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

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2023

OR

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

For the transition period from ___ to ___

Commission File Number 001-37553

REGENXBIO Inc.
(Exact Name of Registrant as Specified in its Charter)

Delaware	**47-1851754**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
9804 Medical Center Drive Rockville, MD	**20850**
(Address of principal executive offices)	**(Zip Code)**

(240) 552-8181
(Registrant's telephone number, including area code)

Not Applicable
(Former name, former address and former fiscal year, if changed since last report)

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, par value $0.0001 per share	RGNX	The Nasdaq Global Select Market

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

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

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

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

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

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

As of July 27, 2023, there were 43,960,108 shares of the registrant's common stock, par value $0.0001 per share, issued and outstanding.

REGENXBIO INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED JUNE 30, 2023

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INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as “anticipate,” “assume,” “believe,” “continue,” “could,” “design,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “objective,” “plan,” “position,” “potential,” “predict,” “project,” “seek,” “should,” or “will,” “would” or by variations of such words or by similar expressions. We have based these forward-looking statements on our current expectations, estimates and assumptions and analyses in light of our experience and our perception of historical trends, current conditions and expected future developments, as well as other factors we believe are appropriate under the circumstances. However, whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, uncertainties, assumptions and other important factors, including, but not limited to:

- our ability to establish and maintain development partnerships, including our collaboration with AbbVie to develop and commercialize ABBV-RGX-314 (formerly RGX-314);
- our ability to obtain and maintain regulatory approval of our product candidates and the labeling for any approved products;
- the timing of enrollment, commencement and completion and the success of our clinical trials, including the timing and commencement of our AFFINITY DUCHENNE™ clinical trial;
- the timing of commencement and completion and the success of preclinical studies conducted by us and our development partners;
- the timely development and launch of new products;
- the scope, progress, expansion and costs of developing and commercializing our product candidates;
- our ability to obtain, maintain and enforce intellectual property protection for our product candidates and technology, and defend against third-party intellectual property-related claims;
- our expectations regarding the development and commercialization of product candidates currently being developed by third parties that utilize our technology;
- our anticipated growth strategies;
- our expectations regarding competition;
- the anticipated trends and challenges in our business and the market in which we operate;
- our ability to attract or retain key personnel;
- the size and growth of the potential markets for our product candidates and the ability to serve those markets;
- the rate and degree of market acceptance of any of our products that are approved;
- our expectations regarding our expenses and revenue;
- our expectations regarding the outcome of legal proceedings;
- our expectations regarding regulatory developments in the United States and foreign countries; and
- changes in the financial markets and banking system that may affect the availability and terms on which we may obtain financing and our ability to accurately predict how long our existing cash resources will be sufficient to fund our anticipated operating expenses.

You should carefully read the factors discussed in the sections titled “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” and the factors discussed elsewhere in this Quarterly Report on Form 10-Q, our Annual Report on Form 10-K for the year ended December 31, 2022 and in our other filings with the U.S. Securities and Exchange Commission (the SEC) for additional discussion of the risks, uncertainties, assumptions and other important factors that could cause our actual results or developments to differ materially and adversely from those projected in the forward-looking statements. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on us or our businesses or operations. Such statements are not guarantees of future performance, and actual results or developments may differ materially and adversely from those projected in the forward-looking statements. These forward-looking statements speak only as of the date of this report. Except as required by law, we disclaim any duty to update any forward-looking statements, whether as a result of new information, future events or otherwise.

Available Information

Our principal offices are located at 9804 Medical Center Drive, Rockville, MD 20850, and our telephone number is (240) 552-8181. Our website address is www.regenxbio.com. The information contained in, or that can be accessed through, our website is not a part of, or incorporated by reference in, this Quarterly Report on Form 10-Q. We file annual, quarterly, and current reports, proxy statements, and other documents with the SEC under the Exchange Act. You may obtain any reports, proxy and information statements, and other information that we file electronically with the SEC at www.sec.gov.

You also may view and download copies of our SEC filings free of charge at our website as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The information contained on, or that can be accessed through, our website will not be deemed to be incorporated by reference in, and is not considered part of, this Quarterly Report on Form 10-Q. Investors should also note that we use our website, as well as SEC filings, press releases, public conference calls and webcasts, to announce financial information and other material developments regarding our business. We use these channels, as well as any social media channels listed on our website, to communicate with investors and members of the public about our business. It is possible that the information that we post on our social media channels could be deemed material information. Therefore, we encourage investors, the media and others interested in our company to review the information that we post on our social media channels.

As used in this Quarterly Report on Form 10-Q, the terms “REGENXBIO,” “we,” “us,” “our” or the “Company” mean REGENXBIO Inc. and its subsidiaries, on a consolidated basis, unless the context indicates otherwise.

AAVIATE, ALTITUDE, ATMOSPHERE, NAV, REGENXBIO and the REGENXBIO logos are our registered trademarks. Any other trademarks appearing in this Quarterly Report on Form 10-Q are the property of their respective holders.

PART I – FINANCIAL INFORMATION

Item 1. Financial Statements.

REGENXBIO INC.
CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands, except per share data)

	June 30, 2023	December 31, 2022
Assets		
Current assets		
Cash and cash equivalents	$ 68,600	$ 96,952
Marketable securities	251,482	267,690
Accounts receivable	21,380	28,082
Prepaid expenses	15,112	13,900
Other current assets	22,235	9,352
Total current assets	378,809	415,976
Marketable securities	95,302	200,560
Accounts receivable, net	1,143	1,504
Property and equipment, net	138,680	141,685
Operating lease right-of-use assets	62,436	65,116
Restricted cash	2,255	2,030
Other assets	3,833	6,397
Total assets	$ 682,458	$ 833,268
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 11,639	$ 27,213
Accrued expenses and other current liabilities	50,517	46,794
Deferred revenue	448	1,829
Operating lease liabilities	6,326	5,997
Liability related to sale of future royalties	48,963	48,601
Total current liabilities	117,893	130,434
Operating lease liabilities	85,254	88,802
Liability related to sale of future royalties	67,377	89,005
Other liabilities	6,079	8,832
Total liabilities	276,603	317,073
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, no shares issued and outstanding at June 30, 2023 and December 31, 2022	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at June 30, 2023 and December 31, 2022; 43,621 and 43,299 shares issued and outstanding at June 30, 2023 and December 31, 2022, respectively	4	4
Additional paid-in capital	996,239	973,145
Accumulated other comprehensive loss	(10,098)	(15,401)
Accumulated deficit	(580,290)	(441,553)
Total stockholders' equity	405,855	516,195
Total liabilities and stockholders' equity	$ 682,458	$ 833,268

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited)
(in thousands, except per share data)

	Three Months Ended June 30, 2023	Three Months Ended June 30, 2022	Six Months Ended June 30, 2023	Six Months Ended June 30, 2022
Revenues				
License and royalty revenue	$ 19,977	$ 32,649	$ 39,115	$ 54,867
Total revenues	19,977	32,649	39,115	54,867
Operating Expenses				
Cost of revenues	9,475	12,951	13,587	28,668
Research and development	59,886	61,008	118,402	116,635
General and administrative	23,698	20,832	46,332	43,150
Other operating expenses	26	391	59	474
Total operating expenses	93,085	95,182	178,380	188,927
Loss from operations	(73,108)	(62,533)	(139,265)	(134,060)
Other Income (Expense)				
Interest income from licensing	40	153	110	247
Investment income	2,127	1,061	4,293	1,860
Interest expense	(1,120)	(6,860)	(3,875)	(12,990)
Total other income (expense)	1,047	(5,646)	528	(10,883)
Loss before income taxes	(72,061)	(68,179)	(138,737)	(144,943)
Income Tax Benefit	—	—	—	41
Net loss	$ (72,061)	$ (68,179)	$ (138,737)	$ (144,902)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	1,524	(2,813)	5,303	(12,194)
Total other comprehensive income (loss)	1,524	(2,813)	5,303	(12,194)
Comprehensive loss	$ (70,537)	$ (70,992)	$ (133,434)	$ (157,096)
Net loss per share, basic and diluted	$ (1.66)	$ (1.58)	$ (3.19)	$ (3.37)
Weighted-average common shares outstanding, basic and diluted	43,531	43,111	43,491	43,028

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited)
(in thousands)

	Three Months Ended June 30, 2023					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at March 31, 2023	43,465	$ 4	$ 984,986	$ (11,622)	$ (508,229)	$ 465,139
Vesting of restricted stock units, net of tax	28	—	—	—	—	—
Exercise of stock options, net of tax	128	—	749	—	—	749
Stock-based compensation expense	—	—	10,504	—	—	10,504
Unrealized gain on available-for-sale securities, net	—	—	—	1,524	—	1,524
Net loss	—	—	—	—	(72,061)	(72,061)
Balances at June 30, 2023	43,621	$ 4	$ 996,239	$ (10,098)	$ (580,290)	$ 405,855

	Three Months Ended June 30, 2022					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at March 31, 2022	42,982	$ 4	$ 939,570	$ (11,950)	$ (237,955)	$ 689,669
Vesting of restricted stock units, net of tax	3	—	—	—	—	—
Exercise of stock options, net of tax	186	—	1,507	—	—	1,507
Stock-based compensation expense	—	—	10,335	—	—	10,335
Unrealized loss on available-for-sale securities, net	—	—	—	(2,813)	—	(2,813)
Net loss	—	—	—	—	(68,179)	(68,179)
Balances at June 30, 2022	43,171	$ 4	$ 951,412	$ (14,763)	$ (306,134)	$ 630,519

	Six Months Ended June 30, 2023					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2022	43,299	$ 4	$ 973,145	$ (15,401)	$ (441,553)	$ 516,195
Vesting of restricted stock units, net of tax	127	—	(419)	—	—	(419)
Exercise of stock options, net of tax	165	—	1,220	—	—	1,220
Issuance of common stock under employee stock purchase plan	30	—	583	—	—	583
Stock-based compensation expense	—	—	21,710	—	—	21,710
Unrealized gain on available-for-sale securities, net	—	—	—	5,303	—	5,303
Net loss	—	—	—	—	(138,737)	(138,737)
Balances at June 30, 2023	43,621	$ 4	$ 996,239	$ (10,098)	$ (580,290)	$ 405,855

	Six Months Ended June 30, 2022					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2021	42,831	$ 4	$ 928,095	$ (2,569)	$ (161,232)	$ 764,298
Vesting of restricted stock units, net of tax	55	—	(284)	—	—	(284)
Exercise of stock options, net of tax	263	—	1,844	—	—	1,844
Issuance of common stock under employee stock purchase plan	22	—	622	—	—	622
Stock-based compensation expense	—	—	21,135	—	—	21,135
Unrealized loss on available-for-sale securities, net	—	—	—	(12,194)	—	(12,194)
Net loss	—	—	—	—	(144,902)	(144,902)
Balances at June 30, 2022	43,171	$ 4	$ 951,412	$ (14,763)	$ (306,134)	$ 630,519

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

REGENXBIO INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited)
(in thousands)

	Six Months Ended June 30,	
	2023	**2022**
Cash flows from operating activities		
Net loss	$ (138,737)	$ (144,902)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	21,710	21,135
Depreciation and amortization	8,613	5,234
Net amortization of premiums on marketable debt securities	1,062	2,678
Net loss on investments	—	72
Imputed interest income from licensing	(110)	(247)
Non-cash interest expense	(1,236)	874
Other non-cash adjustments	(55)	308
Changes in operating assets and liabilities		
Accounts receivable	7,108	(5,028)
Prepaid expenses	(1,212)	2,507
Other current assets	(12,846)	1,435
Operating lease right-of-use assets	2,806	2,017
Other assets	2,564	523
Accounts payable	(14,108)	9,527
Accrued expenses and other current liabilities	3,501	(25,593)
Deferred revenue	(1,212)	3,472
Operating lease liabilities	(3,345)	547
Other liabilities	(2,743)	7,781
Net cash used in operating activities	(128,240)	(117,660)
Cash flows from investing activities		
Purchases of marketable debt securities	(49,795)	(158,360)
Maturities of marketable debt securities	175,502	86,892
Purchases of property and equipment	(7,395)	(20,204)
Net cash provided by (used in) investing activities	118,312	(91,672)
Cash flows from financing activities		
Proceeds from exercise of stock options	1,220	1,844
Taxes paid related to net settlement of stock-based awards	(419)	(284)
Proceeds from issuance of common stock under employee stock purchase plan	583	622
Repayments under liability related to sale of future royalties, net of imputed interest	(19,583)	(16,685)
Net cash used in financing activities	(18,199)	(14,503)
Net decrease in cash and cash equivalents and restricted cash	(28,127)	(223,835)
Cash and cash equivalents and restricted cash		
Beginning of period	98,982	347,239
End of period	$ 70,855	$ 123,404

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

REGENXBIO INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS
(unaudited)

1. Nature of Business

REGENXBIO Inc. (the Company) is a clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. The Company has developed a broad pipeline of gene therapy product candidates using its proprietary adeno-associated virus (AAV) gene delivery platform (NAV Technology Platform), which consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8 and AAV9. In addition to its internal product development efforts, the Company also selectively licenses the NAV® Technology Platform to other leading biotechnology and pharmaceutical companies (NAV Technology Licensees). As of June 30, 2023, the NAV Technology Platform was being applied by NAV Technology Licensees in one commercially available product, Zolgensma®, and in the preclinical and clinical development of a number of other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development and commercialization of certain product candidates. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

The Company has incurred cumulative losses since inception and as of June 30, 2023, had generated an accumulated deficit of $580.3 million. The Company's ability to transition to recurring profitability is dependent upon achieving a level of revenues adequate to support its cost structure, which depends heavily on the successful development, approval and commercialization of its product candidates. The Company may never achieve recurring profitability, and unless and until it does, the Company will continue to need to raise additional capital, to the extent possible. As of June 30, 2023, the Company had cash, cash equivalents and marketable securities of $415.4 million, which management believes is sufficient to fund operations for at least the next 12 months from the date these consolidated financial statements were issued.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying consolidated financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). The interim unaudited consolidated financial statements have been prepared on the same basis as the annual audited consolidated financial statements as of and for the year ended December 31, 2022 included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2022, which was filed with the SEC on February 28, 2023. Certain information and footnote disclosures required by GAAP, which are normally included in the Company’s annual consolidated financial statements, have been omitted pursuant to SEC rules and regulations for interim reporting. In the opinion of management, the accompanying consolidated financial statements reflect all adjustments, which are normal and recurring in nature, necessary for fair financial statement presentation.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year, any other interim periods, or any future year or period. These interim consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2022, and the notes thereto, which are included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2022.

The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of the consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. Management bases its estimates on historical experience and various other factors that it believes are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities, and other reported amounts, that are not readily apparent from other sources. Actual results may differ materially from these estimates. Significant estimates are used in the following areas, among others: license and royalty revenue, the allowance for credit losses, accrued research and development expenses and other accrued liabilities, stock-based compensation expense, interest expense under the liability related to the sale of future royalties, income taxes and the fair value of financial instruments.

Reclassifications

Certain amounts reported in prior periods have been reclassified to conform to current period financial statement presentation. These reclassifications are not material and have no effect on previously reported financial position, results of operations and cash flows.

Restricted Cash

Restricted cash includes money market mutual funds and other deposits used to collateralize irrevocable letters of credit required under the Company's lease agreements and certain other agreements with third parties. The following table provides a reconciliation of cash and cash equivalents and restricted cash as reported on the consolidated balance sheets to the total of these amounts as reported at the end of the period in the consolidated statements of cash flows (in thousands):

	As of June 30,	
	2023	2022
Cash and cash equivalents	$ 68,600	$ 121,374
Restricted cash	2,255	2,030
Total cash and cash equivalents and restricted cash	$ 70,855	$ 123,404

Accounts Receivable

Accounts receivable primarily consist of consideration due to the Company resulting from its license agreements with customers. Accounts receivable include amounts invoiced to licensees as well as rights to consideration which have not yet been invoiced, including unbilled royalties, and for which payment is conditional solely upon the passage of time. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to the Company and any accounts receivable from the licensee which are not contractually payable to the Company are charged off as a reduction of license revenue in the period of the termination. Accounts receivable which are not expected to be received by the Company within 12 months from the reporting date are stated net of a discount to present value and recorded as non-current assets on the consolidated balance sheets. The present value discount is recognized as a reduction of revenue in the period in which the accounts receivable are initially recorded and is accreted as interest income from licensing over the term of the receivables.

Accounts receivable are stated net of an allowance for credit losses, if deemed necessary based on the Company's evaluation of collectability and potential credit losses. Management assesses the collectability of its accounts receivable using the specific identification of account balances, and considers the credit quality and financial condition of its significant customers, historical information regarding credit losses and the Company's evaluation of current and expected future economic conditions. If necessary, an allowance for credit losses is recorded against accounts receivable such that the carrying value of accounts receivable reflects the net amount expected to be collected. Accounts receivable balances are written off against the allowance for credit losses when the potential for collectability is considered remote. Please refer to Note 8 for further information regarding the allowance for credit losses related to accounts receivable.

Fair Value of Financial Instruments

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. Accounting Standards Codification (ASC) 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement. The fair values of the Company's Level 2 instruments are based on quoted market prices or broker or dealer quotations for similar assets. These investments are initially valued at the transaction price and subsequently valued utilizing third party pricing providers or other market observable data. Please refer to Note 4 for further information on the fair value measurement of the Company's financial instruments.

Net Loss Per Share

Basic net loss per share is calculated by dividing net loss applicable to common stockholders by the weighted-average common shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting the weighted-average common shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net loss per share until the contingency has been fully met. For purposes of the diluted net loss per share calculation, common stock equivalents are excluded from the calculation of diluted net loss per share if their effect would be anti-dilutive.

3. Marketable Securities

The following tables present a summary of the Company's marketable securities, which consist solely of available-for-sale debt securities (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
June 30, 2023				
U.S. government and agency securities	$ 109,284	$ 2	$ (2,614)	$ 106,672
Certificates of deposit	7,553	—	(199)	7,354
Corporate bonds	239,185	—	(6,427)	232,758
	$ 356,022	$ 2	$ (9,240)	$ 346,784

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2022				
U.S. government and agency securities	$ 134,485	$ —	$ (3,492)	$ 130,993
Certificates of deposit	7,555	1	(248)	7,308
Corporate bonds	340,752	—	(10,803)	329,949
	$ 482,792	$ 1	$ (14,543)	$ 468,250

As of June 30, 2023 and December 31, 2022, no available-for-sale debt securities had remaining maturities greater than three years. The amortized cost of marketable debt securities is adjusted for amortization of premiums and accretion of discounts to maturity, or to the earliest call date for callable debt securities purchased at a premium.

As of June 30, 2023 and December 31, 2022, the balance in accumulated other comprehensive loss consisted solely of unrealized gains and losses on available-for-sale debt securities, net of reclassification adjustments for realized gains and losses and income tax effects. The Company uses the aggregate portfolio approach to release the tax effects of unrealized gains and losses on available-for-sale debt securities in accumulated other comprehensive loss. Realized gains and losses from the sale or maturity of marketable securities are based on the specific identification method and are included in results of operations as investment income. Unrealized gain (loss) on available-for-sale securities, net, as presented in the consolidated statements of operations and comprehensive loss consisted of the following (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Unrealized gain (loss) before reclassifications	$ 1,524	$ (2,885)	$ 5,303	$ (12,266)
Realized losses (gains) reclassified to investment income	—	72	—	72
Unrealized gain (loss) on available-for-sale securities, net	$ 1,524	$ (2,813)	$ 5,303	$ (12,194)

The following tables present the fair values and unrealized losses of available-for-sale debt securities held by the Company in an unrealized loss position for less than 12 months and 12 months or greater (in thousands):

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
June 30, 2023						
U.S. government and agency securities	$ 29,923	$ (236)	$ 64,013	$ (2,378)	$ 93,936	$ (2,614)
Certificates of deposit	2,885	(39)	4,225	(160)	7,110	(199)
Corporate bonds	23,933	(164)	208,825	(6,263)	232,758	(6,427)
	$ 56,741	$ (439)	$ 277,063	$ (8,801)	$ 333,804	$ (9,240)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2022						
U.S. government and agency securities	$ 91,498	$ (2,014)	$ 38,495	$ (1,478)	$ 129,993	$ (3,492)
Certificates of deposit	4,484	(144)	2,087	(104)	6,571	(248)
Corporate bonds	106,707	(3,866)	223,242	(6,937)	329,949	(10,803)
	$ 202,689	$ (6,024)	$ 263,824	$ (8,519)	$ 466,513	$ (14,543)

As of June 30, 2023, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 113 investment grade security positions. The Company has the intent and ability to hold such securities until recovery, and based on the credit quality of the issuers and low severity of each unrealized loss position relative to its amortized cost basis, the Company did not identify any credit losses associated with its available-for-sale debt securities. The Company did not record an allowance for credit losses on its available-for-sale debt securities as of June 30, 2023 or December 31, 2022. The Company did not recognize any impairment or credit losses on available-for-sale debt securities during the three and six months ended June 30, 2023 and 2022.

4. Fair Value of Financial Instruments

Financial instruments reported at fair value on a recurring basis include cash equivalents and marketable securities. The following tables present the fair value of cash equivalents and marketable securities in accordance with the hierarchy discussed in Note 2 (in thousands):

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
June 30, 2023				
Cash equivalents:				
Money market mutual funds	$ —	$ 41,886	$ —	$ 41,886
Total cash equivalents	—	41,886	—	41,886
Marketable securities:				
U.S. government and agency securities	—	106,672	—	106,672
Certificates of deposit	—	7,354	—	7,354
Corporate bonds	—	232,758	—	232,758
Total marketable securities	—	346,784	—	346,784
Total cash equivalents and marketable securities	$ —	$ 388,670	$ —	$ 388,670

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2022				
Cash equivalents:				
Money market mutual funds	$ —	$ 58,611	$ —	$ 58,611
Corporate bonds	—	993	—	993
Total cash equivalents	—	59,604	—	59,604
Marketable securities:				
U.S. government and agency securities	—	130,993	—	130,993
Certificates of deposit	—	7,308	—	7,308
Corporate bonds	—	329,949	—	329,949
Total marketable securities	—	468,250	—	468,250
Total cash equivalents and marketable securities	$ —	$ 527,854	$ —	$ 527,854

Management estimates that the carrying values of its current accounts receivable, other current assets, accounts payable, accrued expenses and other current liabilities approximate fair value due to the short-term nature of those instruments. Accounts receivable which contain non-current portions and certain non-current payables reported as other liabilities are recorded at their present values using a discount rate that is based on prevailing market rates on the date the amounts were initially recorded. Management does not believe there have been any significant changes in market conditions or credit quality that would cause the discount rates initially used to be materially different from those that would be used as of June 30, 2023 to determine the present value of these instruments. Accordingly, management estimates that the carrying values of its non-current accounts receivable and other liabilities approximate the fair value of those instruments. Management estimates that the carrying value of the liability related to the sale of future royalties approximates fair value. As discussed in Note 6, the carrying value of the liability related to the sale of future royalties is based on the Company's estimate of future royalties expected to be paid by the Company over the life of the arrangement, which are considered Level 3 inputs.

5. Property and Equipment, Net

Property and equipment, net consists of the following (in thousands):

	June 30, 2023	December 31, 2022
Laboratory and manufacturing equipment	$ 74,307	$ 71,801
Computer equipment and software	5,710	4,910
Furniture and fixtures	7,030	6,965
Leasehold improvements	101,633	99,397
Total property and equipment	188,680	183,073
Accumulated depreciation and amortization	(50,000)	(41,388)
Property and equipment, net	$ 138,680	$ 141,685

6. Liability Related to Sale of Future Royalties

In December 2020, the Company entered into a royalty purchase agreement (the Royalty Purchase Agreement) with entities managed by Healthcare Royalty Management, LLC (collectively, HCR). Under the Royalty Purchase Agreement, HCR purchased the Company's rights to a capped amount of Zolgensma royalty payments under the Company's license agreement (the Novartis License) with Novartis Gene Therapies, Inc. (formerly AveXis, Inc.) (Novartis Gene Therapies), including $4.0 million of royalty payments received by the Company in the fourth quarter of 2020 (the Pledged Royalties). In consideration for these rights, HCR paid the Company $200.0 million (the Purchase Price), less $4.0 million representing the payment of the Pledged Royalties to HCR. Beginning upon the effective date of the Royalty Purchase Agreement, Zolgensma royalty payments, up to a specified threshold, shall be paid to HCR, net of upstream royalties payable by the Company to certain licensors in accordance with existing license agreements.

Pursuant to the Royalty Purchase Agreement, the total amount of royalty payments to be received by HCR is subject to an increasing cap (the Cap Amount) equal to (i) $260.0 million applicable for the period from the effective date of the Royalty Purchase Agreement through November 7, 2024, and (ii) $300.0 million applicable for the period from November 8, 2024 through the effective date of termination of the Novartis License. If, on or prior to the defined dates for each Cap Amount, the total amount of royalty payments received by HCR equals or exceeds the Cap Amount applicable to such date, the Royalty Purchase Agreement will automatically terminate and all rights to the Zolgensma royalty payments will revert back to the Company. The Company has no obligation to repay any amounts to HCR if total future Zolgensma royalty payments are not sufficient to achieve the applicable Cap Amount prior to the termination of the Novartis License.

The Company has a call option to repurchase its rights to the purchased royalties from HCR for a repurchase price equal to, as of the option exercise date, $300.0 million minus the total amount of royalty payments received by HCR; provided, however, that with respect to a call option exercised on or before November 7, 2024, in the event that the then applicable Cap Amount minus the total amount of royalty payments received by HCR is less than $1.0 million, the repurchase price shall equal such difference.

The proceeds received from HCR of $196.0 million were recorded as a liability, net of transaction costs of $3.5 million, which is amortized over the estimated life of the arrangement using the effective interest method. In order to determine the amortization of the liability, the Company is required to estimate the total amount of future royalty payments to be received by HCR, subject to the Cap Amount, over the life of the arrangement. The total amount of royalty payments received by HCR under the Royalty Purchase Agreement, less the net proceeds received by the Company of $192.5 million, is recorded as interest expense over the life of the arrangement using the effective interest method. Due to its continuing involvement in the Novartis License, the Company continues to recognize royalty revenue on net sales of Zolgensma and records the royalty payments to HCR as a reduction of the liability when paid. As such payments are made to HCR, the balance of the liability will be effectively repaid over the life of the Royalty Purchase Agreement.

The Company estimates the effective interest rate used to record interest expense under the Royalty Purchase Agreement based on its estimate of future royalty payments to be received by HCR. As of June 30, 2023, the estimated effective interest rate under the Royalty Purchase Agreement was 3.1%. Over the life of the arrangement, the actual effective interest rate will be affected by the amount and timing of the royalty payments received by HCR and changes in the Company’s forecasted royalties. At each reporting date, the Company reassesses its estimate of total future royalty payments to be received by HCR at the applicable Cap Amount, and prospectively adjusts the effective interest rate and amortization of the liability, as necessary.

The following table presents the changes in the liability related to the sale of future royalties under the Royalty Purchase Agreement with HCR (in thousands):

	Liability Related to Sale of Future Royalties
Balance at December 31, 2022	$ 137,606
Zolgensma royalties paid to HCR	(24,694)
Interest expense recognized	3,428
Balance at June 30, 2023	116,340
Current portion of liability related to sale of future royalties	(48,963)
Non-current portion of liability related to sale of future royalties	$ 67,377

7. Commitments and Contingencies

The Trustees of the University of Pennsylvania

In February 2009, the Company entered into a license agreement, which has been amended from time to time (as amended, the Penn License), with The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn) for exclusive, worldwide rights to certain patents owned by Penn underlying the Company’s NAV Technology Platform, as well as exclusive rights to certain data, results and other information. In March 2022, the Company and Penn entered into a letter agreement (the Penn Letter Agreement) pursuant to which the Company will pay to Penn a total of $20.0 million, consisting of (i) $8.0 million paid in April 2022 to satisfy payment of any sublicense fees due or owed in the future under the Penn License as a result of the Company’s collaboration and license agreement with AbbVie Global Enterprises Ltd., and (ii) $12.0 million to satisfy any other past or future obligations of the Company to pay sublicense fees under the Penn License, which is payable in four equal annual installments of $3.0 million beginning in March 2023. The Penn Letter Agreement amended the Penn License to remove the Company’s obligations to pay sublicense fees under the license agreement. The Company remains obligated to pay Penn royalties on net sales of licensed products, milestone fees and reimbursement of certain patent maintenance costs in accordance with the Penn License.

The Company recognized a charge of $9.2 million as cost of revenues upon the execution of Penn Letter Agreement in March 2022, which consisted of $17.3 million representing the present value of the $20.0 million payable under the Penn Letter Agreement, less $8.1 million in sublicense fees previously recognized as expense by the Company in prior periods and accrued as liabilities prior to the effectiveness of the Penn Letter Agreement. The present value discount is accreted as interest expense over the contractual payment period using the effective interest method. In addition to other amounts payable under the Penn License, as of June 30, 2023, the Company had recorded a total of $7.6 million payable under the Penn Letter Agreement, net of present value discount, of which $2.3 million was included in accrued expenses and other current liabilities and $5.3 million was included in other liabilities on the consolidated balance sheet.

8. License and Collaboration Agreements

License and Royalty Revenue

As of June 30, 2023, the Company's NAV Technology Platform was being applied by NAV Technology Licensees in one commercially available product, Zolgensma, and in the development of a number of other licensed products. Additionally, the Company has licensed intellectual property rights to collaborators for the joint development of certain product candidates. Consideration to the Company under its license agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products and (v) other consideration payable upon optional goods and services purchased by licensees. Sublicense fees vary by license and range from a mid-single digit percentage to a low-double digit percentage of license fees received by licensees as a result of sublicenses. Royalties on net sales of commercialized products vary by license and range from a mid-single digit percentage to a low double-digit percentage of net sales by licensees.

License and royalty revenue consisted of the following (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Zolgensma royalties	$ 18,965	$ 28,386	$ 35,091	$ 49,925
Other license and royalty revenue	1,012	4,263	4,024	4,942
Total license and royalty revenue	$ 19,977	$ 32,649	$ 39,115	$ 54,867

Outstanding development milestone payments are evaluated each reporting period and are only included in the transaction price of each license and recognized as license revenue to the extent the milestones are considered probable of achievement. Sales-based milestones are excluded from the transaction price of each license agreement and recognized as royalty revenue in the period of achievement. As of June 30, 2023, the Company's license agreements, excluding additional licenses that could be granted upon the exercise of options by licensees, contained unachieved milestones which could result in aggregate milestone payments to the Company of up to $1.55 billion, including (i) $533.8 million upon the commencement of various stages of clinical trials, (ii) $17.0 million upon the submission of regulatory approval filings, (iii) $123.0 million upon the approval of commercial products by regulatory agencies and (iv) $875.0 million upon the achievement of specified sales targets for licensed products, including milestones payable upon the first commercial sales of licensed products. To the extent the milestone payments are realized by the Company, the Company will be obligated to pay sublicense fees to licensors based on a specified percentage of the fees earned by the Company. The achievement of these milestones is highly dependent on the successful development and commercialization of licensed products and it is at least reasonably possible that some or all of the milestone fees will not be realized by the Company.

Changes in Accounts Receivable, Contract Assets and Deferred Revenue

The following table presents the balances of the Company's net accounts receivable, contract assets and deferred revenue, as well as other information regarding revenue recognized during the periods presented (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Accounts receivable, net, current and non-current:				
Beginning of period	$ 20,161	$ 29,106	$ 29,586	$ 34,701
End of period	$ 22,523	$ 39,534	$ 22,523	$ 39,534
Contract assets:				
Beginning of period	$ 2,000	$ 1,126	$ —	$ 1,074
End of period	$ 2,000	$ —	$ 2,000	$ —
Deferred revenue, current and non-current:				
Beginning of period	$ 1,311	$ 3,333	$ 1,829	$ 3,333
End of period	$ 448	$ 6,636	$ 448	$ 6,636
Revenue recognized during the period from:				
Amounts included in deferred revenue at beginning of period	$ 891	$ —	$ 1,409	$ —
Performance obligations satisfied in previous periods	$ 18,907	$ 28,420	$ 37,040	$ 49,961

Contract assets as of June 30, 2023 are included in other current assets on the consolidated balance sheet. The Company did not record any contract assets as of December 31, 2022.

As of June 30, 2023, the Company had recorded deferred revenue of $0.4 million which represents consideration received or unconditionally due from licensees for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations as of June 30, 2023 consisted of research and development services to be performed by the Company related to licensed products, which will be satisfied as the research and development services are performed. As of June 30, 2023, the aggregate transaction price of the Company's license agreements allocated to performance obligations not yet satisfied, or partially satisfied, was $1.7 million, which is expected to be satisfied over a period of approximately two to three years.

Revenue recognized from performance obligations satisfied in previous periods, as presented in the table above, was primarily attributable to Zolgensma royalties and changes in the transaction prices of the Company's license agreements. Changes in transaction prices were primarily attributable to development milestones achieved or deemed probable of achievement during the periods which were previously not considered probable of achievement, resulting in a cumulative catch-up adjustment to revenue. Revenue recognized during the six months ended June 30, 2023 resulting from performance obligations satisfied in previous periods included $2.0 million in cumulative catch-up adjustments for changes in the probability of achievement of development milestones. There were no cumulative catch-up adjustments to revenue for changes in the probability of achievement of development milestones during the three months ended June 30, 2023 or during the three and six months ended June 30, 2022.

Accounts Receivable, Contract Assets and the Allowance for Credit Losses

Accounts receivable, net consisted of the following (in thousands):

	June 30, 2023	December 31, 2022
Current accounts receivable:		
Billed to customers	$ 393	$ 280
Unbilled Zolgensma royalties	20,303	27,027
Other unbilled	684	775
Allowance for credit losses	—	—
Current accounts receivable, net	21,380	28,082
Non-current accounts receivable:		
Due from Abeona, net of present value discount	4,364	4,152
Other unbilled	1,143	1,504
Allowance for credit losses	(4,364)	(4,152)
Non-current accounts receivable, net	1,143	1,504
Total accounts receivable, net	$ 22,523	$ 29,586

The following table presents the changes in the allowance for credit losses related to accounts receivable and contract assets for the six months ended June 30, 2023 (in thousands):

	Allowance for Credit Losses	
	Accounts Receivable	Contract Assets
Balance at December 31, 2022	$ 4,152	$ —
Changes in present value discount of receivables	212	—
Balance at June 30, 2023	$ 4,364	$ —

The Company's allowance for credit losses as of June 30, 2023 and December 31, 2022 was related solely to accounts receivable from Abeona Therapeutics Inc. (Abeona). Please refer to the section below, "Settlement Agreement with Abeona Therapeutics", for further information regarding amounts due from Abeona and the associated allowance for credit losses. The Company did not record a provision for credit losses for the three and six months ended June 30, 2023 and 2022.

Zolgensma License with Novartis Gene Therapies

In March 2014, the Company entered into an exclusive license agreement (as amended, the Novartis License) with Novartis Gene Therapies (formerly AveXis, Inc.). Under the Novartis License, the Company granted Novartis Gene Therapies an exclusive, worldwide commercial license, with rights to sublicense, to the NAV Technology Platform, as well as other certain rights, for the treatment of spinal muscular atrophy (SMA) in humans by *in vivo* gene therapy. In 2019, Novartis Gene Therapies launched commercial sales of Zolgensma, a licensed product under the Novartis License. In accordance with the Novartis License, the Company recognizes royalty revenue on net sales of Zolgensma.

The Company recognized the following amounts under the Novartis License (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Zolgensma royalties	$ 18,965	$ 28,386	$ 35,091	$ 49,925
Other license revenue	—	31	—	31
Total license and royalty revenue	$ 18,965	$ 28,417	$ 35,091	$ 49,956
Interest income from licensing	$ 7	$ 71	$ 15	$ 76

As of June 30, 2023 and December 31, 2022, the Company had recorded total accounts receivable of $20.5 million and $27.3 million, respectively, from Novartis Gene Therapies under the Novartis License, which consisted primarily of Zolgensma royalties receivable. The Zolgensma royalties receivable recorded as of June 30, 2023 included $10.8 million expected to be paid to HCR in accordance with the Royalty Purchase Agreement discussed in Note 6. The Company recognizes royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by Novartis Gene Therapies, which in certain cases may require the Company to estimate royalty revenue for periods of net sales which have not yet been reported to the Company. Estimated royalties are reconciled to actual amounts reported in subsequent periods, and any differences are recognized as an adjustment to royalty revenue in the period the royalties are reported.

Settlement Agreement with Abeona Therapeutics

In November 2021, the Company entered into a settlement agreement and mutual release with Abeona (the Settlement Agreement) related to claims associated with a license agreement between the parties which was terminated in May 2020. The Settlement Agreement resolved all arbitration and legal proceedings and mutually released each party from any and all claims under the terminated license agreement. Pursuant to the Settlement Agreement, Abeona will pay the Company a total of $30.0 million as follows: (i) $20.0 million which was paid in November 2021, (ii) $5.0 which was paid in November 2022, and (iii) $5.0 million payable on the earlier of the third anniversary of the Settlement Agreement in November 2024 or the closing of a specified type of transaction by Abeona.

As of June 30, 2023 and December 31, 2022, the Company had recorded gross, non-current accounts receivable of $4.4 million and $4.2 million, respectively, from Abeona under the Settlement Agreement. The gross accounts receivable of $4.4 million as of June 30, 2023 consisted of the $5.0 million payment due by November 2024, net of discount to present value. While the Company anticipates taking appropriate measures to enforce the full collection of all amounts due from Abeona under the Settlement Agreement, the Company assessed the collectability of the accounts receivable from Abeona as it relates to credit risk. In performing this assessment, the Company evaluated Abeona's credit profile and financial condition, as well its expectations regarding Abeona's future cash flows and ability to satisfy the contractual obligations of the Settlement Agreement. As a result of its analysis, the Company recorded an allowance for credit losses of $4.4 million and $4.2 million as of June 30, 2023 and December 31, 2022, respectively, related to the non-current accounts receivable due from Abeona. No provision for credit losses was recorded for the three and six months ended June 30, 2023 and 2022 related to the receivable from Abeona. The present value discount of the non-current accounts receivable from Abeona is accreted as interest income from licensing through the contractual due date using the effective interest method. The Company has elected to record increases in the allowance for credit losses associated with the accretion of the present value discount of the receivable as a reduction of the associated interest income, resulting in no interest income recognized during the periods related to the accretion of the present value discount on the non-current receivable from Abeona.

Collaboration Agreements

AbbVie Collaboration and License Agreement

Effective in November 2021, the Company entered into a collaboration and license agreement with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., to jointly develop and commercialize ABBV-RGX-314, the Company's product candidate for the treatment of wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other chronic retinal diseases (the AbbVie Collaboration Agreement).

Pursuant to the AbbVie Collaboration Agreement, the Company granted AbbVie a co-exclusive license to develop and commercialize ABBV-RGX-314 in the United States and an exclusive license to develop and commercialize ABBV-RGX-314 outside the United States. The Company and AbbVie will collaborate to develop ABBV-RGX-314 in the United States, and AbbVie will be responsible for the development of ABBV-RGX-314 in specified markets outside the United States. Through December 31, 2022, the Company was responsible for the development expenses related to certain ongoing clinical trials of ABBV-RGX-314 and the parties shared the additional development expenses related to ABBV-RGX-314. Beginning on January 1, 2023, AbbVie became responsible for the majority of all ABBV-RGX-314 development expenses.

The Company will lead the manufacturing of ABBV-RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead the manufacturing of ABBV-RGX-314 for commercial supply outside the United States. Manufacturing expenses will be allocated between the parties in accordance with the terms of the AbbVie Collaboration Agreement and supply agreements determined in accordance with the agreement. If requested by AbbVie, the Company will manufacture up to a specified portion of ABBV-RGX-314 for commercial supply outside the United States at a price specified in the agreement. AbbVie will lead the commercialization of ABBV-RGX-314 globally, and the Company will participate in U.S. commercialization efforts as provided under a commercialization plan determined in accordance with the agreement. The Company and AbbVie will share equally in the net profits and net losses associated with the commercialization of ABBV-RGX-314 in the United States. Outside the United States, AbbVie will be responsible, at its sole cost, for the commercialization of ABBV-RGX-314.

In consideration for the rights granted under the AbbVie Collaboration Agreement, AbbVie paid the Company an up-front fee of $370.0 million upon the effective date of the agreement in November 2021 and is required to pay to the Company up to $1.38 billion upon the achievement of specified development and sales-based milestones, of which $562.5 million are based on development milestones and $820.0 million are sales-based milestones. AbbVie is also required to pay to the Company tiered royalties on net sales of ABBV-RGX-314 outside the United States at percentages in the mid-teens to low twenties, subject to specified offsets and reductions.

The Company applied the requirements of ASC 606, *Revenue from Contracts with Customers* (Topic 606) to the AbbVie Collaboration Agreement for the units of account in which AbbVie was deemed to be a customer. The Company determined that there is only one material performance obligation under the agreement for the delivery of the intellectual property license to develop and commercialize ABBV-RGX-314 globally. The intellectual property licensed to AbbVie includes the rights to certain patents, data, know-how and other rights developed and owned by the Company, as well as other intellectual property rights exclusively licensed by the Company from various third parties. As of June 30, 2023 and December 31, 2022, the transaction price of the AbbVie Collaboration Agreement was $370.0 million, which consisted solely of the up-front payment received from AbbVie in November 2021. The $370.0 million transaction price was fully recognized as revenue upon the delivery of the license to AbbVie in November 2021. Variable consideration under the AbbVie Collaboration Agreement, which has been excluded from the transaction price, includes $562.5 million in payments for development milestones that have not yet been achieved and were not considered probable of achievement. Additionally, the transaction price excludes sales-based milestone payments of $820.0 million and royalties on net sales of ABBV-RGX-314 outside the United States. Development milestones will be added to the transaction price and recognized as revenue upon achievement, or if deemed probable of achievement. In accordance with the sale- or usage-based royalty exception under Topic 606, royalties on net sales and sales-based milestones will be recognized as revenue in the period the underlying sales occur or milestones are achieved. There were no changes in the transaction price of the AbbVie Collaboration Agreement, and no revenue was recognized, during the three and six months ended June 30, 2023 and 2022.

The Company applied the requirements of ASC 808, *Collaborative Arrangements* (Topic 808) to the AbbVie Collaboration Agreement for the units of account which were deemed to be a collaborative arrangement. Both the Company and AbbVie will perform various activities related to the development, manufacturing and commercialization of ABBV-RGX-314 in the United States. Development costs are shared between the parties in accordance with the terms of the AbbVie Collaboration Agreement, and the parties will share equally in the net profits and losses derived from sales of ABBV-RGX-314 in the United States. The Company accounts for payments to and from AbbVie for the sharing of development and commercialization costs in accordance with its accounting policy for collaborative arrangements. Amounts owed to AbbVie for the Company's share of development costs or commercialization costs incurred by AbbVie are recorded as research and development expense or general and administrative expense, respectively, in the period the costs are incurred. Amounts owed to the Company for AbbVie's share of development costs or commercialization costs incurred by the Company are recorded as a reduction of research and development expense or general and administrative expense, respectively, in the period the costs are incurred. At the end of each reporting period, the Company records a net amount due to or from AbbVie as a result of the cost-sharing arrangement. As of June 30, 2023 and December 31, 2022, the Company had recorded $18.0 million and $6.2 million, respectively, due from AbbVie for net reimbursement of costs incurred for activities performed under AbbVie Collaboration Agreement, which was included in other current assets on the consolidated balance sheets.

The Company recognized the following amounts under the AbbVie Collaboration Agreement (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Net cost reimbursement to (from) AbbVie included in:				
Research and development expense	$ (18,218)	$ (5,212)	$ (36,692)	$ (8,094)
General and administrative expense	251	296	392	204
Total net cost reimbursement to (from) AbbVie	$ (17,967)	$ (4,916)	$ (36,300)	$ (7,890)

9. Stock-based Compensation

Effective January 2023, an additional 1,731,940 shares were authorized for issuance under the 2015 Equity Incentive Plan (the 2015 Plan). As of June 30, 2023, the total number of shares of common stock authorized for issuance under the 2015 Plan and the 2014 Stock Plan (the 2014 Plan) was 17,357,140, of which 2,187,920 remained available for future grants under the 2015 Plan.

Stock-based Compensation Expense

The Company's stock-based compensation expense by award type was as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Stock options	$ 7,416	$ 8,825	$ 15,594	$ 18,092
Restricted stock units	2,852	1,258	5,632	2,511
Employee stock purchase plan	236	252	484	532
	$ 10,504	$ 10,335	$ 21,710	$ 21,135

As of June 30, 2023, the Company had $83.4 million of unrecognized stock-based compensation expense related to stock options, restricted stock units and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 2.6 years.

The Company recorded aggregate stock-based compensation expense in the consolidated statements of operations and comprehensive loss as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Research and development	$ 5,470	$ 5,405	$ 11,540	$ 11,075
General and administrative	5,034	4,930	10,170	10,060
	$ 10,504	$ 10,335	$ 21,710	$ 21,135

Stock Options

The following table summarizes stock option activity under the 2014 Plan and 2015 Plan (in thousands, except per share data):

	Shares	Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (a)
Outstanding at December 31, 2022	7,668	$ 34.43	6.5	$ 15,783
Granted	1,661	$ 21.99		
Exercised	(165)	$ 7.40		
Cancelled or forfeited	(259)	$ 36.73		
Outstanding at June 30, 2023	8,905	$ 32.54	6.7	$ 9,867
Exercisable at June 30, 2023	5,781	$ 34.41	5.5	$ 9,798
Vested and expected to vest at June 30, 2023	8,905	$ 32.54	6.7	$ 9,867

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were in the money at the dates reported.

The weighted-average grant date fair value per share of options granted during the six months ended June 30, 2023 was $13.72. During the six months ended June 30, 2023, the total number of stock options exercised was 164,950, resulting in total proceeds of $1.2 million. The total intrinsic value of options exercised during the six months ended June 30, 2023 was $2.0 million.

Restricted Stock Units

The following table summarizes restricted stock unit activity under the 2015 Plan (in thousands, except per share data):

	Shares	Weighted-average Grant Date Fair Value
Unvested balance at December 31, 2022	613	$ 35.41
Granted	1,005	$ 22.14
Vested	(146)	$ 36.31
Forfeited	(48)	$ 27.37
Unvested balance at June 30, 2023	1,424	$ 26.22

The total intrinsic value of restricted stock units vested during the six months ended June 30, 2023 was $3.2 million.

Employee Stock Purchase Plan

As of June 30, 2023, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 1,426,994, of which 1,091,529 remained available for future issuance. During the six months ended June 30, 2023, 30,223 shares of common stock were issued under the 2015 ESPP.

10. Income Taxes

The Company evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets as of June 30, 2023 and December 31, 2022. Based on the Company's history of operating losses, and other relevant facts and circumstances, the Company concluded that it was more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company provided a full valuation allowance for its net deferred tax assets as of June 30, 2023 and December 31, 2022.

11. Related Party Transactions

FOXKISER LLP

From 2016 until June 2022, the Company was a party to professional services agreements with FOXKISER LLP (FOXKISER), an affiliate of certain stockholders of the Company and an affiliate of a member of the Company's Board of Directors, pursuant to which the Company paid a fixed monthly fee in consideration for certain strategic services provided by FOXKISER. The agreement with FOXKISER was terminated effective June 2022. Expenses incurred under the agreement with FOXKISER were $1.2 million and $2.4 million for the three and six months ended June 30, 2022, respectively, and were recorded as research and development expenses in the consolidated statements of operations and comprehensive loss. The Company did not record any expenses under the agreement with FOXKISER during the three and six months ended June 30, 2023.

12. Net Loss Per Share

Since the Company incurred net losses for the three and six months ended June 30, 2023 and 2022, common stock equivalents were excluded from the calculation of diluted net loss per share for such periods as their effect would be anti-dilutive. Accordingly, basic and diluted net loss per share were the same for such periods. The following potentially dilutive common stock equivalents outstanding at the end of the period were excluded from the computations of weighted-average diluted common shares for the periods indicated as their effects would be anti-dilutive (in thousands):

	Three and Six Months Ended June 30,	
	2023	2022
Stock options issued and outstanding	8,905	7,966
Unvested restricted stock units outstanding	1,424	492
Employee stock purchase plan	73	53
	10,402	8,511

13. Supplemental Disclosures

Other Current Assets

Other current assets consisted of the following (in thousands):

	June 30, 2023	December 31, 2022
Net cost reimbursement due from collaborators	$ 18,035	$ 6,294
License revenue contract assets	2,000	—
Accrued interest on investments	1,600	2,210
Other	600	848
	$ 22,235	$ 9,352

Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following (in thousands):

	June 30, 2023	December 31, 2022
Accrued external research and development expenses	$ 18,433	$ 9,216
Accrued personnel costs	13,827	18,071
Accrued sublicense fees and royalties	12,032	15,768
Accrued external general and administrative expenses	4,799	2,187
Accrued purchases of property and equipment	543	806
Other accrued expenses and current liabilities	883	746
	$ 50,517	$ 46,794

Supplemental Disclosures of Non-cash Investing and Financing Activities

Purchases of property and equipment included in accounts payable and accrued expenses and other current liabilities were $0.8 million as of June 30, 2023, a net decrease of $1.8 million from December 31, 2022, and $2.4 million as of June 30, 2022, a net decrease of $7.7 million from December 31, 2021.

Proceeds due to the Company for sales of non-marketable equity securities included in other current assets as of June 30, 2022 were $0.6 million. No such amounts were recorded as of June 30, 2023.

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

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the financial statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q and with our audited financial statements and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2022, which we filed with the SEC on February 28, 2023. In addition, you should read the "Risk Factors" and "Information Regarding Forward-Looking Statements" sections of this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2022 for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

Overview

We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our investigational gene therapies are designed to deliver functional genes to address genetic defects in cells, enabling the production of therapeutic proteins or antibodies that are intended to impact disease. Through a single administration, gene therapy could potentially alter the course of disease significantly and deliver improved patient outcomes with long-lasting effects.

Overview of Product Candidates

We have developed a broad pipeline of gene therapy programs using our proprietary adeno-associated virus (AAV) gene therapy delivery platform (NAV Technology Platform) to address genetic diseases. Our programs and product candidates are described below:

- **ABBV-RGX-314:** We are developing ABBV-RGX-314 in collaboration with AbbVie as a potential one-time treatment for wet AMD, diabetic retinopathy (DR) and other additional chronic retinal conditions which cause total or partial vision loss. ABBV-RGX-314 is currently being evaluated in nine ongoing clinical trials in the United States and Canada. Such ongoing clinical trials include two pivotal trials, one Phase II bridging study, one Long-term Follow-up study, and a Fellow Eye Treatment study in patients with wet AMD, all utilizing subretinal delivery, as well as two Phase II clinical trials in patients with wet AMD and DR are also ongoing along with two corresponding Long-term Follow-up studies, all utilizing in-office suprachoroidal delivery. ABBV-RGX-314 uses the NAV® AAV8 vector to deliver a gene encoding a therapeutic antibody fragment to inhibit vascular endothelial growth factor (VEGF). We have licensed certain exclusive rights to the SCS Microinjector® from Clearside Biomedical, Inc. (Clearside) to deliver gene therapy treatments to the suprachoroidal space of the eye.

  Enrollment is ongoing in the ATMOSPHERE® and ASCENT™ pivotal trials as well as the Fellow Eye treatment study for the treatment of patients with wet AMD using subretinal delivery. Material from our NAVXpress™ platform process has been incorporated in the pivotal trials and is expected to be produced by us for future commercialization of ABBV-RGX-314. These trials are expected to support global regulatory submissions with the U.S. Food and Drug Agency (FDA) and the European Medicines Agency (EMA) in late 2025 through the first half of 2026.

  Our study evaluating the pharmacodynamics, safety and efficacy of ABBV-RGX-314 in patients with wet AMD using the subretinal delivery approach in a Phase II bridging study using cGMP material produced by our NAVXpress platform process is now fully enrolled. This Phase II pharmacodynamic study is designed to evaluate the same dose levels being used in the two pivotal trials. In July 2023, updated interim data was presented at the American Society of Retina Specialists annual meeting, demonstrating that ABBV-RGX-314 manufactured using our NAVXpress platform process was well tolerated at both dose levels, initial data in the low dose cohort through 6 months exhibited expected protein levels along with stable to improved best corrected visual acuity and central retinal thickness, as well as meaningful reductions in anti-VEGF burden, with most subjects (11/15, 73%) remaining injection-free.

  The AAVIATE® trial, is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of suprachoroidal delivery of ABBV-RGX-314 for the treatment of wet AMD. The ALTITUDE® trial, is a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of ABBV-RGX-314 for the treatment of DR. In July 2023, we presented interim data from the AAVIATE and ALTITUDE trials demonstrating that ABBV-RGX-314 suprachoroidal delivery administered to patients in cohorts at dose level 3 (1.0×10^{12} genome copies per eye) with short-course (seven-week) prophylactic topical steroid eye drops (N=39) resulted in zero reported cases of intraocular inflammation. Time of post-administration follow up ranged from six weeks to six months. We expect to report additional interim data from the ALTITUDE trial at the American Academy of Ophthalmology meeting (November 3-6, 2023). We expect to report additional interim data from the AAVIATE trial, including full six-month results from Cohorts 5 and 6, at the Hawaiian Eye and Retina meeting (January 13-19, 2024).

- **RGX-202:** We are developing RGX-202 as an investigational one-time AAV therapeutic for the treatment of Duchenne muscular dystrophy (Duchenne), using the NAV AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-Terminal (CT) domain as well as a muscle-specific promoter to support a targeted therapy for improved resistance to muscle damage associated with Duchenne.

  AFFINITY DUCHENNE™, is a multicenter, open-label dose evaluation and dose expansion clinical trial to evaluate the safety, tolerability and clinical efficacy of a one-time intravenous (IV) dose of RGX-202 in patients with Duchenne. In July 2023, we reported that two patients have received doses of RGX-202 and that RGX-202 is reported to be well-tolerated in the patients dosed, aged 4 and 10 years, with no reported drug-related serious adverse events. Time of post-administration follow up was 45 days and more than three months. The trial continues to recruit patients (aged 4 to 11 years). The trial is using commercial-scale cGMP manufactured using our NAVXpress platform process. We expect to report additional interim data of the AFFINITY DUCHENNE trial, including longer-term safety and microdystrophin protein expression levels in muscle at three months at the World Muscle Society Congress (October 3-7, 2023).

  The AFFINITY BEYOND™ trial, an observational screening study, is also active and recruiting patients. The primary objective is to evaluate the prevalence of AAV8 antibodies in patients with Duchenne up to 12 years of age. Information collected in this study may be used to identify potential participants for the AFFINITY DUCHENNE trial and potential future trials of RGX-202.

- **RGX-121:** We are developing RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II), a rare disease caused by a deficiency of the *IDS* gene which encodes I2S, an enzyme that is responsible for the breakdown of structures that dispose of waste products inside cells.

  CAMPSIITE™, is a Phase I/II/III multi-center, open-label trial to evaluate the efficacy, safety, tolerability and pharmacodynamics of RGX-121 in patients with MPS II aged 4 months up to 5 years old. We have completed enrollment of 10 MPS II patients to support a Biologics License Application filing in 2024 using the accelerated approval pathway.

  In May 2023, we announced that the FDA granted regenerative medicine advanced therapy (RMAT) designation for RGX-121. RMAT designation is designed to expedite the drug development and review processes for promising new treatments, including gene therapies, and recognizes that the preliminary clinical evidence from RGX-121 indicates its potential to address unmet medical needs for MPS II.

- **RGX-111:** We are developing RGX-111 as an investigational one-time AAV therapeutic for the treatment of Mucopolysaccharidosis Type I (MPS I) also known as Hurler syndrome, a rare disease caused by a deficiency of α-l-iduronidase (IDUA), an enzyme required for the breakdown of structures that dispose of waste products inside cells.

  The Phase I/II clinical trial in patients with MPS I to evaluate the safety, tolerability and pharmacodynamics of RGX-111, as well as the effects of RGX-111 on biomarkers of IDUA activity, neurocognitive development and other outcome measures is fully enrolled with follow-up ongoing. We expect to complete analytical characterization of recently manufactured commercial-scale cGMP material and share additional updates on program plans by the end of 2023.

- **RGX-181:** We are developing RGX-181 for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, a form of Batten disease, caused by mutations in the tripeptidyl peptidase 1 gene.

  Physician investigators in Brazil continue with follow up for the first child with CLN2 disease dosed with RGX-181 under a single-patient investigator-initiated study. We expect investigators to report initial interim data from the single-patient investigator-initiated study, including 6-month results, at the Society for the Study of Inborn Errors of Metabolism Annual Symposium meeting being held August 29 to September 1, 2023.

- **RGX-381:** We are evaluating the safety and tolerability, as well as the effect on retinal anatomic and functional outcomes, of the subretinal delivery of RGX-381 for the treatment of ocular manifestations of CLN2 disease. In August 2023, we announced dosing of the first patient in the Phase I/II trial of RGX-381. Initial data from this trial is expected to be shared in 2024.

- **Exon-Skipping Therapy for Duchenne Muscular Dystrophy:** In July 2023, we announced the development of a potential one-time gene therapy for Duchenne, which is based on a novel exon-skipping construct, targeting exon 53 of the DMD gene. We have data demonstrating that AAV-mediated exon skipping by the expression of antisense ribonucleic acid sequences has the potential to restore high and sustained levels of near full-length dystrophin in Duchenne patients with relevant mutations in the DMD gene. We are initiating Investigational New Drug (IND) application-enabling studies and expect to submit an IND to the FDA in the first half of 2025.

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively license the NAV Technology Platform to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of June 30, 2023, our NAV Technology Platform was being applied in one commercialized product, Zolgensma®, and the preclinical and clinical development of a number of other licensed products. Licensing the NAV Technology Platform allows us to maintain our internal product development focus on our core disease indications and therapeutic areas while still expanding the NAV gene therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform and creating potential additional revenue.

Financial Overview

Revenues

Our revenues to date consist primarily of license and royalty revenue resulting from the licensing of our NAV Technology Platform and other intellectual property rights. We have not generated any revenues from commercial sales of our own products. If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval and adequate labeling, our ability to generate future revenues will be materially compromised.

We license our NAV Technology Platform and other intellectual property rights to other biotechnology and pharmaceutical companies, including collaborators for the joint development and commercialization of our product candidates. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the NAV Technology Platform and other licensed rights. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration from licensees under our license agreements may include: (i) up-front and annual fees, (ii) milestone payments based on the achievement of certain development and sales-based milestones, (iii) sublicense fees, (iv) royalties on sales of licensed products and (v) other consideration payable upon optional goods and services purchased by licensees.

Future license and royalty revenues are dependent on the successful development and commercialization of licensed products, which is uncertain, and revenues may fluctuate significantly from period to period. Additionally, we may never receive consideration in our license agreements that is contemplated on option fees, development and sales-based milestone payments, royalties on sales of licensed products or sublicense fees, given the contingent nature of these payments. Our revenues are concentrated among a low number of licensees and licenses are terminable at the option of the licensee. The termination of our licenses by licensees may materially impact the amount of revenue we recognize in future periods.

Zolgensma Royalties

Royalty revenue to date consists primarily of royalties on net sales of Zolgensma, which is marketed by Novartis Gene Therapies, Inc. (formerly AveXis, Inc.) (Novartis Gene Therapies), a wholly owned subsidiary of Novartis AG (Novartis), for the treatment of spinal muscular atrophy (SMA). Zolgensma is a licensed product under our license agreement with Novartis Gene Therapies for the development and commercialization of treatments for SMA using the NAV Technology Platform.

Collaboration and License Agreement with AbbVie

Effective in November 2021, we entered into a collaboration and license agreement with AbbVie Global Enterprises Ltd. (AbbVie), a subsidiary of AbbVie Inc., to jointly develop and commercialize ABBV-RGX-314 (the AbbVie Collaboration Agreement). The AbbVie Collaboration Agreement may materially impact our future revenues, research and development expenses, other operating expenses and operating cash flows associated with the development and commercialization of ABBV-RGX-314. For additional information regarding the AbbVie Collaboration Agreement, please refer to Note 8, “License and Collaboration Agreements—AbbVie Collaboration and License Agreement” to the accompanying unaudited consolidated financial statements.

Operating Expenses

Our operating expenses consist primarily of cost of revenues, research and development expenses and general and administrative expenses. Personnel costs including salaries, wages, benefits, bonuses and stock-based compensation expense, comprise a significant component of research and development and general and administrative expenses. We allocate indirect expenses associated with our facilities, information technology costs, depreciation and other overhead costs between research and development and general and administrative categories based on employee headcount and the nature of work performed by each employee or using other reasonable allocation methodologies.

Cost of Revenues

Our cost of revenues consists primarily of upstream fees due to our licensors as a result of revenue generated from the licensing of our NAV Technology Platform and other intellectual property rights, including sublicense fees and royalties on net sales of licensed products. Sublicense fees are based on a percentage of license fees received by us from licensees and are recognized in the period that the underlying license revenue is recognized. Royalties are based on a percentage of net sales of licensed products by licensees and are recognized in the period that the underlying sales occur. Future costs of revenues are uncertain due to the nature of our license agreements and significant fluctuations in cost of revenues may occur from period to period.

Research and Development Expense

Our research and development expenses consist primarily of:

- salaries, wages and personnel-related costs, including benefits, travel and stock-based compensation, for our scientific personnel and others performing research and development activities;
- costs related to executing preclinical studies and clinical trials;
- costs related to acquiring, developing and manufacturing materials for preclinical studies and clinical trials;
- fees paid to consultants and other third-parties who support our product candidate development;
- other costs in seeking regulatory approval of our product candidates; and
- direct costs and allocated costs related to laboratories and facilities, depreciation expense, information technology and other overhead.

Up-front fees incurred in obtaining technology licenses for research and development activities, as well as associated milestone payments, are charged to research and development expense as incurred if the technology licensed has no alternative future use.

We expect to continue to incur significant research and development expenses for the foreseeable future as we continue the development of our product candidates and engage in early research and development for prospective product candidates and new technologies. The following table summarizes our research and development expenses incurred during the three and six months ended June 30, 2023 and 2022 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Direct Expenses				
ABBV-RGX-314	$ 5,333	$ 14,604	$ 10,369	$ 22,131
RGX-202	1,568	1,214	6,145	3,707
RGX-121 and RGX-111	7,531	2,799	10,605	6,048
Other product candidates	1,173	779	1,565	1,322
Total direct expenses	15,605	19,396	28,684	33,208
Unallocated Expenses				
Platform and new technologies	11,556	13,026	22,905	26,068
Personnel-related	23,943	22,221	49,417	45,592
Facilities and depreciation expense	7,273	5,088	14,350	9,618
Other unallocated	1,509	1,277	3,046	2,149
Total unallocated expenses	44,281	41,612	89,718	83,427
Total research and development	$ 59,886	$ 61,008	$ 118,402	$ 116,635

Direct expenses related to the development of ABBV-RGX-314 include $18.2 million and $36.7 million for the three and six months ended June 30, 2023, respectively, and $5.2 million and $8.1 million for the three and six months ended June 30, 2022, respectively, in net cost reimbursement from AbbVie under our eye care collaboration which were recorded as a reduction of research and development expenses during the periods. Net cost reimbursement from AbbVie includes reimbursement of personnel and overhead costs attributable to the development of ABBV-RGX-314, the underlying costs of which are reported as unallocated expenses in the table above. We typically utilize our employee and infrastructure resources across our development programs. In general, we do not allocate personnel and other internal costs, such as facilities and other overhead costs, to specific product candidates or development programs. Platform and new technologies include direct costs not identifiable with a specific lead product candidate, including costs associated with our research and development platform used across programs, process development, manufacturing analytics and early research and development for prospective product candidates and new technologies.

General and Administrative Expense

Our general and administrative expenses consist primarily of salaries, wages and personnel-related costs, including benefits, travel and stock-based compensation, for employees performing functions other than research and development. This includes certain personnel in executive, commercial, corporate development, finance, legal, human resources, information technology, facilities and administrative support functions. Additionally, general and administrative expenses include facility-related and overhead costs not otherwise allocated to research and development expense, professional fees for accounting, legal, commercial and other advisory services, expenses associated with obtaining and maintaining patents, insurance costs, costs of our information systems and other general corporate activities. We expect that our general and administrative expenses will continue to increase as we continue to develop, and potentially commercialize, our product candidates.

Other Income (Expense)

Interest Income from Licensing

In accordance with our revenue recognition policy, interest income from licensing consists of imputed interest recognized from significant financing components identified in our license agreements with NAV Technology Licensees as well as interest income accrued on unpaid balances due from licensees.

Investment Income

Investment income consists of interest income earned and gains and losses realized from our cash equivalents, marketable securities and non-marketable equity securities, as well as unrealized gains and losses on marketable equity securities. Cash equivalents are comprised of money market mutual funds and highly liquid debt securities with original maturities of 90 days or less at acquisition. Marketable securities are comprised of available-for-sale debt securities and equity securities.

Interest Expense

Interest expense consists primarily of interest imputed on the liability related to the sale of future Zolgensma royalties to entities managed by Healthcare Royalty Management, LLC (collectively, HCR). Interest expense is recognized using the effective interest method, based on our estimate of total royalty payments expected to be received by HCR under the royalty purchase agreement. For further information regarding the royalty purchase agreement with HCR, please refer to Note 6, “Liability Related to Sale of Future Royalties” to the accompanying unaudited consolidated financial statements.

Critical Accounting Policies and Estimates

This Management’s Discussion and Analysis of Financial Condition and Results of Operations is based on our consolidated financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States of America (GAAP). The preparation of financial statements in conformity with GAAP requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities, and other reported amounts, that are not readily apparent from other sources. Actual results may differ materially from these estimates under different assumptions or conditions.

Our significant accounting policies are fully described in Note 2 to the accompanying unaudited consolidated financial statements and in Note 2 to our audited consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2022. There have been no significant changes in our critical accounting policies and estimates since December 31, 2022.

Results of Operations

Our consolidated results of operations were as follows (in thousands):

	Three Months Ended June 30,			Six Months Ended June 30,		
	2023	2022	Change	2023	2022	Change
Revenues						
License and royalty revenue	$ 19,977	$ 32,649	$ (12,672)	$ 39,115	$ 54,867	$ (15,752)
Total revenues	19,977	32,649	(12,672)	39,115	54,867	(15,752)
Operating Expenses						
Cost of revenues	9,475	12,951	(3,476)	13,587	28,668	(15,081)
Research and development	59,886	61,008	(1,122)	118,402	116,635	1,767
General and administrative	23,698	20,832	2,866	46,332	43,150	3,182
Other operating expenses	26	391	(365)	59	474	(415)
Total operating expenses	93,085	95,182	(2,097)	178,380	188,927	(10,547)
Loss from operations	(73,108)	(62,533)	(10,575)	(139,265)	(134,060)	(5,205)
Other Income (Expense)						
Interest income from licensing	40	153	(113)	110	247	(137)
Investment income	2,127	1,061	1,066	4,293	1,860	2,433
Interest expense	(1,120)	(6,860)	5,740	(3,875)	(12,990)	9,115
Total other income (expense)	1,047	(5,646)	6,693	528	(10,883)	11,411
Loss before income taxes	(72,061)	(68,179)	(3,882)	(138,737)	(144,943)	6,206
Income Tax Benefit	—	—	—	—	41	(41)
Net loss	$ (72,061)	$ (68,179)	$ (3,882)	$ (138,737)	$ (144,902)	$ 6,165

Comparison of the Three Months Ended June 30, 2023 and 2022

License and Royalty Revenue. License and royalty revenue decreased by $12.7 million, from $32.6 million for the three months ended June 30, 2022 to $20.0 million for the three months ended June 30, 2023. The decrease was primarily attributable to Zolgensma royalty revenues, which decreased by $9.4 million, from $28.4 million for the second quarter of 2022 to $19.0 million for the second quarter of 2023. As reported by Novartis, sales of Zolgensma for the second quarter of 2023 decreased by 18% (USD) as compared to the second quarter of 2022, primarily declining in the United States as a result of fewer prevalent patients, and in Europe mainly due to price mix and other one-time events in the second quarter of 2022. Per Novartis, the number of patients treated globally remained relatively stable.

Research and Development Expense. Research and development expenses decreased by $1.1 million, from $61.0 million for the three months ended June 30, 2022 to $59.9 million for the three months ended June 30, 2023. The decrease was primarily attributable to the following:

- a decrease of $2.0 million in manufacturing expenses and other costs of clinical supply for our lead product candidates, largely driven by a decrease in expenses for ABBV-RGX-314 clinical supply;
- a decrease of $1.7 million in costs associated with clinical trial and regulatory activities, largely driven by a decrease in clinical trial expenses for ABBV-RGX-314 as a result of an increase in net development cost reimbursement from AbbVie, and partially offset by an increase in clinical trial expenses for our other lead product candidates; and
- a decrease of $1.2 million in costs associated with preclinical activities and other early stage research and development.

The decrease in research and development expenses was partially offset by the following:

- an increase of $1.9 million in costs for laboratories and facilities used by research and development personnel, including a $1.8 million increase in depreciation expense allocated to research and development functions, largely driven by the activation of our cGMP facility in mid-2022; and
- an increase of $1.7 million in personnel-related costs as a result of increased headcount of research and development personnel, including a $0.1 million increase in stock-based compensation expense, largely driven by the commencement of in-house manufacturing of clinical supply in mid-2022.

The decrease in research and development expenses for ABBV-RGX-314 was largely driven by a shift in the development cost sharing arrangement under our collaboration with AbbVie. In accordance with the AbbVie Collaboration Agreement, through December 31, 2022, we were responsible for development expenses related to certain ongoing clinical trials of ABBV-RGX-314 and the remaining ABBV-RGX-314 development expenses were shared with AbbVie. Beginning in 2023, AbbVie became responsible for the majority of all ABBV-RGX-314 development expenses.

General and Administrative Expense. General and administrative expenses increased by $2.9 million, from $20.8 million for the three months ended June 30, 2022 to $23.7 million for the three months ended June 30, 2023. The increase was primarily attributable to expenses for professional services, including legal and other corporate advisory services, and other corporate overhead costs.

Interest Expense. Interest expense decreased by $5.7 million, from $6.9 million for the three months ended June 30, 2022 to $1.1 million for the three months ended June 30, 2023. The decrease was primarily attributable to a lower balance in our liability related to the sale of future royalties resulting from Zolgensma royalties paid to HCR, as well as changes in the effective interest rate of the liability resulting from changes in the estimated royalties forecasted to be paid to HCR over the life of the royalty purchase agreement.

Comparison of the Six Months Ended June 30, 2023 and 2022

License and Royalty Revenue. License and royalty revenue decreased by $15.8 million, from $54.9 million for the six months ended June 30, 2022 to $39.1 million for the six months ended June 30, 2023. The decrease was primarily attributable to Zolgensma royalty revenues, which decreased by $14.8 million, from $49.9 million for the first half of 2022 to $35.1 million for the first half of 2023. As reported by Novartis, sales of Zolgensma for the first half of 2023 decreased by 16% (USD) as compared to the first half of 2022, primarily declining in the United States as a result of fewer prevalent patients, and in Europe mainly due to price mix and other one-time events. Per Novartis, the number of patients treated globally remained relatively stable.

Cost of Revenues. Cost of revenues decreased by $15.1 million, from $28.7 million for the six months ended June 30, 2022 to $13.6 million for the six months ended June 30, 2023. The decrease was primarily attributable to a non-recurring charge of $9.2 million recognized in the first quarter of 2022 related to the amendment of our license agreement with The Trustees of the University of Pennsylvania (Penn) to buy out our obligation to pay sublicense fees to Penn under the license agreement. The remaining decrease in cost of revenues in the first half of 2023 was primarily attributable to a reduction in upstream royalties payable to licensors on net sales of Zolgensma during the period.

Research and Development Expense. Research and development expenses increased by $1.8 million, from $116.6 million for the six months ended June 30, 2022 to $118.4 million for the six months ended June 30, 2023. The increase was primarily attributable to the following:

- an increase of $5.6 million in costs for laboratories and facilities used by research and development personnel, including a $3.4 million increase in depreciation expense allocated to research and development functions, largely driven by the activation of our cGMP facility in mid-2022; and
- an increase of $3.8 million in personnel-related costs as a result of increased headcount of research and development personnel, including a $0.5 million increase in stock-based compensation expense, largely driven by the commencement of in-house manufacturing of clinical supply in mid-2022.

The increase in research and development expenses was partially offset by the following:

- a decrease of $4.8 million in costs associated with clinical trial and regulatory activities, largely driven by a decrease in clinical trial expenses for ABBV-RGX-314 as a result of an increase in net development cost reimbursement from AbbVie, and partially offset by an increase in clinical trial expenses for our other lead product candidates;
- a decrease of $2.2 million in costs associated with preclinical activities and other early stage research and development; and
- a decrease of $1.2 million in manufacturing expenses and other costs of clinical supply for our lead product candidates, largely driven by a decrease in expenses for ABBV-RGX-314 clinical supply, and partially offset by an increase in clinical supply costs for our other lead product candidates.

The decrease in research and development expenses for ABBV-RGX-314 was largely driven by a shift in the development cost sharing arrangement under our collaboration with AbbVie. In accordance with the AbbVie Collaboration Agreement, through December 31, 2022, we were responsible for development expenses related to certain ongoing clinical trials of ABBV-RGX-314 and the remaining ABBV-RGX-314 development expenses were shared with AbbVie. Beginning in 2023, AbbVie became responsible for the majority of all ABBV-RGX-314 development expenses.

General and Administrative Expense. General and administrative expenses increased by $3.2 million, from $43.2 million for the six months ended June 30, 2022 to $46.3 million for the six months ended June 30, 2023. The increase was primarily attributable to expenses for professional services, including legal and other corporate advisory services, facilities costs and other corporate overhead costs.

Interest Expense. Interest expense decreased by $9.1 million, from $13.0 million for the six months ended June 30, 2022 to $3.9 million for the six months ended June 30, 2023. The decrease was primarily attributable to a lower balance in our liability related to the sale of future royalties resulting from Zolgensma royalties paid to HCR, as well as changes in the effective interest rate of the liability resulting from changes in the estimated royalties forecasted to be paid to HCR over the life of the royalty purchase agreement.

Liquidity and Capital Resources

Sources of Liquidity

As of June 30, 2023, we had cash, cash equivalents and marketable securities of $415.4 million, which were primarily derived from the sale of our common stock, license and royalty revenue and the monetization of our Zolgensma royalty stream. We expect that our cash, cash equivalents and marketable securities as of June 30, 2023, will enable us to fund our operating expenses and capital expenditure requirements, and are sufficient to meet our financial commitments and obligations, for at least the next 12 months from the date of this report, based on our current business plan.

We intend to devote the majority of our current capital to clinical development, seeking regulatory approval of our product candidates and, if approved, commercialization of our product candidates, as well as additional capital expenditures needed to support these activities. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the total amount of operating expenditures and capital outlays necessary to complete the development of our product candidates. Additionally, our estimates are based on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect.

Cash Flows

Our consolidated cash flows were as follows (in thousands):

	Six Months Ended June 30,	
	2023	2022
Net cash used in operating activities	$ (128,240)	$ (117,660)
Net cash provided by (used in) investing activities	118,312	(91,672)
Net cash used in financing activities	(18,199)	(14,503)
Net decrease in cash and cash equivalents and restricted cash	$ (28,127)	$ (223,835)

Cash Flows from Operating Activities

Our net cash used in operating activities for the six months ended June 30, 2023 increased by $10.6 million from the six months ended June 30, 2022. We expect to continue to incur regular net cash outflows from operations for the foreseeable future as we continue the development and advancement of our product candidates and other research programs.

For the six months ended June 30, 2023, our net cash used in operating activities of $128.2 million consisted of a net loss of $138.7 million and unfavorable changes in operating assets and liabilities of $19.5 million, offset by adjustments for non-cash items of $30.0 million. The changes in operating assets and liabilities include a net decrease in total accounts payable and accrued expenses and other current liabilities of $10.6 million, which was driven primarily by the timing of invoices from suppliers and associated payments made by the Company as of the end of the period, and an increase in total prepaid expenses and other current assets of $14.1 million driven primarily by an increase in amounts due from AbbVie for net cost reimbursement under our ABBV-RGX-314 collaboration. The unfavorable changes in operating assets and liabilities were partially offset by a decrease in accounts receivable of $7.1 million, which was driven primarily by a reduction in Zolgensma royalties receivable. Other changes in operating assets and liabilities occurred in the normal course of business as a result of changes in operating working capital. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $21.7 million and depreciation and amortization expense of $8.6 million.

For the six months ended June 30, 2022, our net cash used in operating activities of $117.7 million consisted of a net loss of $144.9 million and unfavorable changes in operating assets and liabilities of $2.8 million, offset by adjustments for non-cash items of $30.1 million. The changes in operating assets and liabilities include a net decrease in total accounts payable and accrued expenses and other current liabilities of $16.1 million, which was driven primarily by decreases in accrued personnel costs, accrued sublicense fees and royalties, and income taxes payable, and an increase in accounts receivable of $5.0 million driven primarily by license fees billed in the second quarter of 2022 which were receivable at the end of the period. The unfavorable changes in operating assets and liabilities were partially offset by an increase in other liabilities of $7.8 million, which was driven primarily by a long-term liability recorded during the period related to the amendment of our license agreement with Penn. Other changes in operating assets and liabilities occurred in the normal course of business as a result of changes in operating working capital. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $21.1 million and depreciation and amortization expense of $5.2 million.

Cash Flows from Investing Activities

For the six months ended June 30, 2023, our net cash provided by investing activities consisted of $175.5 million in maturities of marketable debt securities, offset by $49.8 million to purchase marketable debt securities and $7.4 million to purchase property and equipment.

For the six months ended June 30, 2022, our net cash used in investing activities consisted of $158.4 million to purchase marketable debt securities and $20.2 million to purchase property and equipment, offset by $86.9 million in maturities of marketable debt securities.

Cash Flows from Financing Activities

For the six months ended June 30, 2023, our net cash used in financing activities primarily consisted of $19.6 million of Zolgensma royalties paid, net of imputed interest, under our royalty purchase agreement with HCR, and was partially offset by $1.8 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

For the six months ended June 30, 2022, our net cash used in financing activities primarily consisted of $16.7 million of Zolgensma royalties paid, net of imputed interest, under our royalty purchase agreement with HCR, and was partially offset by $2.5 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

Additional Capital Requirements

Our material capital requirements from known contractual and other obligations primarily relate to vendor service contracts and purchase commitments, in-license agreements, operating lease agreements and our Zolgensma royalty purchase agreement with HCR. Our material commitments and obligations are further described in Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” of our Annual Report on Form 10-K for the year ended December 31, 2022, and in the notes to the audited consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2022. Other than the changes described in the notes to the unaudited consolidated financial statements accompanying this Quarterly Report on Form 10-Q, including Note 7, “Commitments and Contingencies,” there have been no material changes to our commitments and obligations since December 31, 2022.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $580.3 million as of June 30, 2023. Our transition to recurring profitability is dependent upon achieving a level of revenues adequate to support our cost structure, which depends heavily on the successful development, approval and commercialization of our product candidates. We do not expect to achieve such revenues, and expect to continue to incur losses, for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase for the foreseeable future as we continue the development of, and seek regulatory approval for, our product candidates. Subject to obtaining regulatory approval for our product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. Additionally, we expect to continue to incur capital expenditures associated with building out additional laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

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

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;
- the results of our preclinical studies for our product candidates and any subsequent clinical trials;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates;
- the costs associated with building out additional laboratory and manufacturing capacity;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our products, should any of our product candidates receive marketing approval;
- revenue received from commercial sales of Zolgensma and the timing and amount of Zolgensma royalties paid to HCR under our royalty purchase agreement;
- revenue received from other commercial sales of our licensees' and collaborators' products, should any of their product candidates receive marketing approval, and other revenue received under our licensing agreements and collaborations;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements or collaborations remaining in effect, including the AbbVie Collaboration Agreement;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestones or royalty payments under our licensing agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform licensing is dependent in part on the clinical and commercial success of our licensing partners, including the commercialization of Zolgensma, and on maintaining our license agreements with our licensor partners, including GlaxoSmithKline LLC and Penn. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

The issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

Off-Balance Sheet Arrangements

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

Item 3. Quantitative and Qualitative Disclosures about Market Risk.

For information regarding market risk, refer to Item 7A, "Quantitative and Qualitative Disclosures About Market Risk," included in our Annual Report on Form 10-K for the year ended December 31, 2022. There have been no material changes to our exposure to market risk during the six months ended June 30, 2023.

Item 4. Controls and Procedures.

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2023. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under 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.

Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of June 30, 2023, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the quarter ended June 30, 2023 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls

Control systems, no matter how well conceived and operated, are designed to provide a reasonable, but not an absolute, level of assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. Because of the inherent limitations in any control system, misstatements due to error or fraud may occur and not be detected.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we are party to various lawsuits, claims or other legal proceedings that arise in the normal course of our business. We do not believe that we are currently party to any pending legal actions that could reasonably be expected to have a material adverse effect on our business, financial condition, results of operations or cash flows.

Item 1A. Risk Factors.

Our material risk factors are disclosed in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2022. There have been no material changes from the risk factors previously disclosed in such filing.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

On May 10, 2023, a Rule 10b5-1 trading arrangement adopted on May 10, 2022 by Stephen Pakola, M.D., our EVP, Chief Medical Officer, which was intended to satisfy the affirmative defense of Rule 10b5-1(c) (the "Rule 10b5-1 Plan"), expired pursuant to its terms. In the aggregate 3,138 shares of our common stock were sold under the Rule 10b5-1 Plan prior to its expiration.

Other than as described above, during the three months ended June 30, 2023, none of our directors or Section 16 reporting officers adopted or terminated any Rule 10b5-1 trading arrangement or non-Rule 10b5-1 trading arrangement (as such terms are defined in Item 408 of the SEC's Regulation S-K).

Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference Form	Exhibit Number	Filing Date	Filed or Furnished Herewith
3.1	Restated Certificate of Incorporation	8-K	3.1	6/7/21	
3.2	Amended and Restated Bylaws	8-K	3.2	9/22/15	
31.1	Certification of the Chief Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
31.2	Certification of the Chief Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
32.1	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350				X
101	The following materials from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended June 30, 2023 formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (ii) Consolidated Statements of Operations and Comprehensive Loss (iii) Consolidated Statements of Stockholders' Equity (iv) Consolidated Statements of Cash Flows (v) Notes to Consolidated Financial Statements				X
104	The cover page from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended June 30, 2023 formatted in Inline XBRL (included in Exhibit 101)				

The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the SEC and is not to be incorporated by reference into any filing of REGENXBIO Inc. under the Securities Act or the Exchange Act, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

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.

	REGENXBIO Inc.
Dated: August 2, 2023	/s/ Kenneth T. Mills
	Kenneth T. Mills President and Chief Executive Officer (Principal Executive Officer)
Dated: August 2, 2023	/s/ Vittal Vasista
	Vittal Vasista Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)

EXHIBIT 31.1

CERTIFICATION

I, Kenneth T. Mills, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of REGENXBIO Inc.;

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.

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

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

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

Date: August 2, 2023

/s/ Kenneth T. Mills

Kenneth T. Mills
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION

I, Vittal Vasista, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of REGENXBIO Inc.;

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.

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

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

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

Date: August 2, 2023	/s/ Vittal Vasista
	Vittal Vasista **Chief Financial Officer** **(Principal Financial and Accounting Officer)**

EXHIBIT 32.1

CERTIFICATION

In connection with the Quarterly Report of REGENXBIO Inc. (the "Registrant") on Form 10-Q for the quarter ended June 30, 2023 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Kenneth T. Mills, President, Chief Executive Officer and Director of the Registrant, and Vittal Vasista, Chief Financial Officer of the Registrant, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to their respective knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Registrant.

Date: August 2, 2023	/s/ Kenneth T. Mills
	Kenneth T. Mills
	President and Chief Executive Officer
	(Principal Executive Officer)
Date: August 2, 2023	/s/ Vittal Vasista
	Vittal Vasista
	Chief Financial Officer
	(Principal Financial and Accounting Officer)

This certification is made solely for the purposes of 18 U.S.C. Section 1350, subject to the knowledge standard contained therein, and not for any other purpose. A signed original of this written statement required by Section 906 has been provided to the Registrant and will be retained by the Registrant and furnished to the United States Securities and Exchange Commission or its staff upon request.

This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933 or the Securities Exchange Act of 1934 (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.