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, 2021

OR

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

For the transition period from ___ to ___

Commission File Number 001-37553

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

Delaware **(State or other jurisdiction of incorporation or organization)**	**47-1851754** **(I.R.S. Employer Identification No.)**
9600 Blackwell Road, Suite 210 **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 30, 2021, there were 42,509,366 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, 2021

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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 made by us 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:

- the impact of the COVID-19 pandemic on our business, operations and preclinical and clinical development timelines and plans;
- the 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;
- the timing of commencement and completion and the success of preclinical studies conducted by us and our development partners;
- the timely development and launch of new products;
- the scope, progress, expansion and costs of developing and commercializing our product candidates;
- our ability to obtain, maintain and enforce intellectual property protection for our product candidates and technology, and defend against third-party intellectual property-related claims;
- our expectations regarding the development and commercialization of product candidates currently being developed by third parties that utilize our technology;
- our anticipated growth strategies;
- our expectations regarding competition;
- the anticipated trends and challenges in our business and the market in which we operate;
- our ability to attract or retain key personnel;
- the size and growth of the potential markets for our product candidates and the ability to serve those markets;
- the rate and degree of market acceptance of any of our products that are approved;
- our ability to establish and maintain development partnerships;
- our expectations regarding our expenses and revenue;
- our expectations regarding the outcome of legal proceedings, including our arbitration with Abeona Therapeutics Inc. regarding license fees that have not been paid to us and our ability to recover such unpaid fees;
- 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, 2020 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

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, 2021	December 31, 2020
Assets		
Current assets		
Cash and cash equivalents	$ 291,482	$ 338,426
Marketable securities	149,398	137,314
Accounts receivable, net	41,039	42,999
Prepaid expenses	13,839	10,505
Other current assets	2,880	1,953
Total current assets	498,638	531,197
Marketable securities	215,598	46,809
Accounts receivable, net	2,859	3,267
Property and equipment, net	89,342	56,467
Operating lease right-of-use assets	62,607	63,815
Restricted cash	1,330	1,330
Other assets	9,068	5,279
Total assets	$ 879,442	$ 708,164
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 11,311	$ 10,622
Accrued expenses and other current liabilities	41,605	49,082
Deferred revenue	395	449
Operating lease liabilities	1,843	2,500
Liability related to sale of future royalties	28,807	18,794
Total current liabilities	83,961	81,447
Deferred revenue	3,729	3,783
Operating lease liabilities	75,078	70,153
Liability related to sale of future royalties	161,722	174,504
Other liabilities	448	524
Total liabilities	324,938	330,411
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at March 31, 2021 and December 31, 2020	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at March 31, 2021 and December 31, 2020; 42,505 and 37,476 shares issued and outstanding at March 31, 2021 and December 31, 2020, respectively	4	4
Additional paid-in capital	895,079	667,181
Accumulated other comprehensive loss	(1,368)	(360)
Accumulated deficit	(339,211)	(289,072)
Total stockholders' equity	554,504	377,753
Total liabilities and stockholders' equity	$ 879,442	$ 708,164

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, 2021	Three Months Ended March 31, 2020
Revenues		
License and royalty revenue	$ 18,884	$ 17,644
Total revenues	18,884	17,644
Operating Expenses		
Cost of revenues	4,851	3,409
Research and development	39,722	37,035
General and administrative	17,838	14,833
Provision for credit losses and other	515	67
Total operating expenses	62,926	55,344
Loss from operations	(44,042)	(37,700)
Other Income (Expense)		
Interest income from licensing	29	848
Investment income (loss)	580	(3,186)
Interest expense	(6,702)	—
Total other income (expense)	(6,093)	(2,338)
Loss before income taxes	(50,135)	(40,038)
Income Tax Expense	(4)	—
Net loss	$ (50,139)	$ (40,038)
Other Comprehensive Loss		
Unrealized loss on available-for-sale securities, net	(1,008)	(785)
Total other comprehensive loss	(1,008)	(785)
Comprehensive loss	$ (51,147)	$ (40,823)
Net loss per share, basic and diluted	$ (1.20)	$ (1.08)
Weighted-average common shares outstanding, basic and diluted	41,819	37,104

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, 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

	Three Months Ended March 31, 2020					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2019	36,992	$ 4	$ 627,810	$ 205	$ (177,822)	$ 450,197
Exercise of stock options	181	—	2,154	—	—	2,154
Issuance of common stock under employee stock purchase plan	17	—	607	—	—	607
Stock-based compensation expense	—	—	8,017	—	—	8,017
Unrealized loss on available-for-sale securities, net	—	—	—	(785)	—	(785)
Net loss	—	—	—	—	(40,038)	(40,038)
Balances at March 31, 2020	37,190	$ 4	$ 638,588	$ (580)	$ (217,860)	$ 420,152

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, 2021	Three Months Ended March 31, 2020
Cash flows from operating activities		
Net loss	$ (50,139)	$ (40,038)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	9,920	8,017
Depreciation and amortization	1,929	1,994
Provision for credit losses	565	—
Net amortization of premiums on marketable securities	1,288	39
Net realized and unrealized losses (gains) on marketable securities	(7)	5,111
Imputed interest income from licensing	(29)	(848)
Non-cash interest expense	6,702	—
Other non-cash adjustments	(154)	25
Changes in operating assets and liabilities		
Accounts receivable	1,618	(5,744)
Prepaid expenses	(3,334)	(947)
Other current assets	(873)	(2,833)
Operating lease right-of-use assets	1,232	703
Other assets	(3,789)	1,705
Accounts payable	278	3,063
Accrued expenses and other current liabilities	(10,544)	(3,813)
Operating lease liabilities	4,244	(851)
Other liabilities	(38)	(1,156)
Net cash used in operating activities	(41,131)	(35,573)
Cash flows from investing activities		
Purchases of marketable debt securities	(233,627)	(30,692)
Maturities of marketable debt securities	50,465	60,907
Sales of marketable equity securities	—	7,124
Purchases of property and equipment	(31,021)	(4,630)
Net cash provided by (used in) investing activities	(214,183)	32,709
Cash flows from financing activities		
Proceeds from exercise of stock options	1,292	2,154
Proceeds from issuance of common stock under employee stock purchase plan	627	607
Proceeds from public offering of common stock, net of underwriting discounts and commissions	216,438	—
Issuance costs for public offerings of common stock	(251)	—
Repayments under liability related to sale of future royalties	(9,471)	—
Transaction costs for sale of future royalties	(265)	—
Net cash provided by financing activities	208,370	2,761
Net decrease in cash and cash equivalents and restricted cash	(46,944)	(103)
Cash and cash equivalents and restricted cash		
Beginning of period	339,756	70,844
End of period	$ 292,812	$ 70,741
Supplemental disclosures of non-cash investing and financing activities		
Additions to property and equipment through accounts payable and accrued expenses	$ 3,783	$ —

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, 2021, 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 more than 20 licensed products. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

As of March 31, 2021, the Company had generated an accumulated deficit of $339.2 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, 2021, the Company had cash, cash equivalents and marketable securities of $656.5 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, 2020 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2020, which was filed with the SEC on March 1, 2021. 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, 2020, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2020.

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 on 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, non-cash interest expense, 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 is 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.

Reclassifications

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

Restricted Cash

Restricted cash includes money market mutual funds used to collateralize irrevocable letters of credit as required by the Company's lease 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):

	March 31, 2021	March 31, 2020
Cash and cash equivalents	$ 291,482	$ 69,411
Restricted cash	1,330	1,330
Total cash and cash equivalents and restricted cash	$ 292,812	$ 70,741

Accounts Receivable

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

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

Fair Value of Financial Instruments

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

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

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

Net Loss Per Share

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

3. Marketable Securities

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

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
March 31, 2021				
U.S. government and federal agency securities	$ 7,590	$ —	$ (1)	$ 7,589
Certificates of deposit	1,466	23	—	1,489
Corporate bonds	353,423	221	(754)	352,890
Municipal securities	3,025	3	—	3,028
	$ 365,504	$ 247	$ (755)	$ 364,996

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2020				
U.S. government and federal agency securities	$ 12,782	$ 22	$ —	$ 12,804
Certificates of deposit	1,956	34	—	1,990
Corporate bonds	165,850	497	(55)	166,292
Municipal securities	3,035	2	—	3,037
	$ 183,623	$ 555	$ (55)	$ 184,123

As of March 31, 2021 and December 31, 2020, 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, 2021 and December 31, 2020, the balance in the Company's 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 (loss). 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,	
	2021	2020
Unrealized loss before reclassifications	$ (1,001)	$ (755)
Realized gains reclassified to investment income	(7)	(30)
Income tax expense	—	—
Unrealized loss on available-for-sale securities, net	$ (1,008)	$ (785)

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, 2021						
U.S. government and federal agency securities	$ 7,589	$ (1)	$ —	$ —	$ 7,589	$ (1)
Corporate bonds	275,305	(754)	—	—	275,305	(754)
	$ 282,894	$ (755)	$ —	$ —	$ 282,894	$ (755)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2020						
Corporate bonds	$ 55,507	$ (55)	$ —	$ —	$ 55,507	$ (55)
	$ 55,507	$ (55)	$ —	$ —	$ 55,507	$ (55)

As of March 31, 2021, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 49 investment grade security positions. The Company has the intent and ability to hold such securities until recovery, and due to 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, 2021 or December 31, 2020. The Company did not recognize any impairment or credit losses on available-for-sale debt securities during the three months ended March 31, 2021 and 2020.

During the three months ended March 31, 2020, the Company recognized total net realized and unrealized losses of $5.1 million related to its marketable equity securities of Prevail Therapeutics Inc. (Prevail), which were acquired as consideration for a license to the NAV Technology Platform granted to Prevail in August 2017. As of December 31, 2020, the Company had sold all of its Prevail equity securities.

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, 2021				
Cash equivalents:				
Money market mutual funds	$ —	$ 240,240	$ —	$ 240,240
Total cash equivalents	—	240,240	—	240,240
Marketable securities:				
U.S. government and federal agency securities	—	7,589	—	7,589
Certificates of deposit	—	1,489	—	1,489
Corporate bonds	—	352,890	—	352,890
Municipal securities	—	3,028	—	3,028
Total marketable securities	—	364,996	—	364,996
Total cash equivalents and marketable securities	$ —	$ 605,236	$ —	$ 605,236

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2020				
Cash equivalents:				
Money market mutual funds	$ —	$ 96,307	$ —	$ 96,307
Total cash equivalents	—	96,307	—	96,307
Marketable securities:				
U.S. government and federal agency securities	—	12,804	—	12,804
Certificates of deposit	—	1,990	—	1,990
Corporate bonds	—	166,292	—	166,292
Municipal securities	—	3,037	—	3,037
Total marketable securities	—	184,123	—	184,123
Total cash equivalents and marketable securities	$ —	$ 280,430	$ —	$ 280,430

Management estimates that the carrying amounts of its current accounts receivable, accounts payable and 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. 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, 2021 to determine the present value of the receivables. Accordingly, management estimates that the carrying value of its non-current accounts receivable approximates the fair value of those instruments.

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, 2021 and December 31, 2020, non-marketable equity securities had a carrying value of $1.1 million and were included in other assets on the consolidated balance sheets. The Company did not identify any observable price changes or changes in circumstances that would have had an adverse effect on the fair value of the securities as of March 31, 2021 or December 31, 2020. No remeasurements or impairment losses were recorded on non-marketable equity securities during the three months ended March 31, 2021 and 2020.

5. Property and Equipment, Net

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

	March 31, 2021	December 31, 2020
Laboratory and manufacturing equipment	$ 34,124	$ 26,306
Computer equipment and software	3,954	3,764
Furniture and fixtures	4,699	4,114
Leasehold improvements	71,167	44,957
Total property and equipment	113,944	79,141
Accumulated depreciation and amortization	(24,602)	(22,674)
Property and equipment, net	$ 89,342	$ 56,467

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 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 non-cash 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 non-cash interest expense under the Royalty Purchase Agreement based on its estimate of future royalty payments to be received by HCR. As of March 31, 2021, the estimated effective interest rate under the agreement was 13.9%. 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):

	Three Months Ended March 31, 2021
Liability related to sale of future royalties, beginning balance	$ 193,298
Zolgensma royalties paid to HCR	(9,471)
Non-cash interest expense	6,702
Liability related to sale of future royalties, ending balance	190,529
Current portion of liability related to sale of future royalties	(28,807)
Liability related to sale of future royalties, non-current	$ 161,722

7. Capitalization

In January 2021, the Company completed a public offering of 4,899,000 shares of its common stock (inclusive of 639,000 shares pursuant to the full exercise by the underwriters of their option to purchase additional shares) at a price of $47.00 per share. The aggregate net proceeds received by the Company from the offering, inclusive of the underwriters' option exercise, were $216.1 million, net of underwriting discounts and commissions and offering expenses payable by the Company.

8. License and Royalty Revenue

As of March 31, 2021, the Company's NAV Technology Platform was being applied by NAV Technology Licensees in one commercially available product, Zolgensma, and in the development of more than 20 licensed products. Consideration to the Company under its license agreements may include: (i) up-front and annual fees, (ii) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products. 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.

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, 2021, 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 $194.8 million, including (i) $23.3 million upon the commencement of various stages of clinical trials, (ii) $21.0 million upon the submission of regulatory approval filings, (iii) $93.5 million upon the approval of commercial products by regulatory agencies and (iv) $57.0 million upon the achievement of specified sales targets for 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 milestones by licensees 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, 2021	Three Months Ended March 31, 2020
Accounts receivable, net, current and non-current:		
Balance, beginning of period	$ 46,266	$ 42,303
Additions	18,718	18,142
Deductions	(21,086)	(11,550)
Balance, end of period	$ 43,898	$ 48,895
Contract assets:		
Balance, beginning of period	$ 350	$ —
Additions	649	350
Deductions	(350)	—
Balance, end of period	$ 649	$ 350
Deferred revenue, current and non-current:		
Balance, beginning of period	$ 4,232	$ 3,333
Additions	—	—
Deductions	(108)	—
Balance, end of period	$ 4,124	$ 3,333
Revenue recognized during the period from:		
Amounts included in deferred revenue at beginning of period	$ 108	$ —
Performance obligations satisfied in previous periods	$ 18,651	$ 10,379

Additions to accounts receivable during the periods presented consisted primarily of receivables recorded related to royalties on net sales of Zolgensma, new licenses granted by the Company, the achievement of development milestones by licensees and interest income from licensing recognized 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 primarily of development milestones deemed probable of achievement by licensees during the period. Deductions to contract assets during the periods presented consisted of the achievement of such milestones and billing of the associated milestone payments by the Company.

As of March 31, 2021, the Company had recorded deferred revenue of $4.1 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 the licensee 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 royalty revenues as well as 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, 2021	December 31, 2020
Current accounts receivable:		
Billed to customers	$ 30,584	$ 30,573
Unbilled	18,698	20,104
Allowance for credit losses	(8,243)	(7,678)
Current accounts receivable, net	41,039	42,999
Non-current accounts receivable:		
Unbilled	2,859	3,267
Allowance for credit losses	—	—
Non-current accounts receivable, net	2,859	3,267
Total accounts receivable, net	$ 43,898	$ 46,266

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, 2021 (in thousands):

	Accounts Receivable	Contract Assets
Balance at December 31, 2020	$ 7,678	$ —
Provision for credit losses	565	—
Write-offs	—	—
Balance at March 31, 2021	$ 8,243	$ —

The Company's allowance for credit losses as of March 31, 2021 and December 31, 2020 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 for the three months ended March 31, 2021 was $0.6 million and was related solely to changes in estimates regarding the allowance for credit losses associated with the accounts receivable from Abeona. No provision for credit losses was recorded for the three months ended March 31, 2020.

Novartis Gene Therapies, Inc.

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,	
	2021	2020
Royalties on net sales of Zolgensma	$ 18,263	$ 9,978
Total license and royalty revenue	$ 18,263	$ 9,978
Interest income from licensing	$ 6	$ 7

As of March 31, 2021 and December 31, 2020, the Company had recorded total accounts receivable of $18.1 million and $19.6 million, respectively, from Novartis Gene Therapies under the March 2014 License, which consisted primarily of unbilled receivables for Zolgensma royalties. Zolgensma royalties receivable as of March 31, 2021 included $13.2 million which was expected to be paid to HCR in accordance with the Royalty Purchase Agreement discussed in Note 6.

Abeona Therapeutics Inc.

In November 2018, the Company entered into a license agreement with Abeona, as amended, (the November 2018 License), for the development and commercialization 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 was terminated. As a result of the termination, Abeona was required to pay a $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. As of April 30, 2021, the Company had not received any portion of the $28.0 million in license fees due from Abeona under the license agreement. Unpaid balances due under the November 2018 License accrue interest at 1.5% per month.

In May 2020, subsequent to 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 disputes Abeona’s claim and filed a counterclaim in arbitration demanding payment of the $28.0 million of unpaid fees from Abeona, plus accrued interest. Based on its evaluation of the merits of Abeona’s claims, the Company did not record any liabilities related these claims as of March 31, 2021, and the Company currently expects that its demand for payment in full will be upheld in arbitration. A binding arbitration was held in March 2021 and the arbitrators’ decision is pending. The Company intends to enforce the full collection of all amounts due from Abeona; however, the outcome of the arbitration and timing of payment from Abeona remain uncertain.

As of March 31, 2021 and December 31, 2020, the Company had recorded gross accounts receivable of $30.1 million from Abeona under the November 2018 License, which consisted of the $8.0 million fee due April 1, 2020, the $20.0 million fee due within 15 days of the termination of the license agreement in May 2020 and accrued interest on the outstanding balances. While the Company currently expects its demand for payment in full will be upheld in arbitration and intends to enforce the full collection of all amounts due, the Company assessed the collectability of the $30.1 million due 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 this obligation upon the completion of arbitration in 2021. As a result of its analyses, the Company recorded an allowance for credit losses of $8.2 million and $7.7 million as of March 31, 2021 and December 31, 2020, respectively, related to the accounts receivable due from Abeona. The Company recorded a provision for credit losses of $0.6 million for the three months ended March 31, 2021 as a result of changes in estimates regarding the allowance during the period.

As of March 31, 2021 and December 31, 2020, the Company had recognized interest income from licensing of $2.1 million related to the unpaid license fees from Abeona under the November 2018 License, which is included in the gross accounts receivable balance of $30.1 million. In accordance with its interest accrual policy, the Company ceased the recognition of interest income accrued under the license agreement subsequent to the recognition of the allowance for credit losses in the third quarter of 2020, and will continue to maintain the accounts receivable from Abeona on non-accrual status unless and until such amounts are deemed to be collectable. However, the Company intends to enforce the full collection of all accrued interest contractually due from Abeona upon the completion of arbitration.

9. Stock-based Compensation

In January 2021, the Board of Directors authorized an additional 1,499,037 shares to be issued under the 2015 Equity Incentive Plan (the 2015 Plan). As of March 31, 2021, the total number of shares of common stock authorized for issuance under the 2015 Plan and the 2014 Stock Plan (the 2014 Plan) was 13,911,954, of which 2,447,174 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,	
	2021	2020
Stock options	$ 9,015	$ 7,779
Restricted stock units	679	—
Employee stock purchase plan	226	238
	$ 9,920	$ 8,017

As of March 31, 2021, the Company had $93.8 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 2.8 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, 2021	Three Months Ended March 31, 2020
Research and development	$ 5,031	$ 4,047
General and administrative	4,889	3,970
	$ 9,920	$ 8,017

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, 2020	6,361	$ 31.21	7.2	$ 101,356
Granted	1,170	$ 44.92		
Exercised	(105)	$ 12.30		
Cancelled or forfeited	(80)	$ 45.48		
Outstanding at March 31, 2021	7,346	$ 33.51	7.4	$ 51,356
Exercisable at March 31, 2021	4,126	$ 26.47	6.2	$ 50,065
Vested and expected to vest at March 31, 2021	7,346	$ 33.51	7.4	$ 51,356

(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, 2021 was $27.28. During the three months ended March 31, 2021, the total number of stock options exercised was 104,638, resulting in total proceeds of $1.3 million. The total intrinsic value of options exercised during the three months ended March 31, 2021 was $3.6 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, 2020	—	$ —
Granted	257	$ 44.92
Vested	—	$ —
Forfeited	(3)	$ 44.97
Unvested balance at March 31, 2021	254	$ 44.92

No restricted stock units vested during the three months ended March 31, 2021 and 2020.

Employee Stock Purchase Plan

In January 2021, the Board of Directors authorized an additional 374,759 shares to be issued under the 2015 ESPP. As of March 31, 2021, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 998,683, of which 803,728 remained available for future issuance. During the three months ended March 31, 2021, 19,042 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. Based on the Company's history of operating losses, including three-year cumulative loss positions as of March 31, 2021 and December 31, 2020, the Company concluded that it is 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, 2021 and December 31, 2020.

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. Expenses incurred under the agreement with FOXKISER for the three months ended March 31, 2021 and 2020 were $1.2 million and $1.2 million, 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, 2021 and 2020, 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,	
	2021	**2020**
Stock options issued and outstanding	7,346	6,438
Unvested restricted stock units outstanding	254	—
Employee stock purchase plan	30	32
	7,630	6,470

13. Supplemental Disclosures

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

	March 31, 2021	**December 31, 2020**
Accrued purchases of property and equipment	$ 11,231	$ 7,853
Accrued personnel costs	7,970	13,155
Accrued external research and development expenses	7,792	9,738
Accrued sublicense fees and royalties	6,828	12,160
Accrued income taxes payable	4,005	3,135
Accrued external general and administrative expenses	3,657	2,865
Other accrued expenses and current liabilities	122	176
	$ 41,605	$ 49,082

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, 2020, which we filed with the SEC on March 1, 2021. 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, 2020 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 gene therapy product candidates are designed to deliver genes to cells to address genetic defects or to enable cells in the body to produce therapeutic proteins that are intended to impact disease. Through a single administration, our gene therapy product candidates are designed to provide long-lasting effects, potentially significantly altering the course of disease and delivering improved patient outcomes.

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 through two modalities: AAV-mediated antibody delivery and monogenic gene replacement. The AAV-mediated antibody delivery modality is designed to treat serious and chronic diseases by delivering the genes necessary for the sustained production of therapeutic antibodies *in vivo*. Our monogenic gene replacement approach builds upon the well-understood mechanism of replacing a dysfunctional or missing gene with a functional copy of the gene in order to enable sustained production of necessary proteins.

Gene therapy using NAV Vectors for AAV-mediated antibody delivery

- **RGX-314:** We are developing RGX-314 as a novel, single-administration gene therapy for the treatment of 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 advancing two separate routes of administration of RGX-314 to the eye, through a standardized subretinal delivery procedure as well as by delivery to the suprachoroidal space using the SCS Microinjector™ licensed from Clearside Biomedical, Inc.

  In January 2021, we announced that we completed an End of Phase 2 meeting with the U.S. Food and Drug Administration (FDA) to discuss the details of a pivotal program to evaluate the efficacy and safety of RGX-314 in patients with wet AMD using the subretinal delivery approach. We plan to conduct two randomized, well-controlled clinical trials to evaluate the efficacy and safety of RGX-314 in patients with wet AMD, enrolling approximately 700 patients total. The first pivotal trial (ATMOSPHERE™) is active and enrolling patients. Based on the outcome of these trials, the pivotal program is expected to support a Biologics License Application (BLA) filing in 2024.

  In February 2021, we announced additional positive data from the patients enrolled in the ongoing Phase I/II trial of RGX-314 for the treatment of wet AMD and its Long-Term Follow-Up study. As of January 22, 2021, RGX-314 continued to be generally well-tolerated across all dose cohorts. Durable treatment effect was observed in patients in Cohorts 4 and 5 at 1.5 years after administration of RGX-314, including stable visual acuity, decreased retinal thickness, and reductions in anti-VEGF injection burden. Long-term, durable treatment effect was demonstrated in Cohort 3 over three years, including mean improvement in vision and stable retinal thickness, and reductions in anti-VEGF treatment burden.

  We are also conducting a Phase II trial of the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet AMD known as AAVIATE®. We have completed enrollment in Cohort 1 of this trial, and we plan to report interim data from Cohort 1 in the third quarter of 2021. We have also completed enrollment in Cohort 2 and expect to report interim data from Cohort 2 in the second half of 2021. In addition, we have expanded AAVIATE and began dosing in a third cohort of patients. Cohort 3 will evaluate the efficacy, safety and tolerability of RGX-314 in up to 20 patients who are neutralizing antibody (NAb) positive. The same dose evaluated in Cohort 2, $5.0\text{x}10^{11}$ genomic copies per eye (GC/eye) of RGX-314, will be delivered to patients in Cohort 3 via a single injection. As with Cohorts 1 and 2, patients in Cohort 3 will not receive prophylactic immune suppressive corticosteroid therapy before or after administration of RGX-314.

  In addition, we announced in December 2020 that the first patient had been dosed in ALTITUDE™, a Phase II trial of the suprachoroidal delivery of RGX-314 for the treatment of DR. Patient enrollment continues and we expect to report initial data from this trial in 2021.

- **AAV-Mediated Antibody Expression for the Treatment of Hereditary Angioedema (HAE):** We are developing a novel, one-time treatment utilizing a NAV Vector to deliver a gene encoding for a therapeutic antibody that targets and binds to plasma kallikrein, a key protein left unregulated in patients with HAE. HAE is a chronic and severe disease characterized by recurring severe swelling (angioedema), most commonly in the face, airway, intestines and limbs. We expect to provide a program update in 2021.
- **AAV-Mediated Antibody Expression for the Treatment of Neurodegenerative Diseases:** We have established a research program in partnership with Neurimmune AG (Neurimmune) to jointly develop and commercialize novel gene therapies using NAV Vectors to deliver human antibodies for chronic neurodegenerative diseases, with an initial focus on diseases associated with the accumulation and deposition of the microtubule-associated protein tau (tauopathies) and alpha-synuclein (alpha-synucleinopathies). We expect to provide a program update in 2021.

Gene therapy programs for the potential treatment of rare monogenic diseases

- **RGX-202:** We are developing RGX-202 for the treatment of Duchenne Muscular Dystrophy (DMD), a severe, progressive, degenerative muscle disease caused by mutations in the gene which encodes dystrophin, a protein involved in muscle cell structure and function. Without functional dystrophin protein, muscles throughout the body degenerate and become weak. We expect to submit an Investigational New Drug (IND) application for this program in mid-2021.
- **RGX-121:** We are developing RGX-121 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type II (MPS II), a severe genetic lysosomal storage disease caused by deficiency of iduronate-2-sulfatase (I2S), an enzyme that is responsible for breakdown of cellular waste products.

  We are conducting a Phase I/II trial of RGX-121 in patients with MPS II up to the age of 5 years old. As reported in February 2021, RGX-121 was well-tolerated in Cohorts 1 and 2 of the Phase I/II trial, and no drug-related SAEs were reported. Biomarker data from patients in both cohorts indicated encouraging signals of I2S enzyme activity in the central nervous system following one-time administration of RGX-121, with consistent reductions of heparan sulfate (HS) and D2S6, a component of HS. Patients in Cohorts 1 and 2 also demonstrated continued neurocognitive development and evidence of I2S enzyme activity in plasma and urine following administration of RGX-121. In April 2021, we announced that the first patient has been dosed in Cohort 3 of the ongoing Phase I/II trial.

  In addition, the first patient has been dosed in a second Phase I/II trial of RGX-121 for the treatment of pediatric patients with MPS II over the age of 5 years old.
- **RGX-111:** We are developing RGX-111 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type I (MPS I), a severe genetic lysosomal storage disease caused by deficiency of α-l-iduronidase (IDUA), an enzyme required for breakdown of cellular waste products. We have completed dosing of patients in the first cohort of a Phase I/II clinical trial for RGX-111.
- **RGX-181:** We are developing RGX-181 for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, one of the most common forms of Batten disease, caused by mutations in the tripeptidyl peptidase 1 (TPP1) gene. An IND was submitted to the FDA, after which the FDA notified REGENXBIO that its proposed trial had been placed on clinical hold and the agency requested more information to support the initial dose selection and certain study drug administration procedures. REGENXBIO is evaluating the FDA's requests and plans to provide an update on the program in the second half of 2021.
- **RGX-381:** We are developing RGX-381 for the treatment of ocular manifestations of CLN2 disease. Based on communication with the FDA and the update from the RGX-181 program, we now expect to provide a program update in the second half of 2021.

In addition to our lead product candidates described above, we have also funded, and plan to continue to fund, preclinical research on potential product candidate programs that may become part of our internal product development pipeline. We have partnered with a number of leading academic institutions and will continue to seek partnerships with innovative institutions to develop novel NAV gene therapy product candidates.

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, 2021, our NAV Technology Platform was being applied in one FDA approved product (Zolgensma®), and the preclinical and clinical development of more than 20 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 actively monitoring the impact of the COVID-19 pandemic 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. We have implemented a work-from-home policy for all employees who are not essential to be onsite, and we may take further 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 new headquarters, including our current good manufacturing practice production facility, has been delayed from our original estimates, and may be delayed further, 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 the three months ended March 31, 2021 and 2020 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 is 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, 2020 for further discussion of the risks we face as a result of the COVID-19 pandemic.

Financial Overview

Revenues

Our revenues to date primarily consist of license and royalty revenue resulting from the licensing of our NAV Technology Platform. 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 to other biotechnology and pharmaceutical companies. 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. 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) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products.

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.

Future license and royalty revenues are dependent on the successful development and commercialization of licensed products by our licensees, 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.

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, 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 NAV Technology 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 NAV Technology 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 NAV Technology 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 expense primarily consists of:

- salaries and personnel-related costs, including benefits 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
- allocated facility-related costs, depreciation expense and other overhead.

Up-front fees incurred in obtaining technology licenses for research and development activities, as well as associated milestone payments, are expensed 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:

- 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 one additional pivotal trial) 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);
- preclinical research and development and a planned clinical trial for RGX-202 for the treatment of DMD;
- 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 and a planned clinical trial for RGX-181 for the treatment of CLN2 disease;
- preclinical research and development and a planned clinical trial for RGX-381 for the treatment of ocular manifestations of CLN2 disease;

- preclinical research and development for potential product candidates to treat HAE;
- preclinical research and development for potential product candidates to treat neurodegenerative diseases, including tauopathies and alpha-synucleinopathies, under our collaboration with Neurimmune;
- preclinical research and development for potential product candidates addressing other diseases across a range of therapeutics areas;
- 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, 2021 and 2020 (in thousands):

	Three Months Ended March 31,	
	2021	2020
Direct Expenses		
RGX-314	$ 3,341	$ 6,133
RGX-202	1,918	1,252
RGX-121	1,596	2,340
RGX-181 and RGX-381	769	1,323
Other product candidates	664	958
Total direct expenses	8,288	12,006
Unallocated Expenses		
Platform and new technologies	7,388	5,932
Personnel-related	19,661	15,859
Facilities and depreciation expense	4,129	2,652
Other unallocated	256	586
Total unallocated expenses	31,434	25,029
Total research and development	$ 39,722	$ 37,035

Platform and new technologies include direct costs not identifiable with a specific lead product candidate, including costs associated with our research and development platform, 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 expense consists primarily of salaries and personnel-related costs, including benefits 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 and administrative support functions. Other general and administrative expenses include facility-related and overhead costs not otherwise allocated to research and development expense, professional fees for accounting, legal and advisory services, expenses associated with obtaining and maintaining patents, insurance costs, costs of our information systems and other commercial and general corporate activities. We expect that our general and administrative expense 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 (Loss)

Investment income (loss) consists of interest income earned and gains and losses realized from our cash equivalents and marketable 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 of non-cash interest imputed on the liability related to the sale of future Zolgensma royalties to entities managed by Healthcare Royalty Management, LLC (collectively, HCR). Non-cash 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.

Critical Accounting Policies and Significant Judgments 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, 2020. There have been no significant changes in our critical accounting policies since December 31, 2020.

Results of Operations

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

	Three Months Ended March 31, 2021	Three Months Ended March 31, 2020	Change
Revenues			
License and royalty revenue	$ 18,884	$ 17,644	$ 1,240
Total revenues	18,884	17,644	1,240
Operating Expenses			
Cost of revenues	4,851	3,409	1,442
Research and development	39,722	37,035	2,687
General and administrative	17,838	14,833	3,005
Provision for credit losses and other	515	67	448
Total operating expenses	62,926	55,344	7,582
Loss from operations	(44,042)	(37,700)	(6,342)
Other Income (Expense)			
Interest income from licensing	29	848	(819)
Investment income (loss)	580	(3,186)	3,766
Interest expense	(6,702)	—	(6,702)
Total other income (expense)	(6,093)	(2,338)	(3,755)
Loss before income taxes	(50,135)	(40,038)	(10,097)
Income Tax Expense	(4)	—	(4)
Net loss	$ (50,139)	$ (40,038)	$ (10,101)

Comparison of the Three Months Ended March 31, 2021 and 2020

License and Royalty Revenue. License and royalty revenue increased by $1.2 million, from $17.6 million for the three months ended March 31, 2020 to $18.9 million for the three months ended March 31, 2021. The increase was primarily attributable to Zolgensma royalty revenues, which increased by $8.3 million, from $10.0 million for the first quarter of 2020 to $18.3 million for the first quarter of 2021. As reported by Novartis, sales of Zolgensma for the first quarter of 2021 increased by 88% as compared to the first quarter of 2020. The increase in revenues was partially offset by $7.2 million of non-recurring revenue recognized during the three months ended March 31, 2020 related to a license granted to Ultragenyx Pharmaceutical Inc. during the period.

Research and Development Expense. Research and development expenses increased by $2.7 million, from $37.0 million for the three months ended March 31, 2020 to $39.7 million for the three months ended March 31, 2021. The increase was primarily attributable to the following:

- an increase of $3.8 million for personnel-related costs as a result of increased headcount of research and development personnel, including a $1.0 million increase in stock-based compensation expense;
- an increase of $1.7 million for external costs associated with clinical trial and regulatory activities for our lead product candidates; and
- an increase of $1.2 million for laboratory costs and facilities used by research and development personnel, including depreciation expense allocated to research and development functions.

The increase in research and development expenses was partially offset by a $3.9 million decrease in external costs associated with manufacturing-related services, primarily related to RGX-314.

General and Administrative Expense. General and administrative expenses increased by $3.0 million, from $14.8 million for the three months ended March 31, 2020 to $17.8 million for the three months ended March 31, 2021. The increase was primarily attributable to the following:

- an increase of $1.8 million for professional services, primarily related to commercial consulting and legal services; and
- an increase of $1.2 million for personnel-related costs as a result of increased headcount of general and administrative personnel, including a $0.9 million increase in stock-based compensation expense.

Investment Income (Loss). Investment income was $0.6 million for the three months ended March 31, 2021, as compared to investment loss of $3.2 million for the three months ended March 31, 2020, a change of $3.8 million. The change was primarily attributable to net realized and unrealized losses of $5.1 million recognized in the first quarter of 2020 related to our marketable equity securities of Prevail Therapeutics Inc. (Prevail). We sold all of our Prevail equity securities prior to the first quarter of 2021. The change in investment income was partially offset by a decrease of $1.3 million in interest income in the first quarter of 2021, primarily attributable to lower yields on investments in cash equivalents and marketable debt securities.

Interest Expense. Interest expense increased by $6.7 million for the three months ended March 31, 2021, from zero for the three months ended March 31, 2020. Interest expense consists solely of non-cash interest recognized under our royalty purchase agreement with HCR for the sale of future Zolgensma royalties which occurred in December 2020.

Liquidity and Capital Resources

Sources of Liquidity

As of March 31, 2021, we had cash, cash equivalents and marketable securities of $656.5 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, 2021, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months from the date of this report, based on our current business plan.

In January 2021, we completed a public offering of 4,899,000 shares of our common stock (inclusive of 639,000 shares pursuant to the full exercise by the underwriters of their option to purchase additional shares) at a price of $47.00 per share. The aggregate net proceeds from the offering, inclusive of the underwriters’ option exercise, were $216.1 million, net of underwriting discounts and commissions and offering expenses payable by us.

We intend to devote the majority of our current capital to clinical development, seeking regulatory approval of our product candidates and capital expenditures to build out additional office, laboratory and manufacturing capacity, including the buildout of our future corporate, manufacturing and research headquarters at 9804 Medical Center Drive in Rockville, Maryland. 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 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,	
	2021	2020
Net cash used in operating activities	$ (41,131)	$ (35,573)
Net cash provided by (used in) investing activities	(214,183)	32,709
Net cash provided by financing activities	208,370	2,761
Net decrease in cash and cash equivalents and restricted cash	$ (46,944)	$ (103)

Cash Flows from Operating Activities

Our net cash used in operating activities for the three months ended March 31, 2021 increased by $5.6 million from the three months ended March 31, 2020. The increase was largely driven by an increase in operating expenses of $7.6 million in the first quarter of 2021. We expect to continue to incur net cash out outflows from operations for the foreseeable future as we continue the development and advancement of our lead product candidates and other research programs.

For the three months ended March 31, 2021, our net cash used in operating activities of $41.1 million consisted of a net loss of $50.1 million and changes in working capital of $11.2 million, offset by $20.2 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 related to the ongoing buildout of our new headquarters facility in Rockville, Maryland. Other changes in working capital were incurred in the normal course of business, primarily as a result of differences in the timing of payments to service providers and the period in which such costs are incurred. 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 $6.7 million and depreciation and amortization expense of $1.9 million.

For the three months ended March 31, 2020, our net cash used in operating activities of $35.6 million consisted of a net loss of $40.0 million and changes in working capital of $9.9 million, offset by $14.3 million in adjustments for non-cash items. The changes in working capital include an increase in accounts receivable of $5.7 million which was largely driven by new licenses granted by us during the first quarter of 2020, and a decrease in accrued expenses and other current liabilities of $3.8 million which was largely driven by a decrease in accrued personnel costs as of March 31, 2020. Other changes in working capital were incurred in the normal course of business, primarily as a result of differences in the timing of payments to service providers and the period in which such costs are incurred. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of $8.0 million, net losses on our Prevail equity securities of $5.1 million and depreciation and amortization expense of $2.0 million.

Cash Flows from Investing Activities

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. The substantial majority of our capital expenditures in first quarter of 2021 were related to the build out of our future corporate, manufacturing and research headquarters at 9804 Medical Center Drive in Rockville, Maryland. We expect capital expenditures to continue to increase in 2021 as a result of the ongoing build out of this facility. Total remaining capital expenditures related to the build out of the facility at 9804 Medical Center Drive, net of remaining amounts to be reimbursed by the landlord under our tenant improvement allowance, are expected to be in the mid-double-digit millions (USD) and are expected to be incurred into 2022. However, the actual amount and timing of these capital expenditures are uncertain and may differ materially from our current estimates.

For the three months ended March 31, 2020, our net cash provided by investing activities consisted of $68.0 million in sales and maturities of marketable securities, offset by $30.7 million to purchase marketable debt securities and $4.6 million to purchase property and equipment.

Cash Flows from Financing Activities

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 $9.5 million of Zolgensma royalties paid to HCR during the period under the Zolgensma royalty purchase agreement.

For the three months ended March 31, 2020, our net cash provided by financing activities consisted of $2.8 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $339.2 million as of March 31, 2021. 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 our capital expenditures will continue to increase due to costs associated with building out additional office, laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts, particularly with respect to the build out of our facility at 9804 Medical Center Drive as discussed above. 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 NAV Technology Licensees' 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;
- 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, the majority of which may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform sublicensing is dependent in part on the clinical and commercial success of our licensing partners. 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.

Contractual Obligations, Commitments and Contingencies

There have been no material changes to our contractual obligations, commitments and contingencies as of March 31, 2021 from the information provided in Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” included in our Annual Report on Form 10-K for the year ended December 31, 2020.

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, 2020. There have been no material changes to our exposure to market risk during the three months ended March 31, 2021.

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, 2021. 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, 2021, 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, 2021 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.

For information regarding our legal proceedings with Abeona Therapeutics Inc., please refer to Note 8, "License and Royalty Revenue—Abeona Therapeutics Inc.," to the accompanying unaudited consolidated financial statements.

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, 2020. 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: Form	Exhibit Number	Filing Date	Filed or Furnished Herewith
3.1	Restated Certificate of Incorporation	8-K	3.1	9/22/15	
3.2	Amended and Restated Bylaws	8-K	3.2	9/22/15	
31.1	Certification of the Chief Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
31.2	Certification of the Chief Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
32.1	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350				X
101	The following materials from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2021 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, 2021 formatted in Inline XBRL (included in Exhibit 101)				

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

SIGNATURES

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

	REGENXBIO Inc.
Dated: May 5, 2021	/s/ Kenneth T. Mills
	Kenneth T. Mills President and Chief Executive Officer (Principal Executive Officer)
Dated: May 5, 2021	/s/ Vittal Vasista
	Vittal Vasista Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)

EXHIBIT 31.1

CERTIFICATION

I, Kenneth T. Mills, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of REGENXBIO Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant’s other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
   a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   c. evaluated the effectiveness of the registrant’s disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   d. disclosed in this report any change in the registrant’s internal control over financial reporting that occurred during the registrant’s most recent fiscal quarter (the registrant’s fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant’s internal control over financial reporting.
5. The registrant’s other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant’s auditors and the audit committee of the registrant’s board of directors (or persons performing the equivalent functions):
   a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant’s ability to record, process, summarize and report financial information; and
   b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant’s internal control over financial reporting.

Date: May 5, 2021

/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 5, 2021

/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, 2021 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 5, 2021	/s/ Kenneth T. Mills **Kenneth T. Mills** **President and Chief Executive Officer** **(Principal Executive Officer)**
Date: May 5, 2021	/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.