities applicable to the facility. We were responsible for costs of constructing interior improvements within the facility that exceed a construction allowance of \$26.0 million provided by the landlord. As previously disclosed in our most recent Annual Report on Form 10-K and in this Quarterly Report, the termination of the Seyon Lease is the subject of pending litigation with the landlord. As of September 30, 2024, the landlord has collected \$3.6 million from our security deposit in lieu of rent payments and has fully utilized such deposit.

In connection with the termination of the Seyon Lease, during the nine months ended September 30, 2024, we recorded a material impairment loss of non-cash charges of \$45.8 million in an impairment of the Seyon Lease right-of-use asset, \$6.2 million in an impairment of construction in progress, and the write-off of \$3.9 million in tenant improvement allowance receivable from the landlord. In addition, during the nine months ended September 30, 2024, we recognized

\$3.7 million in accretion and other lease related expenses, which resulted in a \$59.6 million loss on termination of lease in our condensed consolidated statement of operations and comprehensive loss. Accretion and other lease related expenses related to the Seyon Lease will continue to be recognized in loss on lease termination on our condensed consolidated statement of operations and comprehensive loss. As of September 30, 2024, as we have not met the criteria to extinguish the lease liability pursuant to ASC 405 *Liabilities*, and we had \$62.0 million in operating lease liability related to the Seyon Lease on our condensed consolidated balance sheet. Additionally, in accordance with ASC 450 *Contingencies*, we have assessed the probability of realizing a material loss contingency for the potential losses, including obligations to pay any future variable lease costs, under the Seyon Lease. Based on this assessment, we have determined that it is not probable that we will be obligated to pay any such amounts. As a result, we have not accrued for any amounts related to the potential losses, primarily variable lease costs, such as common area fees and other charges, as they relate to the Seyon Lease.

8. Equity

As of September 30, 2024, 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 2024, 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 \$237.0 million. Upon our entry into this sales agreement, we terminated our prior at-the-market program pursuant to a prior sales agreement that we entered into in August 2021. At the time of termination, approximately \$237.3 million out of an aggregate of \$250.0 million remained unsold under the prior sales agreement. As of November 6, 2024, the issuance date of these condensed consolidated financial statements, we have not issued and sold any shares of our common stock pursuant to the August 2024 sales agreement.

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.

9. 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 2024, the number of shares of common stock authorized for issuance under the 2020 Plan was increased from 16,813,962 shares to 19,462,688 shares. Upon the effectiveness of the 2020 Plan, we ceased granting additional awards under the 2017 Plan.

The Plans are administered by the board of directors or, at the discretion of the board of directors, by a committee of the board of directors. The exercise prices, vesting and other restrictions on any award under the Plans are determined at the 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 initial public offering, or IPO, in June 2020, the fair value of our 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, 2024, 790,738 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, 2024, we granted time-based options to certain employees for the purchase of an aggregate of 3,693,074 shares of common stock with a weighted average grant date fair value of \$1.87 per share that vest over a weighted average period of approximately four years.

Employee stock purchase plan

In May 2020, our board of directors adopted, and in June 2020, our stockholders approved, the 2020 Employee Stock Purchase Plan, or the 2020 ESPP, which became effective June 11, 2020. The 2020 ESPP is administered by our board of directors or by a committee appointed by the board of directors. The number of shares of common stock 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 2024, the number of shares of common stock authorized for issuance under the 2020 ESPP was increased from 2,115,792 shares to 2,777,974 shares. As of September 30, 2024, 2,204,771 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:

(in thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development expenses	\$ 1,520	\$ 2,874	\$ 4,452	\$ 8,608
General and administrative expenses	2,232	3,130	6,997	9,685
Total	\$ 3,752	\$ 6,004	\$ 11,449	\$ 18,293

As of September 30, 2024, total unrecognized compensation cost related to unvested time-based stock options and restricted stock units was \$14.7 million, with \$13.2 million expected to be recognized over a weighted average period of 2.1 years and \$1.5 million expected to be recognized over a weighted average period of 2.2 years, respectively.

10. 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. On February 20, 2024, our Landlord served us with a complaint, filed in the Court with respect to the Seyon Lease. The complaint sought declaratory judgment that we unlawfully terminated the Seyon Lease and also asserted a claim for breach of contract damages. Following receipt of the complaint, we filed a counterclaim against Landlord asserting breach of contract and violation of Massachusetts General Law Chapter 93A. On October 29, 2024, the Court determined that although we did not have the right to terminate the Seyon Lease, Landlord's subsequent termination was effective, and that we had alleged sufficient facts to state a claim for breach of contract and violation of Massachusetts General Law Chapter 93A by Landlord. We will continue to vigorously defend the action and our rights with respect to this matter. As a result, we may continue to incur costs and expenses relating to this facility, and we may remain responsible for payments under the Seyon Lease, which may have a material adverse effect on our business, results of operations or financial condition.

11. 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,	
	2024	2023
Unvested restricted stock units	374,076	1,179,349
Stock options to purchase common stock	13,215,012	9,676,685
Total	13,589,088	10,856,034

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 non-viral genetic medicines to provide durable, redosable treatments for potentially hundreds of millions of patients living with rare and prevalent diseases. We are developing two distinct and complementary platforms that we believe will enable highly differentiated therapeutic applications.

Our first platform is a potent, highly selective cell-targeted lipid nanoparticle, or ctLNP, delivery system for nucleic acids, which is designed to avoid off-target clearance by the liver and spleen, enabling ctLNPs to persist in systemic circulation and allowing for highly selective and potent ligand-driven targeting to specific tissues and cell types. The identification and optimization of new ligands to target new tissues and cell types is an efficient, flexible, and modular process, which we believe may allow us to rapidly expand our portfolio. We have demonstrated selective delivery of a T cell-targeted ctLNP carrying messenger RNA, or mRNA, cargo encoding a CAR with expression that was efficient and dose dependent. We plan to assess the efficacy of T cell-targeted ctLNPs delivering immune-quiet DNA, or iqDNA, in mice.

Our second platform is our novel iqDNA, a partially single-stranded DNA, which is an optimized variant of our closed-ended DNA, or ceDNA, designed to enable long-lasting high levels of gene expression from non-integrating episomes, while avoiding innate immune sensors that have long prevented DNA from use in non-viral systems. Underpinning the iqDNA platform is our highly scalable capsid-free manufacturing process that uses our proprietary cell-free rapid enzymatic synthesis, or RES, to produce highly pure iqDNA at scale. We continue to leverage RES to advance our iqDNA platform as it allows for precise chemical and structural changes to DNA, enabling the enhancement of DNA functionality through the engineering of molecular design and components. We have developed a second generation of iqDNA that achieved greater luciferase expression than a first generation iqDNA.

We are advancing a portfolio of programs guided by the potent and highly selective delivery of mRNA and/or iqDNA to T cells, hematopoietic stem cells, or HSCs, and hepatocytes. Our work in T cells initially focuses on *in vivo* reprogramming of this cell type to treat cancer and autoimmune diseases. Our HSC research is focused initially on *in vivo* gene editing of HSCs for hematologic disorders, prioritizing sickle cell disease and beta-thalassemia. Our work in hepatocytes prioritizes hemophilia A, a rare monogenic disease that results from mutations in a single gene, has significant unmet need, and clear biomarkers for development.

We plan to expand our portfolio to include programs for additional indications in other tissues, including retina, skeletal muscle, and the central nervous system, or CNS, by developing discrete ctLNPs, each with a unique targeting ligand engineered to provide targeted delivery of mRNA and/or iqDNA to T cells, HSCs and hepatocytes or delivery of antibody genes to direct the liver to produce therapeutic antibodies from patients’ own cells, which we refer to as endogenous therapeutic antibody production, or ETAP.

In November 2023, following a review of strategic priorities and a determination by our management and board of directors to implement a strategic reorganization to invest in our ctLNP delivery system to develop wholly-owned programs for extrahepatic cell types and to develop our iqDNA platform for our lead program in hemophilia A and other programs, we

announced a strategic reorganization, pursuant to which we undertook a reduction in force, or RIF, and implemented reductions in operational expenditures including current Good Manufacturing Practice readiness and manufacturing expenses. We completed the RIF during the second quarter of 2024. As part of the restructuring, we are prioritizing investment in the development of our ctLNP delivery system for wholly-owned programs in extrahepatic cell types and to develop iqDNA for our lead program in hemophilia A.

In July 2021, we entered into a lease agreement to build out a current Good Manufacturing Practice-compliant manufacturing facility, in Waltham, Massachusetts, or the Seyon Lease. On January 31, 2024, we notified Waltham CenterPoint I Investment Group, LLC, or Landlord, of termination of the Seyon Lease due to Landlord's breach of its obligations to us under the Seyon Lease and returned possession of the premises to the Landlord, effective January 31, 2024. On February 14, 2024, Landlord sent us a notice of the termination of the Seyon Lease due to our non-payment of rent. On February 20, 2024, Landlord served us with a complaint, filed in Massachusetts Superior Court, or the Court, with respect to the Seyon Lease. The complaint sought declaratory judgment that we unlawfully terminated the Seyon Lease and also asserted a claim for breach of contract damages. Following receipt of the complaint, we filed a counterclaim against Landlord asserting breach of contract and violation of Massachusetts General Law Chapter 93A. On October 29, 2024, the Court determined that although we did not have the right to terminate the Seyon Lease, Landlord's subsequent termination was effective, and that we had alleged sufficient facts to state a claim for breach of contract and violation of Massachusetts General Law Chapter 93A by Landlord. We will continue to vigorously defend the action and our rights with respect to this matter.

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.

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 Agreement.

Since our inception in October 2016, we have focused substantially all of our resources on building our non-viral genetic medicine platforms, 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 collaboration revenue under 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 2024, 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 \$237.0 million. Upon our entry into this sales agreement, we terminated our prior at-the-market program pursuant to a prior sales agreement that we entered into in August 2021. At the time of termination, approximately \$237.3 million out of an aggregate of \$250.0 million remained unsold under the prior sales agreement. As of November 6, 2024, the issuance date of this Quarterly Report, we have not issued and sold any shares of our common stock pursuant to the August 2024 sales agreement.

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, 2024 and 2023, we reported net losses of \$110.3 million and \$91.4 million, respectively. As of September 30, 2024, we had an accumulated deficit of \$681.7 million. We expect to continue to incur significant expenses and increasing operating losses for at least the next several years. We expect that our expenses and capital requirements will increase substantially in connection with our ongoing activities, particularly if and as we:

- continue our current research programs and conduct additional research programs, including pursuant to our collaboration with Moderna;
- expand the capabilities of our proprietary non-viral genetic medicine platforms;
- advance any product candidates we identify into preclinical and clinical development;
- obtain, expand, maintain, defend and enforce our intellectual property portfolio;
- seek marketing approvals for any product candidates that successfully complete clinical trials;
- hire additional clinical, regulatory and scientific personnel;
- 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;

- ultimately establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval; and
- add operational, legal, compliance, financial and management information systems and personnel to support our research, product development, and future commercialization efforts.

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 the second half of 2027. 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 as 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, stock-based compensation and severance 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 contract research organizations, or 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 platforms 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;
- our ability to raise additional funds necessary to complete preclinical and clinical development of any product candidates we may develop;
- the timing of the submission and acceptance of IND applications or comparable foreign applications that allow commencement of future clinical trials for any product candidates we may develop;
- 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, clinical and initial commercial supply;
- the availability of specialty raw materials for use in production of any product candidates we may develop;
- 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, stock-based compensation and severance expense, 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 our research progresses toward clinical studies and we will increase our headcount. 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.

Loss on lease termination

Loss on termination of lease consists of expenses recognized for the impairments of right-of-use asset and construction in progress, write-off of tenant improvement allowance receivable, accretion and other lease-related expenses in connection with the termination of the Seyon Lease.

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, 2024 and 2023

The following table summarizes our results of operations for the three and nine months ended September 30, 2024 and 2023:

(in thousands)	Three Months Ended September 30,		Change 2024 vs 2023	Nine Months Ended September 30,		Change 2024 vs 2023
	2024	2023		2024	2023	
Revenue:						
Collaboration revenue	\$ 7,554	\$ 2,146	\$ 5,408	15,704	3,026	\$ 12,678
Operating expenses:						
Research and development	15,088	21,862	(6,774)	\$ 45,811	\$ 65,694	(19,883)
General and administrative	9,181	11,641	(2,460)	29,124	37,474	(8,350)
Loss on lease termination	1,169	—	1,169	59,596	—	59,596
Total operating expenses	25,438	33,503	(8,065)	134,531	103,168	31,363
Loss from operations	(17,884)	(31,357)	13,473	(118,827)	(100,142)	(18,685)
Other income:						
Other income and interest income, net	2,571	3,091	(520)	8,541	8,716	(175)
Net loss	\$ (15,313)	\$ (28,266)	\$ 12,953	\$ (110,286)	\$ (91,426)	\$ (18,860)

Collaboration revenue

During the three months ended September 30, 2024, we recognized \$7.6 million in collaboration revenue, compared to \$2.1 million for the three months ended September 30, 2023. During the nine months ended September 30, 2024, we recognized \$15.7 million in collaboration revenue, compared to \$3.0 million for the nine months ended September 30, 2023. The increase in collaboration revenue during the three and nine months ended September 30, 2024 was due to increased reimbursable activity under our Collaboration Agreement with Moderna, which commenced in the second quarter of 2023 and additional revenue related to the change in estimate, during the three months of September 30, 2024, due to revisions in the research plan resulting in a reduction in the estimated timeline of future research services and total estimated costs. The change in estimate resulted in an increase of \$3.8 million in collaboration revenue and a decrease in net loss per share, basic and diluted, of \$0.06 for both the three and nine months ended September 30, 2024. For additional information on our collaboration with Moderna and the accounting thereunder, refer to Note 4, Collaboration and License Agreement.

Research and development expenses

The following table summarizes our research and development expenses for the three and nine months ended September 30, 2024 and 2023:

(in thousands)	Three Months Ended September 30,		Change 2024 vs 2023	Nine Months Ended September 30,		Change 2024 vs 2023
	2024	2023		2024	2023	
Personnel-related	\$ 5,096	\$ 6,940	\$ (1,844)	\$ 13,867	\$ 21,032	\$ (7,165)
Facilities-related	3,230	3,434	(204)	10,240	10,326	(86)
Preclinical and manufacturing	2,904	4,905	(2,001)	10,527	15,111	(4,584)
Stock-based compensation	1,520	2,874	(1,354)	4,452	8,608	(4,156)
Lab supplies	1,120	1,463	(343)	2,777	3,318	(541)
Consulting and professional services	415	486	(71)	1,220	1,504	(284)
License fees	17	44	(27)	203	915	(712)
Other	786	1,716	(930)	2,525	4,880	(2,355)
Total research and development expenses	\$ 15,088	\$ 21,862	\$ (6,774)	\$ 45,811	\$ 65,694	\$ (19,883)

Research and development expenses were \$15.1 million for the three months ended September 30, 2024, compared to \$21.9 million for the three months ended September 30, 2023. The decreases in personnel-related costs of \$1.8 million and stock-based compensation costs of \$1.4 million were driven primarily by decreased headcount as a result of the RIF in November 2023. The decrease in preclinical and manufacturing costs of \$2.0 million was driven primarily by decreased preclinical activities.

Research and development expenses were \$45.8 million for the nine months ended September 30, 2024, compared to \$65.7 million for the nine months ended September 30, 2023. The decreases in personnel-related costs of \$7.2 million and stock-based compensation costs of \$4.2 million were driven primarily by decreased headcount as a result of the RIF in November 2023. The decrease in preclinical and manufacturing costs of \$4.6 million was driven primarily by decreased preclinical activities.

General and administrative expenses

The following table summarizes our general and administrative expenses for the three and nine months ended September 30, 2024 and 2023:

(in thousands)	Three Months Ended September 30,			Change 2024 vs 2023	Nine Months Ended September 30,			Change 2024 vs 2023
	2024	2023			2024	2023		
Personnel-related	\$ 2,966	\$ 3,646	\$ (680)	\$ 9,076	\$ 12,195	\$ (3,119)		
Stock-based compensation	2,232	3,130	(898)	6,997	9,685	(2,688)		
Facilities-related	1,396	2,495	(1,099)	5,024	7,443	(2,419)		
Professional and consultant fees	2,260	1,794	466	6,951	6,409	542		
Other	327	576	(249)	1,076	1,742	(666)		
Total general and administrative expenses	\$ 9,181	\$ 11,641	\$ (2,460)	\$ 29,124	\$ 37,474	\$ (8,350)		

General and administrative expenses were \$9.2 million for the three months ended September 30, 2024, compared to \$11.6 million for the three months ended September 30, 2023. The decrease in personnel-related costs of \$0.7 million and stock-based compensation costs of \$0.9 million were driven primarily by decreased headcount as a result of the RIF in November 2023. The decrease in facilities-related costs of \$1.1 million was driven primarily by the termination of the Seyon Lease in January 2024.

General and administrative expenses were \$29.1 million for the nine months ended September 30, 2024, compared to \$37.5 million for the nine months ended September 30, 2023. The decrease in personnel-related costs of \$3.1 million and stock-based compensation costs of \$2.7 million were driven primarily by decreased headcount as a result of the RIF in November 2023. The decrease in facilities-related costs of \$2.4 million was driven primarily by the termination of the Seyon Lease in January 2024.

Loss on lease termination

During the three and nine months ended September 30, 2024, we recognized a non-cash charge of \$1.2 million and \$59.6 million, respectively, in connection with the termination of the Seyon Lease. The non-cash charge recognized during the nine months ended September 30, 2024 included a material impairment loss comprised of \$45.8 million in right-of-use asset, \$6.2 million in construction in progress, a write-off of \$3.9 million in tenant improvement allowance receivable from the landlord and \$3.7 million in accretion and other lease-related expenses.

Other income and interest income, net

Other income and interest income, net for the three and nine months ended September 30, 2024 was \$2.6 million and \$8.5 million as compared to \$3.1 million and \$8.7 million for the three and nine months ended September 30, 2023. The decrease in other income and interest income, net during the three and nine months ended September 30, 2024 was primarily due to a decrease in interest yields on 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 platforms. 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. During the nine months ended September 30, 2024, we have recognized \$15.7 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 collaboration revenue under our collaboration with Moderna. In August 2024, 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 \$237.0 million. Upon our entry into this sales agreement, we terminated our prior at-the-market program pursuant to a prior sales agreement that we entered into in August 2021. At the time of termination, approximately \$237.3 million out of an aggregate of \$250.0 million remained unsold under the prior sales agreement. As of November 6, 2024, the issuance date of the condensed consolidated financial statements, we have not issued and sold any shares of our common stock pursuant to the August 2024 sales agreement. 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, 2024, we had cash, cash equivalents, and marketable securities of \$199.8 million.

Cash flows

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

(in thousands)	Nine Months Ended September 30,	
	2024	2023
Net cash used in operating activities	\$ (72,779)	\$ (27,767)
Net cash provided by (used in) investing activities	24,245	(7,784)
Net cash provided by financing activities	106	35,740
Net (decrease) increase in cash, cash equivalents and restricted cash	\$ (48,428)	\$ 189

Operating activities

During the nine months ended September 30, 2024, operating activities used \$72.8 million of cash, primarily resulting from our net loss of \$110.3 million and the net changes in our operating assets and liabilities of \$30.6 million and offset by the net of non-cash charges of \$68.1 million. Net changes in our operating assets and liabilities for the nine months ended September 30, 2024 consisted of a \$2.2 million increase in collaboration receivable, \$12.2 million decrease of deferred revenue, a \$2.8 million decrease in operating lease right-of-use assets, a \$0.7 million decrease in prepaid expenses and other current assets, a \$2.8 million increase of other noncurrent assets, a \$9.6 million decrease of accrued expense and other current liabilities and accounts payable and a \$7.2 million decrease in operating lease liability.

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.

Changes in accrued expenses and other current liabilities and accounts payable were generally due to payments of accrued employee bonus and severance benefits and the timing of vendor invoicing and payments.

Investing activities

During the nine months ended September 30, 2024, net cash provided by investing activities was \$24.2 million, primarily due to \$142.0 million in maturities of marketable securities offset by purchases of marketable securities of \$115.9 million and property and equipment of \$2.0 million during the period. 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.

Financing activities

During the nine months ended September 30, 2024, net cash provided by financing activities was \$0.1 million, consisting of \$0.3 million in proceeds from employee stock option exercises and sales of common stock in connection to the 2020 Employee Stock Purchase Plan, offset \$0.2 million in payments for repurchases of common stock for employee tax withholdings. 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.

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 costs and scope of the continued development of our non-viral genetic medicine platforms;
- the identification of additional research programs and product candidates;
- 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 scope, progress, costs and results of preclinical and clinical development for any product candidates we may develop;
- our research and development costs and the receipt of 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 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 of operational, financial and management information systems and associated personnel;

- the extent to which our previously announced RIF achieves the anticipated cost savings;
- 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 the second half of 2027. 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 and revenue recognition. 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.

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 \$178.2 million as of September 30, 2024. During the nine months ended September 30, 2024, 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, 2024. 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, 2024, 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, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

For a discussion of material legal proceedings, see Note 10. Commitments and Contingencies—Legal Proceedings in Part I, Item 1, Financial Statements of our Quarterly Report on Form 10-Q for the quarter ended March 31, 2024..

Item 1A. Risk Factors.

In addition to the other information set forth in this Quarterly Report, you should carefully consider the factors discussed in Part I, Item 1A Risk Factors in our Annual Report, which could materially affect our business, financial condition, or future results.

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 (each 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
10.1	Sales Agreement, dated as of August 7, 2024, by and between the registrant and TD Securities (USA) LLC (incorporated by reference to Exhibit 1.2 to the registrant's Registration Statement on Form S-3, File No. 333-281335, filed August 7, 2024)
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 6, 2024

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

Date: November 6, 2024

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

**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 13a-15(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 6, 2024

/s/ Geoff McDonough

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

**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 13a-15(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 6, 2024

/s/ Matthew Norkunas

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

**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, 2024 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 6, 2024

**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, 2024 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 6, 2024
