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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 March 31, 2022

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**	
(Address of principal executive offices)	**20850** **(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 April 28, 2022, there were 43,133,927 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 MARCH 31, 2022

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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 “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “assume,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would” or by variations of such words or by similar expressions. We have based these forward-looking statements on our current expectations 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 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;
- the impact of the COVID-19 pandemic on our business, operations and preclinical and clinical development timelines and plans;
- 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
- 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 elsewhere in this Quarterly Report on Form 10-Q, our Annual Report on Form 10-K for the year ended December 31, 2021 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, 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)

	March 31, 2022	December 31, 2021
Assets		
Current assets		
Cash and cash equivalents	$ 191,873	$ 345,209
Marketable securities	202,261	112,230
Accounts receivable, net	27,022	32,439
Prepaid expenses	18,542	18,752
Other current assets	7,179	10,196
Total current assets	446,877	518,826
Marketable securities	370,659	391,907
Accounts receivable, net	2,084	2,262
Property and equipment, net	135,264	131,547
Operating lease right-of-use assets	59,925	60,904
Restricted cash	2,030	2,030
Other assets	8,529	6,428
Total assets	$ 1,025,368	$ 1,113,904
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 24,708	$ 11,387
Accrued expenses and other current liabilities	48,423	76,111
Deferred revenue	3,333	3,333
Operating lease liabilities	2,121	1,752
Liability related to sale of future royalties	41,352	37,889
Total current liabilities	119,937	130,472
Operating lease liabilities	85,568	84,929
Liability related to sale of future royalties	122,514	133,460
Other liabilities	7,680	745
Total liabilities	335,699	349,606
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, no shares issued and outstanding at March 31, 2022 and December 31, 2021	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at March 31, 2022 and December 31, 2021; 42,982 and 42,831 shares issued and outstanding at March 31, 2022 and December 31, 2021, respectively	4	4
Additional paid-in capital	939,570	928,095
Accumulated other comprehensive loss	(11,950)	(2,569)
Accumulated deficit	(237,955)	(161,232)
Total stockholders' equity	689,669	764,298
Total liabilities and stockholders' equity	$ 1,025,368	$ 1,113,904

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 March 31,	
	2022	2021
Revenues		
License and royalty revenue	$ 22,218	$ 18,884
Total revenues	22,218	18,884
Operating Expenses		
Cost of revenues	15,717	4,851
Research and development	55,627	39,722
General and administrative	22,318	17,838
Credit losses and other	83	515
Total operating expenses	93,745	62,926
Loss from operations	(71,527)	(44,042)
Other Income (Expense)		
Interest income from licensing	94	29
Investment income	799	580
Interest expense	(6,130)	(6,702)
Total other income (expense)	(5,237)	(6,093)
Loss before income taxes	(76,764)	(50,135)
Income Tax Benefit (Expense)	41	(4)
Net loss	$ (76,723)	$ (50,139)
Other Comprehensive Loss		
Unrealized loss on available-for-sale securities, net	(9,381)	(1,008)
Total other comprehensive loss	(9,381)	(1,008)
Comprehensive loss	$ (86,104)	$ (51,147)
Net loss per share, basic and diluted	$ (1.79)	$ (1.20)
Weighted-average common shares outstanding, basic and diluted	42,944	41,819

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 March 31, 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	52	—	(284)	—	—	(284)
Exercise of stock options, net of tax	76	—	337	—	—	337
Issuance of common stock under employee stock purchase plan	22	—	622	—	—	622
Stock-based compensation expense	—	—	10,800	—	—	10,800
Unrealized loss on available-for-sale securities, net	—	—	—	(9,381)	—	(9,381)
Net loss	—	—	—	—	(76,723)	(76,723)
Balances at March 31, 2022	42,982	$ 4	$ 939,570	$ (11,950)	$ (237,955)	$ 689,669

	Three Months Ended March 31, 2021					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2020	37,476	$ 4	$ 667,181	$ (360)	$ (289,072)	$ 377,753
Issuance of common stock upon public offering, net of transaction costs of $14,194	4,899	—	216,059	—	—	216,059
Exercise of stock options	111	—	1,292	—	—	1,292
Issuance of common stock under employee stock purchase plan	19	—	627	—	—	627
Stock-based compensation expense	—	—	9,920	—	—	9,920
Unrealized loss on available-for-sale securities, net	—	—	—	(1,008)	—	(1,008)
Net loss	—	—	—	—	(50,139)	(50,139)
Balances at March 31, 2021	42,505	$ 4	$ 895,079	$ (1,368)	$ (339,211)	$ 554,504

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

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

	Three Months Ended March 31,	
	2022	**2021**
Cash flows from operating activities		
Net loss	$ (76,723)	$ (50,139)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	10,800	9,920
Depreciation and amortization	2,591	1,929
Provision for credit losses	—	565
Net amortization of premiums on marketable debt securities	1,460	1,288
Net gains on investments	—	(7)
Imputed interest income from licensing	(94)	(29)
Non-cash interest expense	54	3,786
Other non-cash adjustments	(43)	(154)
Changes in operating assets and liabilities		
Accounts receivable	5,689	1,618
Prepaid expenses	210	(3,334)
Other current assets	3,042	(873)
Operating lease right-of-use assets	979	1,232
Other assets	(2,101)	(3,789)
Accounts payable	16,755	278
Accrued expenses and other current liabilities	(26,473)	(10,544)
Operating lease liabilities	1,008	4,244
Other liabilities	6,935	(38)
Net cash used in operating activities	(55,911)	(44,047)
Cash flows from investing activities		
Purchases of marketable debt securities	(129,486)	(233,627)
Maturities of marketable debt securities	49,862	50,465
Purchases of property and equipment	(10,967)	(31,021)
Net cash used in investing activities	(90,591)	(214,183)
Cash flows from financing activities		
Proceeds from exercise of stock options	337	1,292
Taxes paid related to net settlement of stock-based awards	(284)	—
Proceeds from issuance of common stock under employee stock purchase plan	622	627
Proceeds from public offering of common stock, net of underwriting discounts and commissions	—	216,438
Issuance costs for public offering of common stock	—	(251)
Repayments under liability related to sale of future royalties, net of imputed interest	(7,509)	(6,555)
Transaction costs for sale of future royalties	—	(265)
Net cash provided by (used in) financing activities	(6,834)	211,286
Net decrease in cash and cash equivalents and restricted cash	(153,336)	(46,944)
Cash and cash equivalents and restricted cash		
Beginning of period	347,239	339,756
End of period	$ 193,903	$ 292,812

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, AAV9 and AAVrh10. 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 March 31, 2022, 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 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.

As of March 31, 2022, the Company had generated an accumulated deficit of $238.0 million since inception. As the Company has incurred cumulative losses since inception, transition to recurring profitability is dependent upon achieving a level of revenues adequate to support the Company's 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 March 31, 2022, the Company had cash, cash equivalents and marketable securities of $764.8 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, 2021 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2021, which was filed with the SEC on March 1, 2022. 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, 2021, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2021.

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.

The Company is actively monitoring the impact of the COVID-19 pandemic on its business, results of operations and financial condition. The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company's business, results of operations and financial condition in the future remains unknown at this time and will depend on future developments that are highly unpredictable. The most significant estimates affecting the Company's consolidated financial statements that may be impacted by the COVID-19 pandemic are related to the Company's assessment of credit losses on accounts receivable, contract assets and available-for-sale debt securities.

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 other certain agreements. 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):

	Three Months Ended March 31,	
	2022	2021
Cash and cash equivalents	$ 191,873	$ 291,482
Restricted cash	2,030	1,330
Total cash and cash equivalents and restricted cash	$ 193,903	$ 292,812

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.

Correction of Previously Issued Financial Statements

During the quarter ended December 31, 2021, the Company identified an immaterial error in the presentation of payments made under the liability related to the sale of future royalties in the consolidated statements of cash flows for the three months ended March 31, 2021, six months ended June 30, 2021 and nine months ended September 30, 2021. Payments made under the liability related to the sale of future royalties were presented as cash outflows from financing activities in the interim financial statements for these periods. Upon further review, the Company determined that the amount of these payments attributable to imputed interest expense should be presented as cash outflows from operating activities, and only the amount attributable to principal repayments should be presented as cash outflows from financing activities. The amounts previously reported as cash outflows from financing activities which should have been reported as cash outflows from operating activities were $2.9 million for the three months ended March 31, 2021, $9.1 million for the six months ended June 30, 2021 and $15.3 million for the nine months ended September 30, 2021. The Company evaluated the materiality of these errors from both a quantitative and qualitative perspective and concluded that they were immaterial to the aforementioned previously issued interim financial statements taken as a whole. The error in presentation did not have an impact on the financial statements for any periods prior to 2021, and did not have an impact on the previously reported assets, liabilities, stockholders' equity or results of operations for the interim periods ended March 31, 2021, June 30, 2021 and September 30, 2021. Although the Company determined the error was not material to its previously issued interim financial statements for 2021, the Company is revising the previously issued interim financial statements to correct for such error, which revision has been effected in the accompanying consolidated statement of cash flows for the three months ended March 31, 2021, and will be effected in connection with its future filings of Form 10-Q for the interim periods ended June 30, 2022 and September 30, 2022. The accompanying consolidated statement of cash flows for the three months ended March 31, 2021 reflects the as corrected impact of correcting the error, resulting in an increase in net cash used in operating activities and a corresponding increase in net cash provided by financing activities of $2.9 million as compared to the previously issued interim financial statements for the period ended March 31, 2021.

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
March 31, 2022				
U.S. government and agency securities	$ 175,578	$ —	$ (2,041)	$ 173,537
Certificates of deposit	3,664	—	(73)	3,591
Corporate bonds	404,768	6	(8,982)	395,792
	$ 584,010	$ 6	$ (11,096)	$ 572,920

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2021				
U.S. government and agency securities	$ 60,118	$ —	$ (229)	$ 59,889
Certificates of deposit	2,936	2	(11)	2,927
Corporate bonds	442,792	62	(1,533)	441,321
	$ 505,846	$ 64	$ (1,773)	$ 504,137

As of March 31, 2022 and December 31, 2021, 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 March 31, 2022 and December 31, 2021, 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 loss on available-for-sale securities, net, as presented in the statements of operations and comprehensive loss consisted of the following (in thousands):

	Three Months Ended March 31,	
	2022	2021
Unrealized loss before reclassifications	$ (9,381)	$ (1,001)
Realized gains reclassified to investment income	—	(7)
Income tax expense	—	—
Unrealized loss on available-for-sale securities, net	$ (9,381)	$ (1,008)

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
March 31, 2022						
U.S. government and agency securities	$ 173,537	$ (2,041)	$ —	$ —	$ 173,537	$ (2,041)
Certificates of deposit	3,591	(73)	—	—	3,591	(73)
Corporate bonds	318,265	(7,503)	72,721	(1,479)	390,986	(8,982)
	$ 495,393	$ (9,617)	$ 72,721	$ (1,479)	$ 568,114	$ (11,096)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2021						
U.S. government and agency securities	$ 59,889	$ (229)	$ —	$ —	$ 59,889	$ (229)
Certificates of deposit	2,195	(11)	—	—	2,195	(11)
Corporate bonds	385,115	(1,533)	—	—	385,115	(1,533)
	$ 447,199	$ (1,773)	$ —	$ —	$ 447,199	$ (1,773)

As of March 31, 2022, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 132 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 March 31, 2022 or December 31, 2021. The Company did not recognize any impairment or credit losses on available-for-sale debt securities during the three months ended March 31, 2022 and 2021.

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
March 31, 2022				
Cash equivalents:				
Money market mutual funds	$ —	$ 124,116	$ —	$ 124,116
Total cash equivalents	—	124,116	—	124,116
Marketable securities:				
U.S. government and agency securities	—	173,537	—	173,537
Certificates of deposit	—	3,591	—	3,591
Corporate bonds	—	395,792	—	395,792
Total marketable securities	—	572,920	—	572,920
Total cash equivalents and marketable securities	$ —	$ 697,036	$ —	$ 697,036

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2021				
Cash equivalents:				
Money market mutual funds	$ —	$ 236,340	$ —	$ 236,340
Total cash equivalents	—	236,340	—	236,340
Marketable securities:				
U.S. government and agency securities	—	59,889	—	59,889
Certificates of deposit	—	2,927	—	2,927
Corporate bonds	—	441,321	—	441,321
Total marketable securities	—	504,137	—	504,137
Total cash equivalents and marketable securities	$ —	$ 740,477	$ —	$ 740,477

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 are recorded at their present values using a discount rate that is based on prevailing market rates and the credit profile of the licensee on the date the amounts are initially recorded. Certain non-current payables reported as other liabilities on the consolidated balance sheets are recorded at their present values using a discount rate that is based on prevailing market rates and the credit profile of the Company on the date the amounts are 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 March 31, 2022 to determine the present value of these receivables and liabilities. 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 to HCR over the life of the arrangement, which are considered Level 3 inputs.

Non-marketable Equity Securities

Non-marketable equity securities are measured at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. As of March 31, 2022 and December 31, 2021, the Company did not hold any non-marketable equity securities. No remeasurements or impairment losses were recorded on non-marketable equity securities during the three months ended March 31, 2022 and 2021.

5. Property and Equipment, Net

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

	March 31, 2022	December 31, 2021
Laboratory and manufacturing equipment	$ 61,043	$ 56,976
Computer equipment and software	4,545	4,268
Furniture and fixtures	6,839	6,804
Leasehold improvements	94,462	92,535
Total property and equipment	166,889	160,583
Accumulated depreciation and amortization	(31,625)	(29,036)
Property and equipment, net	$ 135,264	$ 131,547

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 agreement, HCR purchased the Company's rights to a capped amount of Zolgensma royalty payments under the Company's license agreement 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 agreement, Zolgensma royalty payments, up to a specified threshold, will 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 under the agreement 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 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 license agreement with Novartis Gene Therapies. 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 license agreement with Novartis Gene Therapies.

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 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 underlying license agreement with Novartis Gene Therapies, 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 March 31, 2022, the estimated effective interest rate under the agreement was 14.8%. 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, 2021	$ 171,349
Zolgensma royalties paid to HCR	(13,586)
Interest expense recognized	6,103
Balance at March 31, 2022	163,866
Current portion of liability related to sale of future royalties	(41,352)
Liability related to sale of future royalties, non-current	$ 122,514

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, with The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn) (the Penn License) 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 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., which is payable within 30 days of the effective date of the Penn Letter Agreement, 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 on first anniversary of the effective date of the Penn Letter Agreement. The Penn Letter Agreement amended the Penn License to remove the Company's obligations to pay sublicense fees under the license agreement. The Company will continue to be 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.

In connection with the execution of the Penn Letter Agreement, the Company's royalty obligations under its March 2009 license agreement with GlaxoSmithKline LLC (GSK) (the GSK License) were assigned by GSK to Penn. Beginning upon the effective date of the Penn Letter Agreement in March 2022, any royalties payable by the Company under the GSK License will be paid to Penn rather than GSK. The Company will continue to be obligated to pay GSK sublicense fees and reimbursement of certain patent maintenance costs in accordance with the GSK License.

The Company recognized a charge of $9.2 million as cost of revenues during the three months ended March 31, 2022 related to the execution of Penn Letter Agreement, 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 March 31, 2022, the Company had recorded a total of $17.4 million payable to Penn under the Penn Letter Agreement, net of present value discount, of which $10.0 million was included in accounts payable and accrued expenses and other current liabilities and $7.4 million was included in other liabilities on the consolidated balance sheet.

8. License and Collaboration Agreements

License and Royalty Revenue

As of March 31, 2022, 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 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 March 31,	
	2022	2021
Zolgensma royalties	$ 21,539	$ 18,263
Other license and royalty revenue	679	621
Total license and royalty revenue	$ 22,218	$ 18,884

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 March 31, 2022, 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 $1.58 billion, including (i) $537.8 million upon the commencement of various stages of clinical trials, (ii) $21.0 million upon the submission of regulatory approval filings, (iii) $141.0 million upon the approval of commercial products by regulatory agencies and (iv) $877.0 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 changes in 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 March 31,	
	2022	2021
Accounts receivable, net, current and non-current:		
Balance, beginning of period	$ 34,701	$ 46,266
Additions	28,057	18,718
Deductions	(33,652)	(21,086)
Balance, end of period	$ 29,106	$ 43,898
Contract assets:		
Balance, beginning of period	$ 1,074	$ 350
Additions	602	649
Deductions	(550)	(350)
Balance, end of period	$ 1,126	$ 649
Deferred revenue, current and non-current:		
Balance, beginning of period	$ 3,333	$ 4,232
Additions	—	—
Deductions	—	(108)
Balance, end of period	$ 3,333	$ 4,124
Revenue recognized during the period from:		
Amounts included in deferred revenue at beginning of period	$ —	$ 108
Performance obligations satisfied in previous periods	$ 21,541	$ 18,651

Additions to accounts receivable during the periods presented consisted primarily of royalties on net sales of Zolgensma and the achievement of development milestones by licensees during the period. Deductions to accounts receivable during the periods presented consisted primarily of amounts collected from licensees and increases in the allowance for credit losses, as discussed further below. Additions to contract assets during the periods presented consisted of development milestones deemed probable of achievement by licensees during the period and revenue recognized from research and development services performed by the Company for which payment by the licensee is not unconditional. Deductions to contract assets during the periods presented consisted of the achievement of development milestones by licensees and billing of the associated milestone payments by the Company. Contract assets as of March 31, 2022 and December 31, 2021 are included in other current assets on the consolidated balance sheets.

As of March 31, 2022, the Company had recorded deferred revenue of $3.3 million which represents consideration received from licensees for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations consisted of (i) options granted to licensees that provide material rights to to acquire additional licenses from the Company, which will be satisfied upon the exercise or expiration of the options and (ii) 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.

Revenue recognized from performance obligations satisfied in previous periods 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.

Accounts Receivable, Contract Assets and the Allowance for Credit Losses

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

	March 31, 2022	December 31, 2021
Current accounts receivable:		
Billed to customers	$ 440	$ 365
Unbilled	26,582	32,074
Allowance for credit losses	—	—
Current accounts receivable, net	27,022	32,439
Non-current accounts receivable:		
Unbilled	5,937	6,020
Allowance for credit losses	(3,853)	(3,758)
Non-current accounts receivable, net	2,084	2,262
Total accounts receivable, net	$ 29,106	$ 34,701

The following table presents the changes in the allowance for credit losses related to accounts receivable and contract assets for the three months ended March 31, 2022 (in thousands):

	Allowance for Credit Losses	
	Accounts Receivable	Contract Assets
Balance at December 31, 2021	$ 3,758	$ —
Provision for credit losses	—	—
Changes in present value discount of receivables	95	—
Write-offs	—	—
Balance at March 31, 2022	$ 3,853	$ —

The Company's allowance for credit losses as of March 31, 2022 and December 31, 2021 was related solely to accounts receivable from Abeona Therapeutics Inc. (Abeona). Please refer to the section below, Abeona Therapeutics Inc., for further information regarding amounts due from Abeona and the associated allowance for credit losses. The Company's provision for credit losses was zero and $0.6 million for the three months ended March 31, 2022 and 2021, respectively, and was related solely to changes in estimates regarding the collectability of the accounts receivable due from Abeona.

Zolgensma License with Novartis Gene Therapies

In March 2014, the Company entered into an exclusive license agreement, as amended, (the March 2014 License) with Novartis Gene Therapies (formerly AveXis, Inc.). Under the March 2014 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. Novartis Gene Therapies launched commercial sales of Zolgensma, a licensed product under the March 2014 License, in the second quarter of 2019, upon which the Company began recognizing royalty revenue on net sales of the licensed product.

The Company recognized the following amounts under the March 2014 License with Novartis Gene Therapies (in thousands):

	Three Months Ended March 31,	
	2022	2021
Zolgensma royalties	$ 21,539	$ 18,263
Total license and royalty revenue	$ 21,539	$ 18,263
Interest income from licensing	$ 5	$ 6

As of March 31, 2022 and December 31, 2021, the Company had recorded total accounts receivable of $21.0 million and $26.6 million, respectively, from Novartis Gene Therapies under the March 2014 License, which consisted primarily of Zolgensma royalties receivable. The Zolgensma royalties receivable recorded as of March 31, 2022 included $14.3 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 royalty revenues are adjusted, as necessary.

Settlement Agreement with Abeona Therapeutics

In November 2018, the Company entered into a license agreement with Abeona (as amended, the November 2018 License) for the treatment of various diseases using the NAV Technology Platform. Pursuant to the November 2018 License, Abeona was required to pay a license fee of $8.0 million to the Company no later than April 1, 2020. Abeona failed to make this payment, and in April 2020, the Company delivered to Abeona a notice of its breach of the license agreement and written demand for payment. Upon expiration of the applicable cure period in May 2020, the license agreement terminated. As a result of the termination, Abeona was required to pay an additional $20.0 million license fee to the Company within 15 days of the termination date, which otherwise would have been due to the Company in November 2020. Unpaid balances due under the November 2018 License accrue interest at 1.5% per month.

In May 2020, after the termination of the November 2018 License, Abeona filed a claim in arbitration alleging that the Company had breached certain responsibilities to communicate with Abeona regarding the Company's prosecution of licensed patents under the November 2018 License. The Company disputed Abeona's claim and filed a counterclaim in arbitration demanding payment of the $28.0 million of unpaid fees from Abeona, plus accrued interest. A binding arbitration was held in March 2021, and the arbitration tribunal issued its ruling in July 2021, which denied Abeona's claim and upheld the Company's counterclaim. The arbitration tribunal's ruling, which was subsequently amended to reflect a minor adjustment in the computation of accrued interest, awarded the Company a total of $33.6 million in damages and accrued interest payable by Abeona.

Subsequent to the arbitration tribunal's ruling in July 2021, Abeona filed an additional claim in a second arbitration to enforce a purported settlement relating to the unpaid fees, which the Company disputed. In November 2021, the Company and Abeona entered into a settlement agreement and mutual release (the Settlement Agreement) to resolve all arbitration and legal proceedings and mutually release each party from any and all claims under the November 2018 License. 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 million payable in November 2022, which is fully secured by an irrevocable standby letter of credit issued to the Company by a reputable U.S. financial institution, 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 March 31, 2022 and December 31, 2021, the Company had recorded gross accounts receivable of $8.9 million and $8.8 million, respectively, from Abeona under the Settlement Agreement. The gross accounts receivable of $8.9 million as of March 31, 2022 consisted of current accounts receivable of $5.0 million for the payment due in November 2022, and non-current accounts receivable of $3.9 million for the present value of the $5.0 million payment due by November 2024. 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 $3.9 million and $3.8 million as of March 31, 2022 and December 31, 2021, respectively, related to the non-current accounts receivable due from Abeona. The Company recorded a provision for credit losses of zero and $0.6 million for the three months ended March 31, 2022 and 2021, respectively, as a result of changes in estimates regarding the allowance during the periods. 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.

AbbVie Collaboration and License Agreement

In September 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 RGX-314, the Company's product candidate for the treatment of wet AMD, DR and other chronic retinal diseases (the AbbVie Collaboration Agreement). The AbbVie Collaboration Agreement became effective in November 2021.

Pursuant to the AbbVie Collaboration Agreement, the Company granted AbbVie a co-exclusive license to develop and commercialize RGX-314 in the United States and an exclusive license to develop and commercialize RGX-314 outside the United States. The Company and AbbVie will collaborate to develop RGX-314 in the United States, and AbbVie will be responsible for the

development of RGX-314 in specified markets outside the United States. Through December 31, 2022, the Company will be responsible for development expenses for certain ongoing trials of RGX-314 and the parties will share additional development expenses related to RGX-314. Beginning on January 1, 2023, AbbVie will be responsible for the majority of all RGX-314 development expenses.

The Company will lead the manufacturing of RGX-314 for clinical development and U.S. commercial supply, and AbbVie will lead the manufacturing of 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 RGX-314 for commercial supply outside the United States at a price specified in the agreement. AbbVie will lead the commercialization of 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 RGX-314 in the United States. Outside the United States, AbbVie will be responsible, at its sole cost, for the commercialization of 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 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 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 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 March 31, 2022 and December 31, 2021, the transaction price of the AbbVie Collaboration Agreement was $370.0 million, which consisted solely of the up-front payment received 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 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 months ended March 31, 2022.

The Company applied the requirements of 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 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 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 March 31, 2022 and December 31, 2021, the Company had recorded $3.1 million and $5.9 million, respectively, due from AbbVie for reimbursement of costs incurred for activities performed under AbbVie Collaboration Agreement, which is 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 March 31, 2022
Net cost reimbursement from AbbVie for collaboration activities included in:	
Research and development expense	$ 2,882
General and administrative expense	92
Total net cost reimbursement from AbbVie	$ 2,974

9. Stock-based Compensation

In January 2022, the Board of Directors authorized an additional 1,713,246 shares to be issued under the 2015 Equity Incentive Plan (the 2015 Plan). As of March 31, 2022, the total number of shares of common stock authorized for issuance under the 2015 Plan and the 2014 Stock Plan (the 2014 Plan) was 15,625,200, of which 2,693,341 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 March 31,	
	2022	2021
Stock options	$ 9,267	$ 9,015
Restricted stock units	1,254	679
Employee stock purchase plan	279	226
	$ 10,800	$ 9,920

As of March 31, 2022, the Company had $88.9 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 2.7 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 March 31,	
	2022	2021
Research and development	$ 5,670	$ 5,031
General and administrative	5,130	4,889
	$ 10,800	$ 9,920

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, 2021	7,126	$ 34.16	6.8	$ 41,128
Granted	1,224	$ 33.93		
Exercised	(80)	$ 4.61		
Cancelled or forfeited	(98)	$ 38.57		
Outstanding at March 31, 2022	8,172	$ 34.37	7.1	$ 40,352
Exercisable at March 31, 2022	5,028	$ 31.52	6.0	$ 39,522
Vested and expected to vest at March 31, 2022	8,172	$ 34.37	7.1	$ 40,352

(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 three months ended March 31, 2022 was $20.37. During the three months ended March 31, 2022, the total number of stock options exercised was 80,123, resulting in total proceeds of $0.4 million. The total intrinsic value of options exercised during the three months ended March 31, 2022 was $1.8 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, 2021	277	$ 43.62
Granted	265	$ 33.92
Vested	(61)	$ 44.94
Forfeited	(10)	$ 38.80
Unvested balance at March 31, 2022	471	$ 38.10

The total intrinsic value of restricted stock units vested during the three months ended March 31, 2022 was $2.0 million. No restricted stock units vested during the three months ended March 31, 2021.

Employee Stock Purchase Plan

In January 2022, the Board of Directors authorized an additional 428,311 shares to be issued under the 2015 ESPP. As of March 31, 2022, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 1,426,994, of which 1,175,112 remained available for future issuance. During the three months ended March 31, 2022, 22,373 shares of common stock were issued under the 2015 ESPP.

10. Income Taxes

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets as of March 31, 2022 and December 31, 2021. 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 March 31, 2022 and December 31, 2021.

11. Related Party Transactions

FOXKISER LLP

Since 2016, the Company has been 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 pays a fixed monthly fee in consideration for certain strategic services provided by FOXKISER. Effective January 2019, the Company entered into a new professional services agreement with FOXKISER with similar terms and conditions as the previous agreements. The agreement was amended effective June 2019 to expand the scope of the services provided and increase the monthly fee. Effective August 2020, the agreement was further amended to extend the term of the agreement by two years through December 2022. The agreement may be terminated by either party with six months' advanced written notice. In December 2021, the Company provided notice of termination of the agreement to FOXKISER, with such termination to be effective in June 2022. Expenses incurred under the agreement with FOXKISER were $1.2 million and $1.2 million for the three months ended March 31, 2022 and 2021, respectively, and were recorded as research and development expenses in the consolidated statements of operations and comprehensive loss.

12. Net Loss Per Share

Since the Company incurred net losses for the three months ended March 31, 2022 and 2021, common stock equivalents were excluded from the calculation of diluted net loss per share 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 Months Ended March 31,	
	2022	2021
Stock options issued and outstanding	8,172	7,346
Unvested restricted stock units outstanding	471	254
Employee stock purchase plan	37	30
	8,680	7,630

13. Supplemental Disclosures

Accrued Expenses and Other Current Liabilities

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

	March 31, 2022	December 31, 2021
Accrued income taxes payable	$ 12,672	$ 11,325
Accrued external research and development expenses	10,855	11,783
Accrued sublicense fees and royalties	9,057	23,483
Accrued personnel costs	7,852	19,849
Accrued purchases of property and equipment	4,042	5,285
Accrued external general and administrative expenses	3,607	3,642
Other accrued expenses and current liabilities	338	744
	$ 48,423	$ 76,111

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 $5.4 million as of March 31, 2022, a net decrease of $4.7 million from December 31, 2021, and $13.3 million as of March 31, 2021, a net increase of $3.8 million from December 31, 2020.

Proceeds due to the Company for sales of non-marketable equity securities included in other current assets as of March 31, 2022 were $0.6 million. No such amounts were recorded as of March 31, 2021.

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, 2021, which we filed with the SEC on March 1, 2022. 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, 2021 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:

- **RGX-314:** We are developing RGX-314 in collaboration with AbbVie as a potential one-time treatment for wet age-related macular degeneration (wet AMD), diabetic retinopathy (DR) and other additional chronic retinal conditions which cause total or partial vision loss. We are evaluating two separate routes of administration of RGX-314 to the eye: a subretinal delivery procedure as well as a targeted, in-office administration to the suprachoroidal space. 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 two pivotal trials, ATMOSPHERE™ and ASCENT™, to evaluate the efficacy and safety of RGX-314 in patients with wet AMD using the subretinal delivery approach, and we expect these pivotal trials to support a Biologics Licensing Application (BLA) submission in 2024. ATMOSPHERE and ASCENT are multi-center, randomized, active-controlled trials to evaluate the efficacy and safety of a single-administration of RGX-314 versus standard of care in patients with wet AMD. We initiated the pivotal program using cGMP material produced from our existing manufacturing process and plan to incorporate our scalable suspension cell culture manufacturing process to support future commercialization, upon completion of a bridging study.

  We are also evaluating the efficacy, safety and tolerability of suprachoroidal delivery of RGX-314 through AAVIATE®, a multi-center, open label, randomized, controlled, dose-escalation Phase II trial of RGX-314 for the treatment of wet AMD. Enrollment in AAVIATE is expected to be completed in the first half of 2022. Cohorts 4 and 5 are evaluating RGX-314 at a third dose level of $1x10^{12}$ genomic copies per eye (GC/eye). Cohort 5 is evaluating RGX-314 in patients who are neutralizing antibody (NAb) positive. As in previous cohorts, patients will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

  Enrollment is complete in ALTITUDE™, a multi-center, open label, randomized, controlled, dose-escalation Phase II trial to evaluate the efficacy, safety and tolerability of RGX-314 for the treatment of DR. Cohorts 2 and 3 are evaluating RGX-314 at an increased dose level of $5x10^{11}$ GC/eye, with Cohort 3 evaluating RGX-314 in patients who are NAb positive. As in Cohort 1, patients did not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314. As of January 18, 2022, suprachoroidal delivery of RGX-314 continued to be well tolerated in the 15 patients dosed with RGX-314 in Cohort 1 in ALTITUDE, with no drug-related serious adverse events (SAEs), and no intraocular inflammation observed. Of the patients dosed with RGX-314 in Cohort 1, 47% demonstrated a two-step or greater improvement from baseline on the Early Treatment Diabetic Retinopathy Study-Diabetic Retinopathy Severity Scale (ETDRS-DRSS) at six months, compared to 0% in the observational control group. One patient (7%) dosed with RGX-314 continued to demonstrate a four-step improvement. The percentage of Cohort 1 patients dosed with RGX-314 achieving at least two-step improvement at six months in RGX-314 treated eyes (47%) increased from the previously reported three-month results (33%).

- **RGX-202:** We are developing RGX-202 for the treatment of Duchenne muscular dystrophy (Duchenne), a rare disease caused by mutations in the gene responsible for making dystrophin, a protein of central importance for muscle cell structure and function. Without dystrophin, muscles throughout the body degenerate and become weak, eventually leading to loss of movement and independence, required support for breathing, cardiomyopathy and premature death.

We have received clearance of our Investigational New Drug (IND) application by the U.S. Food and Drug Administration (the FDA) to evaluate RGX-202 in a first-in-human, Phase I/II clinical trial named AFFINITY DUCHENNE™. This will be a multicenter, open-label dose escalation and dose expansion clinical study to evaluate the safety, tolerability and clinical efficacy of RGX-202 in patients with Duchenne. We have taken proactive measures that will result in the delayed dosing of the first patient in AFFINITY DUCHENNE due to an unexpected observation in the final stages of manufacturing at one of our third-party manufacturers. We continue to prepare for trial initiation, including readying clinical trial sites and manufacturing additional clinical supply for the upcoming trial. We anticipate dosing the first patient in this trial in the first half of 2023.

- **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.

  We are conducting a Phase I/II trial of RGX-121 in patients with MPS II under the age of 5 years old to evaluate the safety and tolerability of RGX-121, as well as the effects of RGX-121 on biomarkers of I2S enzyme activity, neurocognitive development and other clinical measures. As of December 20, 2021, RGX-121 continued to be well-tolerated, with no drug-related SAEs across three dose levels. Preliminary results indicated dose-dependent reductions in key cerebrospinal fluid biomarkers, with patients in Cohort 3 approaching normal levels of the D2S6 biomarker. Measures of neurodevelopmental function from patients in Cohorts 1 and 2 demonstrated continued developmental skill acquisition up to 2 years after RGX-121 administration. Evidence of systemic enzyme expression and biomarker activity continued to be observed. We continue with plans for enrollment in the Cohort 3 expansion arm of this trial using commercial-scale cGMP material.

  Enrollment is ongoing in a second Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II ages 5-18 years old to evaluate the safety of a single administration of RGX-121, the effects of RGX-121 on biomarkers of I2S enzyme activity, and changes in cognitive function, adaptive behavior, daily function and quality of life.

- **RGX-111:** We are developing RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I), a rare disease caused by a deficiency of IDUA, an enzyme required for the breakdown of structures that dispose of waste products inside cells.

  We are conducting a 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. As of December 20, 2021, RGX-111 was well tolerated across two dose levels in the Phase I/II trial and in the single-patient IND, with no drug-related SAEs. Biomarker and neurodevelopmental assessments indicated encouraging central nervous system profile in patients dosed with RGX-111, with emerging evidence of systemic biomarker activity observed. We continue with plans for enrollment in the Cohort 2 expansion arm of the Phase I/II trial.

- **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 (TPP1)* gene.

- **RGX-381:** We are developing RGX-381 for the treatment of the ocular manifestations of CLN2 disease.

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 March 31, 2022, our NAV Technology Platform was being applied in one FDA approved product (Zolgensma®), and the preclinical and clinical development of a number of partnered programs. 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.

Impact of COVID-19

We are continuing to actively monitor the impact of the COVID-19 pandemic, including the emergence of variant strains, on our business, results of operations and financial condition. Our offices, laboratories, clinical trial sites, prospective clinical trial sites, contract research organizations (CROs), contract manufacturing organizations (CMOs) and other collaborators and partners are located in jurisdictions where quarantines, executive orders, shelter-in-place orders, guidelines, and other similar orders and restrictions intended to control the spread of the disease have been put in place by governmental authorities. At certain times during the COVID-19 pandemic, we have implemented a work-from-home policy for all employees who are not essential to be onsite, and we may take additional actions that alter our operations, as may be required by federal, state or local authorities or which we determine are in the best interests of our employees.

The COVID-19 pandemic has caused delays to our clinical trials and may further delay or prevent us from proceeding with our clinical trials. Our other business initiatives, such as preclinical development and manufacturing operations, may also be affected by the COVID-19 pandemic. For example, the construction of our current good manufacturing practice production facility was delayed from our original estimates due to various government orders and restrictions relating to the COVID-19 pandemic. In addition, if the business and operations of our licensees are adversely affected by the COVID-19 pandemic, our revenues could in turn be adversely affected. We are proactively taking measures to mitigate or reduce any adverse impact of the COVID-19 pandemic on the progress of our clinical trials and other business initiatives.

Our results of operations for three months ended March 31, 2022 and 2021 were not significantly impacted by the COVID-19 pandemic. However, the full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition in the future remains unknown at this time and will depend on future developments that are highly unpredictable. Please refer to the “Risk Factors” section of our Annual Report on Form 10-K for the year ended December 31, 2021 for further discussion of the risks we face as a result of the COVID-19 pandemic.

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.

Collaboration and License Agreement with AbbVie

In September 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 RGX-314 (the AbbVie Collaboration Agreement). The AbbVie Collaboration Agreement became effective in November 2021. 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 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, 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.

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, milestone payments 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. Milestone payments are payable to licensors upon the achievement of specified milestones by licensees and are recognized in the period the milestone is achieved or deemed probable of achievement. 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 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 plan to increase our research and development expenses for the foreseeable future as we continue development of our product candidates. Our current and planned research and development activities include the following:

- continued development of RGX-314 products under our collaboration with AbbVie, including:
    - a Phase I/II clinical trial and associated long-term follow-up study to evaluate the safety and efficacy of the subretinal delivery of RGX-314 for the treatment of wet AMD;
    - pivotal trials (ATMOSPHERE and ASCENT) to evaluate the safety and efficacy of the subretinal delivery of RGX-314 for the treatment of wet AMD;
    - Phase II clinical trials to evaluate the safety and efficacy of the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet AMD (AAVIATE) and DR (ALTITUDE); and
    - additional long-term follow-up and other studies associated with RGX-314.

- a Phase I/II clinical trial to evaluate the safety and efficacy of RGX-202 for the treatment of Duchenne (AFFINITY DUCHENNE);
- Phase I/II clinical trials to evaluate the safety and efficacy of RGX-121 for the treatment of MPS II;
- a Phase I/II clinical trial to evaluate the safety and efficacy of RGX-111 for the treatment of MPS I;
- preclinical research and development for RGX-181 for the treatment of CLN2 disease and RGX-381 for the treatment of the ocular manifestations of CLN2 disease;
- preclinical research and development for potential product candidates addressing other diseases across a range of therapeutics areas and other new technologies;
- continued investment in advanced manufacturing analytics and process development activities; and
- continued acquisition and manufacture of clinical trial materials in support of our anticipated clinical trials.

The following table summarizes our research and development expenses incurred during the three months ended March 31, 2022 and 2021 (in thousands):

	Three Months Ended March 31,	
	2022	2021
Direct Expenses		
RGX-314 (net of cost reimbursement from AbbVie)	$ 7,527	$ 3,341
RGX-202	2,493	1,918
RGX-121 and RGX-111	3,249	1,923
RGX-181 and RGX-381	260	769
Other product candidates	283	337
Total direct expenses	13,812	8,288
Unallocated Expenses		
Platform and new technologies	13,042	7,388
Personnel-related	23,371	19,661
Facilities and depreciation expense	4,530	4,129
Other unallocated	872	256
Total unallocated expenses	41,815	31,434
Total research and development	$ 55,627	$ 39,722

Direct expenses related to the development of RGX-314 for the three months ended March 31, 2022 include $2.9 million in net costs reimbursable by AbbVie under our eye care collaboration which were recorded as a reduction of research and development expenses during the period. 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. We typically utilize our employee and infrastructure resources across our development programs. We do not allocate personnel and other internal costs, such as facilities and other overhead costs, to specific product candidates or development programs.

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, 2021. There have been no significant changes in our critical accounting policies and estimates since December 31, 2021.

Results of Operations

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

	Three Months Ended March 31,		
	2022	2021	Change
Revenues			
License and royalty revenue	$ 22,218	$ 18,884	$ 3,334
Total revenues	22,218	18,884	3,334
Operating Expenses			
Cost of revenues	15,717	4,851	10,866
Research and development	55,627	39,722	15,905
General and administrative	22,318	17,838	4,480
Credit losses and other	83	515	(432)
Total operating expenses	93,745	62,926	30,819
Loss from operations	(71,527)	(44,042)	(27,485)
Other Income (Expense)			
Interest income from licensing	94	29	65
Investment income	799	580	219
Interest expense	(6,130)	(6,702)	572
Total other income (expense)	(5,237)	(6,093)	856
Loss before income taxes	(76,764)	(50,135)	(26,629)
Income Tax Benefit (Expense)	41	(4)	45
Net loss	$ (76,723)	$ (50,139)	$ (26,584)

Comparison of the Three Months Ended March 31, 2022 and 2021

License and Royalty Revenue. License and royalty revenue increased by $3.3 million, from $18.9 million for the three months ended March 31, 2021 to $22.2 million for the three months ended March 31, 2022. The increase was primarily attributable to Zolgensma royalty revenues, which increased by $3.3 million, from $18.3 million for the first quarter of 2021 to $21.5 million for the first quarter of 2022. As reported by Novartis, sales of Zolgensma for the first quarter of 2022 increased by 14% as compared to the first quarter of 2021, driven by geographic expansion of product access.

Cost of Revenues. Cost of revenues increased by $10.9 million, from $4.9 million for the three months ended March 31, 2021 to $15.7 million for the three months ended March 31, 2022. The increase 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. For further information regarding the amendment of the license agreement with Penn, please refer to Note 7, “Commitments and Contingencies” to the accompanying unaudited consolidated financial statements.

Research and Development Expense. Research and development expenses increased by $15.9 million, from $39.7 million for the three months ended March 31, 2021 to $55.6 million for the three months ended March 31, 2022. The increase was primarily attributable to the following:

- an increase of $8.5 million in costs associated with clinical trial and regulatory activities for our lead product candidates, largely driven by RGX-314 clinical trials;
- an increase of $3.7 million in personnel-related costs as a result of increased headcount of research and development personnel, including a $0.6 million increase in stock-based compensation expense, largely driven by the expected commencement of in-house manufacturing of clinical supply in 2022;
- an increase of $2.9 million in manufacturing-related expenses, primarily related to clinical supply for our lead product candidates;
- an increase of $1.5 million in costs of laboratories and facilities used by research and development personnel, including a $0.6 million increase in depreciation expense allocated to research and development functions, largely driven by the occupation of our new corporate, research and manufacturing headquarters in mid-2021; and
- an increase of $1.3 million in costs associated with preclinical activities and other early stage research and development.

The increase in research and development expenses was partially offset by $2.9 million of net development cost reimbursement from AbbVie recorded in the first quarter of 2022 under our RGX-314 collaboration, which was recorded as a reduction of research and development expenses.

General and Administrative Expense. General and administrative expenses increased by $4.5 million, from $17.8 million for the three months ended March 31, 2021 to $22.3 million for the three months ended March 31, 2022. The increase was primarily attributable to the following:

- an increase of $1.6 million in personnel-related costs as a result of increased headcount of general and administrative personnel, including a $0.2 million increase in stock-based compensation expense; and
- an increase of $1.6 million for professional services, primarily related to legal and other advisory services.

Liquidity and Capital Resources

Sources of Liquidity

As of March 31, 2022, we had cash, cash equivalents and marketable securities of $764.8 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 March 31, 2022, 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 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. Furthermore, given the continuing uncertainty and volatile market and economic conditions caused by the COVID-19 pandemic, as well as the potential for further effects due to a resurgence in COVID-19 infections, we will continue to monitor the nature and extent of the impact of the COVID-19 pandemic on our liquidity and capital resources.

Cash Flows

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

	Three Months Ended March 31,	
	2022	2021
Net cash used in operating activities	$ (55,911)	$ (44,047)
Net cash used in investing activities	(90,591)	(214,183)
Net cash provided by (used in) financing activities	(6,834)	211,286
Net decrease in cash and cash equivalents and restricted cash	$ (153,336)	$ (46,944)

Cash Flows from Operating Activities

Our net cash used in operating activities for the three months ended March 31, 2022 increased by $11.9 million from the three months ended March 31, 2021. The increase was largely driven by an increase in operating expenses in the first quarter of 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 three months ended March 31, 2022, our net cash used in operating activities of $55.9 million consisted of a net loss of $76.7 million, offset by adjustments for non-cash items of $14.8 million and favorable changes in working capital of $6.0 million. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $10.8 million and depreciation and amortization expense of $2.6 million. The changes in working capital include a decrease in accounts receivable of $5.7 million which was largely driven by a decrease in Zolgensma royalties receivable, a decrease in other current assets of $3.0 million which was largely driven by a decrease in amounts due from AbbVie for net reimbursement of development costs under our RGX-314 collaboration, and an increase in other liabilities of $6.9 million which was largely driven by a long-term liability recorded during the period resulting from the amendment of our license agreement with Penn. The favorable changes in working capital were partially offset by a net decrease in total accounts payable and accrued expenses and other current liabilities of $9.7 million, which was primarily driven by a decrease in accrued personnel costs. Other changes in working capital were incurred in the normal course of business.

For the three months ended March 31, 2021, our net cash used in operating activities of $44.0 million consisted of a net loss of $50.1 million and unfavorable changes in working capital of $11.2 million, offset by $17.3 million in adjustments for non-cash items. The changes in working capital include a $10.5 million decrease in accrued expenses and other current liabilities which was largely driven by decreases in accrued personnel costs, accrued royalties payable to licensors and accrued external research and development expenses as of March 31, 2021. The changes in working capital were partially offset by an increase in operating lease liabilities of $4.2 million which was largely driven by funds received under our tenant improvement allowance for the buildout of our new headquarters facility in Rockville, Maryland. Other changes in working capital were incurred in the normal course of business. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of $9.9 million, non-cash interest expense recognized under our royalty purchase agreement with HCR of $3.8 million and depreciation and amortization expense of $1.9 million.

Cash Flows from Investing Activities

For the three months ended March 31, 2022, our net cash used in investing activities consisted of $129.5 million to purchase marketable debt securities and $11.0 million to purchase property and equipment, offset by $49.9 million in maturities of marketable debt securities. The substantial majority of our capital expenditures for the three months ended March 31, 2022 were related to the build out of our corporate, manufacturing and research headquarters at 9804 Medical Center Drive in Rockville, Maryland. We have completed the build out of this facility, and capital expenditures in 2022 are expected to be lower than 2021 as a result of the completed build out of this facility.

For the three months ended March 31, 2021, our net cash used in investing activities consisted of $233.6 million to purchase marketable debt securities and $31.0 million to purchase property and equipment, offset by $50.5 million in maturities of marketable debt securities.

Cash Flows from Financing Activities

For the three months ended March 31, 2022, our net cash used in financing activities primarily consisted of $7.5 million of Zolgensma royalties paid to HCR under our royalty purchase agreement, net of imputed interest, and was partially offset by $1.0 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

For the three months ended March 31, 2021, our net cash provided by financing activities primarily consisted of $216.2 million in net proceeds received from a public offering of our common stock completed in January 2021, net of underwriting discounts and commissions and other offering expenses paid during the period, and was partially offset by $6.6 million of Zolgensma royalties paid to HCR, net of imputed interest, under our royalty purchase agreement.

Additional Capital Requirements

Our financial obligations primarily consist of vendor contracts to provide research services and other purchase commitments with suppliers. In the normal course of business, we enter into services agreements with contract research organizations, contract manufacturing organizations and other third parties. Generally, these agreements provide for termination upon notice, with specified amounts due upon termination based on the timing of termination and the terms of the agreement. The amounts and timing of payments under these agreements are uncertain and contingent upon the initiation and completion of the services to be provided.

Our commitments also include obligations to our licensors under our in-license agreements, which may include sublicense fees, milestones fees, royalties and reimbursement of patent maintenance costs. Sublicense fees are payable to licensors when we sublicense underlying intellectual property to third parties; the fees are based on a percentage of the license fees we receive from sublicensees. Milestone fees are payable to licensors upon our future achievement of certain development and regulatory milestones. Royalties are payable to licensors based on a percentage of net sales of licensed products. Patent maintenance costs are payable to licensors as reimbursement for the cost of maintaining of license patents. Due to the contingent nature of the payments, the amounts and timing of payments to licensors under our in-license agreements are uncertain and may fluctuate significantly from period to period.

We have entered into a number of long-term operating leases for office, laboratory and manufacturing space in Rockville, Maryland and New York, New York, as well as a number of laboratory and other equipment leases. Please refer to Note 6 to the audited consolidated financial statements accompanying our Annual Report on Form 10-K for the year ended December 31, 2021 for further information regarding our lease commitments. There have been no material changes to our leasing arrangements since December 31, 2021.

Under the terms of our royalty purchase agreement with HCR, our future Zolgensma royalties, less amounts payable by us to certain licensors, will be payable to HCR up to a specified capped amount. As of March 31, 2022, the total amount of future Zolgensma royalties to be paid to HCR under the agreement was $194.2 million if paid by November 7, 2024, or $234.2 million if paid after that date. We have no obligation to repay any amounts to HCR if total future Zolgensma royalty payments are not sufficient to repay these amounts.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $238.0 million as of March 31, 2022. 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 other revenue, if any, received in connection with commercial sales of our licensees' and collaborators' products, should any of their product candidates receive marketing approval;
- 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. 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, 2021. There have been no material changes to our exposure to market risk during the three months ended March 31, 2022.

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 March 31, 2022. 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 March 31, 2022, 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 March 31, 2022 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, 2021. 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.

None.

Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
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	
10.1†	Letter Agreement dated March 21, 2022 between the Company and The Trustees of the University of Pennsylvania				X
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 March 31, 2022 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 March 31, 2022 formatted in Inline XBRL (included in Exhibit 101)				

† Portions of this exhibit have been omitted.

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: May 4, 2022

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

Dated: May 4, 2022

/s/ Vittal Vasista
Vittal Vasista
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Certain identified information has been excluded from this exhibit because such information both (i) is not material and (ii) would likely cause competitive harm if publicly disclosed. Excluded information is indicated with brackets and asterisks.

[Letterhead of Penn Center for Innovation]

March 21, 2022

VIA ELECTRONIC MAIL
CONFIDENTIAL

Ken Mills, President and CEO
REGENXBIO Inc.
9804 Medical Center Drive
Rockville, MD 20850

Re: Confidential Letter Agreement

Dear Ken:

The purpose of this letter agreement (the "2022 Letter Agreement") is to amend aspects of the Parties' relationship and also to memorialize the terms of a confidential settlement of potential claims between The Trustees of the University of Pennsylvania ("Penn") and REGENXBIO Inc. ("REGENXBIO"), effective as of March 21, 2022 (the "Effective Date"). Penn and REGENXBIO each may be referred to herein as a "Party," and collectively as the "Parties." This letter also serves to release GSK from certain claims by REGENXBIO that REGENXBIO had, has, or may have under various agreements listed below.

Penn and REGENXBIO hereby acknowledge and agree that this 2022 Letter Agreement covers any and all claims[1] that have been asserted or that could have been asserted against each other through the Effective Date based on:

- a License Agreement dated February 24, 2009 between Penn and REGENXBIO (then known as ReGenX, LLC) relating to a gene therapy technology platform based on certain novel adeno associated viruses ("AAVs") discovered by Dr. James M. Wilson, M.D., Ph.D. of Penn's School of Medicine ("Dr. Wilson"), as amended (the "Penn-REGENXBIO License");
- a License Agreement dated May 31, 2002 between GlaxoSmithKline LLC (formerly SmithKline Beecham Corporation, d/b/a GlaxoSmithKline) ("GSK") and Penn, as amended (the "Penn-GSK License"); and
- a Sublicense Agreement dated March 6, 2009 between REGENXBIO and GSK, as amended (the "GSK-REGENXBIO Sublicense").

1 Including but not limited to those described in correspondence between the parties identified in Exhibit A.

Any and all rights to patents or patent applications or intellectual property licensed by REGENXBIO under any of the foregoing licenses or sublicenses are referred to herein as the "Licensed Patents." The subset of Licensed Patents that are the subject of the GSK-REGENXBIO Sublicense are referred to herein as the "Sublicensed Patents."

To avoid the costs and uncertainties of further protracted disputes between the parties, and without admission by either Party of fault, liability or wrongdoing, the Parties hereby agree as follows:

1. Representations and Warranties.

A. Wilson Lab Sponsors/ Programs. Penn represents and warrants that Exhibit B hereto contains a listing of certain commercial entities that, as of the Effective Date, have an agreement to sponsor research, to collaborate with, or to receive license rights for discoveries relating to adeno-associated vectors made by Dr. Wilson and/or any individuals who are under the direct supervision or control of Dr. Wilson at Penn, including all core laboratories that report directly to Dr. Wilson (collectively, the "Wilson Lab"). Such commercial entities and any future sponsors added in accordance with the procedures described in paragraph 4 are referred to herein as the "Wilson Lab Sponsors." Penn further represents and warrants that for each such Wilson Lab Sponsor included in Exhibit B, the exhibit also sets forth the product candidate(s), including [****], for which, as of the Effective Date, each such Sponsor has agreed to sponsor research or otherwise collaborate, and/or for which such commercial entity has received license or option rights from Penn ("Wilson Lab Programs"). Penn may supplement Exhibit B after the Effective Date to add new Wilson Lab Sponsors and/or Programs provided these additions are in accordance with the procedures described in paragraph 4.

B. REGENXBIO Sublicensees. REGENXBIO represents and warrants that (1) Exhibit C hereto contains a full and complete listing of every sublicensee that has received rights in the Licensed Patents, including option rights, from REGENXBIO (the "REGENXBIO Sublicensees"), and (2) complete and accurate copies of the entire executed sublicense agreements for such REGENXBIO Sublicensees, written in the English language, have been provided to Penn. REGENXBIO Sublicensees that are exclusively sublicensed or optioned under the Licensed Patents may be referred to as the "REGENXBIO Exclusive Sublicensees." REGENXBIO acknowledges that it will, within [****] after REGENXBIO enters into any additional sublicense agreements, deliver to Penn a complete and accurate copy of the entire executed sublicense agreement written in the English language, and also agrees to contemporaneously update Exhibit C.

C. REGENXBIO Products. REGENXBIO represents and warrants that Exhibit D.1 hereto contains a full and complete listing, including [****], of every product candidate involving AAVs that REGENXBIO has in development as of the Effective Date. REGENXBIO represents and warrants that Exhibit D.2 hereto contains, to the best of REGENXBIO's knowledge, a full and complete listing, including [****], of every AAV product candidate with respect to which REGENXBIO has sublicensed rights to a Licensed Patent. REGENXBIO may reasonably supplement Exhibits D.1 and D.2 within [****] of additional product candidates being actively developed by REGENXBIO or its sublicensees. REGENXBIO shall not supplement Exhibits D.1 or D.2 to add product candidates that would compete directly with a Covered Wilson Lab Program without Penn's consent, not to be unreasonably withheld.

2. Penn and REGENXBIO Release. For good and valuable consideration, the receipt of which is hereby acknowledged, Penn and REGENXBIO, their past, present and future trustees, shareholders, officers, directors, parents, subsidiaries, affiliates, predecessors, successors, assigns, employees, agents and insurers, and all persons acting by, through, under or in concert with any of them (the "Releasors"), do unconditionally, fully, finally and forever remise, release, relinquish, compromise and discharge all claims, counterclaims, suits, demands, liabilities, or causes of action of any kind, in law or equity, known or unknown, that they have or may have against each other, including without limitation all claims for contribution or indemnity that might be asserted between them, arising out of or related to the Penn-REGENXBIO License, the Penn-GSK License, or the GSK-REGENXBIO Sublicense, through the Effective Date, including without limitation: (a) any claim against REGENXBIO for underpayment of past or currently due royalties pursuant to Section 3.3 of the GSK-REGENXBIO Sublicense, including any such claims obtained by Penn from GSK pursuant to the assignment attached as Exhibit F; (b) any claim against REGENXBIO for non-payment or underpayment of past or current sublicense fees pursuant to Section 3.5 of the Penn-REGENXBIO License; (c) any other claim against REGENXBIO for failure to make appropriate payments under the Penn-REGENXBIO License or the Penn-GSK License; (d) [****]; and (e) any other claim against Penn arising out of or related to any Penn agreements with third parties to sponsor research or collaborate with, or to license technology from, Penn and/or the Wilson Lab. The Parties agree that these releases do not extend to any royalties for sales during the first quarter of 2022 that will become due after March 31, 2022, or any claims arising out of conduct occurring after the Effective Date, or any claims for breach of this 2022 Letter Agreement.

3. Release of Current Wilson Lab Sponsors. For good and valuable consideration, the receipt of which is hereby acknowledged, REGENXBIO, its past, present and future shareholders, officers, directors, parents, subsidiaries, affiliates, predecessors, successors, assigns, employees, agents and insurers (the "REGENXBIO Releasors"), do unconditionally, fully, finally and forever remise, release, relinquish, compromise and discharge all claims, counterclaims, suits, demands, liabilities, or causes of action of any kind, in law or equity, that they have or may have against the Wilson Lab Sponsors listed on Exhibit B as of the Effective

Date, their past, present and future general partners, limited partners, shareholders, members, officers, directors, parents, subsidiaries, affiliates, predecessors, successors, controlling persons, contractors, assigns, attorneys, employees, agents, sublicensees, collaborators and insurers, and all persons acting by, through, under or in concert with any of them (the "Wilson Lab Sponsor Released Parties"), including without limitation all claims for contribution or indemnity that might be asserted between them, through the Effective Date, arising out of or related to any Penn agreements with third parties to sponsor research or collaborate with, or to license technology from, Penn and/or the Wilson Lab. The Parties agree that these releases do not extend to any claims arising out of conduct occurring after the Effective Date or breach of this 2022 Letter Agreement. The Parties further agree that these releases do not extend to any claims of patent infringement other than those involving the Sublicensed Patents.

4. Future Wilson Lab Sponsors and Programs. In advance of finalizing any agreement after the Effective Date with (1) a new commercial entity Penn wishes to identify as a new Wilson Lab Sponsor pursuant to paragraph 1.A hereof ("Proposed Wilson Lab Sponsor"), or (2) a current or new Wilson Lab Sponsor to add a new Wilson Lab Program that Penn wishes to protect pursuant to paragraph 1.A and 7 hereof (a "Future Wilson Lab Program"), Penn will notify REGENXBIO of the proposed sponsor and the program's proposed product candidates, including [****]. Such additional Future Wilson Lab Program and Proposed Wilson Lab Sponsor will be considered a Covered Wilson Lab Program, as defined below, and a Wilson Lab Sponsor, as defined above, *only* after receiving REGENXBIO's written consent, which shall not unreasonably be withheld or delayed. REGENXBIO's withholding of such consent is reasonable if: [****]. Once a Future Wilson Lab Program or Proposed Wilson Lab Sponsor has received REGENXBIO's written consent, it will be automatically added to Exhibit B. The Wilson Lab Programs listed on Exhibit B as of the Effective Date and any new Wilson Lab Programs consented to by REGENXBIO pursuant to this paragraph, sometimes are referred to collectively as the "Covered Wilson Lab Programs."

5. REGENXBIO Release of GSK. For good and valuable consideration, the receipt of which is hereby acknowledged, the REGENXBIO Releasors do unconditionally, fully, finally and forever remise, release, relinquish, compromise and discharge all claims, counterclaims, suits, demands, liabilities, or causes of action of any kind, in law or equity, known or unknown, that they have or may have against GSK, its past, present and future shareholders, officers, directors, parents, subsidiaries, affiliates, predecessors, successors, assigns, employees, agents and insurers, and all persons acting by, through, under or in concert with it including without limitation all claims for contribution or indemnity that might be asserted against it, arising out of or related to (a) [****]; and (b) any other claim against GSK arising out of or related to any Penn agreements with third parties to sponsor research or collaborate with, or to license technology from, Penn and/or the Wilson Lab. Nothing in the preceding releases shall be construed to release REGENXBIO from claims by GSK arising out of or related to Section 3.4 of the GSK-REGENXBIO Sublicense Agreement.

6. Covenant Not to Sue Penn/the Wilson Lab. REGENXBIO agrees that Penn and the Wilson Lab may continue conducting gene therapy research without restriction outside of the

field identified on Exhibit E, and that REGENXBIO will not assert that such research constitutes a breach of any provision of the Penn-GSK License or the Penn-REGENXBIO License, even if such research is sponsored by a commercial entity. REGENXBIO hereby covenants and agrees not to sue Penn, including its past, present and future trustees, officers, directors, parents, subsidiaries, affiliates, predecessors, successors, controlling persons, contractors, assigns, attorneys, employees, agents and insurers for any past, present or future Wilson Lab activities, that might otherwise infringe a valid claim of a Licensed Patent, including but not limited to commercially sponsored research, outside of the field identified on Exhibit E. Notwithstanding the foregoing, Penn agrees that Dr. Wilson and other members of the Wilson Lab under his supervision [****].

7. Covenant Not to Sue Wilson Lab Sponsors. REGENXBIO hereby covenants and agrees not to sue any Wilson Lab Sponsor, including its past, present and future officers, directors, parents, subsidiaries, affiliates, predecessors, successors, controlling persons, contractors, assigns, attorneys, employees, agents and insurers for infringement of any Sublicensed Patent based upon any past, present or future activities relating to or arising out of a Covered Wilson Lab Program, including but not limited to sponsored research [****]; *provided, however*, that the covenant and agreement not to sue Wilson Lab Sponsors shall not: [****]. The Parties agree that, for purposes of the release set forth in paragraph 3 and the covenant not to sue set forth in this paragraph 7, the Wilson Lab Sponsors with Covered Wilson Lab Programs shall be considered third-party beneficiaries of this 2022 Letter Agreement and shall have the right to enforce such provisions directly to the extent they deem such enforcement necessary or advisable to protect their rights.

8. Covenant Not to Sue GSK. REGENXBIO agrees that Penn and the Wilson Lab may continue conducting gene therapy research without restriction outside of the field identified on Exhibit E, and that REGENXBIO will not assert that such research constitutes a breach of any provision of the Penn-GSK License, the Penn-REGENXBIO License or the GSK-REGENXBIO Sublicense, even if such research is sponsored by a commercial entity. REGENXBIO hereby covenants and agrees not to sue GSK, its past, present and future shareholders, officers, directors, parents, subsidiaries, affiliates, predecessors, successors, assigns, employees, agents and insurers, and all persons acting by, through, under or in concert with it for any past, present or future Wilson Lab activities, that might otherwise infringe a valid claim of a Licensed Patent, including but not limited to commercially sponsored research, outside of the field identified on Exhibit E.

9. Sublicense Fees. Section 3.5 of the Penn-REGENXBIO License is hereby deleted in its entirety.

10. Payments. In consideration of the Mutual General Release, amendment to the Penn-REGENXBIO License as set forth in paragraph 9, and in full satisfaction of its past, present, and future obligations to pay sublicense fees pursuant to Section 3.5 of the Penn-REGENXBIO License, REGENXBIO shall pay Penn, as follows:

A. REGENXBIO shall pay Penn Twelve Million Dollars ($12,000,000) ("Initial Settlement Payment") by wire transfer. The Initial Settlement Payment may be paid to Penn in the form of annual installments, with the first payment of Three Million Dollars ($3,000,000) due on the first anniversary of the Effective Date, and Three Million Dollars ($3,000,000) due on each anniversary of the first payment until the total amount has been paid; and

B. REGENXBIO shall pay Penn Eight Million Dollars ($8,000,000) by wire transfer within [****] of the Effective Date as consideration arising from and related to the AbbVie/REGENXBIO strategic partnership and sublicense agreement announced on September 13, 2021.

Any amounts provided for under this paragraph that remain unpaid shall immediately become due and payable within [****] of any of the following events: [****]. The offsets provided for in Section 1.1(b)(ii) of the Penn-REGENXBIO License shall not apply to any payment required pursuant to this paragraph. Any amounts that are not paid by REGENXBIO when due will accrue interest at [****]. No interest will accrue on REGENXBIO's annual installment payments made pursuant to this paragraph 10.A if REGENXBIO elects to pay the Initial Settlement Amount in the form of annual installments.

11. Assignment of GSK Royalty Rights. REGENXBIO hereby consents to the assignment from GSK to Penn of any and all past, present and future rights to receive royalties and reports, and to have access to records, pursuant to Sections 3.3, 3.5 and 3.6 of the GSK-REGENXBIO License, as provided in the Assignment, Release and Eighth Amendment to the Penn-GSK License, attached hereto as Exhibit F. Nothing in this 2022 Letter Agreement shall diminish Penn's right to amounts due to Penn pursuant to Section 3.2 of the Penn-REGENXBIO License or any other amounts due under the existing Penn-REGENXBIO License, except those amounts due under Section 3.5, which has been deleted pursuant to paragraph 9, and nothing in this 2022 Letter Agreement shall be used to interpret Section 3.2 of the Penn-REGENXBIO License. Notwithstanding GSK's assignment of rights under Section 3.5 of the GSK-REGENXBIO License, REGENXBIO agrees to deliver reports pursuant to Section 3.5.1 to both Penn and GSK.

12. Future Royalty Payments Under the GSK-REGENXBIO Sublicense. The Parties agree that, in consideration of the Mutual General Release and other good and valuable consideration, REGENXBIO will continue to pay royalties pursuant to Section 3.3 of the GSK-REGENXBIO Sublicense [****] at the royalty percentages for [****] Licensed Products. REGENXBIO agrees to pay royalties payable pursuant to the GSK-REGENXBIO Sublicense at the [****] royalty percentage for all other current and future Licensed Products that would otherwise infringe a Valid Claim covering a therapeutic use of a vector or compound, including but not limited to: [****]. For such other products, REGENXBIO shall pay royalties at the [****] royalty percentage [****].

13. No Admission of Liability. Nothing contained herein is an admission by either of the Parties, or any Wilson Lab Sponsor, of any liability, fault, or wrongdoing, nor a concession as to any issue of contract interpretation.

14. Joint Press Release. The Parties agree to issue, within [****] of the Effective Date, a joint press release to be reasonably agreed. Other than the agreed upon press release, no Party shall issue any press release or other publicity material or make any public representation that refers to the existence of this 2022 Letter Agreement without the prior written consent of the other Party (which consent shall not be unreasonably withheld or delayed), unless permitted by paragraph 16. Prior to the issuance of the press release, the Parties shall provide the entire press release to GSK for its review. The Parties shall permit GSK to review the joint press release and comment on any content referring to or affecting GSK.

15. Future Activities. The Parties agree to pursue potential new collaboration and alliance opportunities together and to conduct further exploration of joint participation and potential philanthropic commitments to the Institutes for Life Changing Medicines at Penn at a time and forum to be mutually agreed.

16. Confidentiality. Apart from the joint press release referenced in paragraph 14, each Party agrees not to disclose any term of this 2022 Letter Agreement, and/or any information provided pursuant the terms of this 2022 Letter Agreement, to any third party without the prior written consent of the other Party (which consent shall not be unreasonably withheld or delayed); *provided, however*, that: (a) each Party shall be free to disclose such information to the extent disclosure is required by order or regulation of a government agency, court or other tribunal having jurisdiction, *provided, however*, that, to the extent permitted by law, such Party shall not make any such disclosure (other than a filing of information or materials with the U.S. Securities and Exchange Commission or an equivalent authority in another jurisdiction or a relevant stock exchange that is made with a request for confidential treatment for any part of such disclosure for which such treatment may reasonably be expected to be granted) without first notifying the other Party and allowing such Party a reasonable opportunity to seek a protective order and/or injunctive relief from the obligation to make such disclosure; (b) each Party shall be free to disclose such information to [****], provided that such entities and/or individuals are obligated to keep such terms confidential to the same extent as said Party; (c) Penn shall be free to disclose such information to GSK and Wilson Lab Sponsors, and REGENXBIO shall be free to disclose such information to GSK and [****], provided that such entities are obligated to keep such terms confidential to the same extent as said Parties; and (d) each Wilson Lab Sponsor shall be free to disclose the release set forth in paragraph 3, the covenant not to sue set forth in paragraph 7, and any portion of Exhibit B pertaining to the Sponsor's programs, to [****], provided that such entities are obligated to keep such terms confidential to the same extent as such Wilson Lab Sponsor. [****].

17. Governing Law. This 2022 Letter Agreement shall be governed by and interpreted in accordance with the laws of the Commonwealth of Pennsylvania without giving effect to any conflict of laws provision. Any disputes arising from or relating to this 2022 Letter Agreement or the subject matter hereof, shall be subject to the exclusive jurisdiction of the United States District Court for the Eastern District of Pennsylvania, *provided, however*, that, if for any reason, jurisdiction over such dispute does not exist in such federal court, the dispute shall be brought and litigated exclusively in the Pennsylvania state courts.

18. Authority and Binding Effect. Each Party represents that: (a) the person executing this 2022 Letter Agreement on its behalf is fully empowered, authorized, and entitled to enter into this 2022 Letter Agreement on behalf of the Party for whom he or she is executing it; and (b) this 2022 Letter Agreement is a legal, valid, and binding obligation, enforceable against it in accordance with its terms. The obligations and rights under this 2022 Letter Agreement shall be binding upon and inure to the benefit of, as the case may be, the Parties' owners, employees, successors, assigns, heirs, and personal representatives.

19. Complete Agreement. This 2022 Letter Agreement constitutes the complete agreement between the Parties as to the subject matter identified herein. Any modifications to this 2022 Letter Agreement must be made in writing and signed by both Parties. To the extent that there is any inconsistency between this 2022 Letter Agreement and the terms of any of the referenced license or sublicense agreements, this 2022 Letter Agreement shall govern. The Penn-GSK License, the Penn-RGX License and the GSK-RGX Sublicense remain in full force and effect unless expressly modified, waived, amended or superseded by the terms of this 2022 Letter Agreement.

Very truly yours,

/s/ John S. Swartley

John S. Swartley, Ph.D
Associate Vice Provost for Research
Managing Director, Penn Center for Innovation
on behalf of The Trustees of the University of Pennsylvania

AGREED ON BEHALF OF REGENXBIO INC.:

By: /s/ Ken Mills

Name: Ken Mills

Title: President & CEO

Date: March 22, 2022

ACKNOWLEDGED BY GSK:

By: /s/ Justin T. Huang

Name: Justin T. Huang

Title: Secretary

Date: Mar. 22, 2022

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: May 4, 2022

/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: May 4, 2022

/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 March 31, 2022 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: May 4, 2022

/s/ Kenneth T. Mills

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

Date: May 4, 2022

/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.