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

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2020

OR

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

For the transition period from ___ to ___

Commission File Number 001-37553

REGENXBIO Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**47-1851754**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
9600 Blackwell Road, Suite 210 Rockville, MD	**20850**
(Address of principal executive offices)	**(Zip Code)**

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

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

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	RGNX	The Nasdaq Global Select Market

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

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

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

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

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

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

As of July 31, 2020, there were 37,334,013 shares of the registrant’s common stock, par value $0.0001 per share, issued and outstanding.

REGENXBIO INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED JUNE 30, 2020

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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 and maintain intellectual property protection for our product candidates and technology;
- 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;
- our expectations regarding regulatory developments in the United States and foreign countries; and
- the use or sufficiency of our cash and cash equivalents and needs for additional financing.

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, 2019 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, www.regenxbio.com, 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.

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

PART I – FINANCIAL INFORMATION

Item 1. Financial Statements.

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

	June 30, 2020	December 31, 2019
Assets		
Current assets		
Cash and cash equivalents	$ 94,222	$ 69,514
Marketable securities	174,964	226,696
Accounts receivable	42,876	38,148
Prepaid expenses	9,749	6,475
Other current assets	7,271	4,199
Total current assets	329,082	345,032
Marketable securities	70,054	103,785
Accounts receivable	3,618	4,155
Property and equipment, net	32,696	28,973
Operating lease right-of-use assets	8,635	10,078
Restricted cash	1,330	1,330
Other assets	4,323	4,555
Total assets	$ 449,738	$ 497,908
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 10,901	$ 6,409
Accrued expenses and other current liabilities	25,839	24,846
Deferred revenue	450	—
Operating lease liabilities	3,013	2,421
Total current liabilities	40,203	33,676
Deferred revenue	4,007	3,333
Operating lease liabilities	7,085	8,874
Other liabilities	582	1,828
Total liabilities	51,877	47,711
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at June 30, 2020 and December 31, 2019	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at June 30, 2020 and December 31, 2019; 37,291 and 36,992 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	4	4
Additional paid-in capital	648,729	627,810
Accumulated other comprehensive income	750	205
Accumulated deficit	(251,622)	(177,822)
Total stockholders' equity	397,861	450,197
Total liabilities and stockholders' equity	$ 449,738	$ 497,908

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenues				
License and royalty revenue	$ 16,566	$ 7,881	$ 34,210	$ 8,765
Total revenues	16,566	7,881	34,210	8,765
Operating Expenses				
Cost of revenues	4,684	1,927	8,093	1,956
Research and development	38,111	29,483	75,146	54,686
General and administrative	15,554	13,405	30,387	24,963
Other operating expenses (income)	50	(62)	117	(62)
Total operating expenses	58,399	44,753	113,743	81,543
Loss from operations	(41,833)	(36,872)	(79,533)	(72,778)
Other Income				
Interest income from licensing	1,849	762	2,697	1,375
Investment income	5,722	34,524	2,536	37,519
Total other income	7,571	35,286	5,233	38,894
Loss before income taxes	(34,262)	(1,586)	(74,300)	(33,884)
Income Tax Benefit	500	129	500	199
Net loss	$ (33,762)	$ (1,457)	$ (73,800)	$ (33,685)
Other Comprehensive Income				
Unrealized gain on available-for-sale securities, net	1,330	530	545	1,151
Total other comprehensive income	1,330	530	545	1,151
Comprehensive loss	$ (32,432)	$ (927)	$ (73,255)	$ (32,534)
Basic and diluted net loss per share	$ (0.91)	$ (0.04)	$ (1.98)	$ (0.92)
Weighted-average basic and diluted common shares outstanding	37,257	36,669	37,180	36,518

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

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

	Three Months Ended June 30, 2020					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at March 31, 2020	37,190	$ 4	$ 638,588	$ (580)	$ (217,860)	$ 420,152
Exercise of stock options	101	—	1,825	—	—	1,825
Stock-based compensation expense	—	—	8,316	—	—	8,316
Unrealized gain on available-for-sale securities, net	—	—	—	1,330	—	1,330
Net loss	—	—	—	—	(33,762)	(33,762)
Balances at June 30, 2020	37,291	$ 4	$ 648,729	$ 750	$ (251,622)	$ 397,861

	Three Months Ended June 30, 2019					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at March 31, 2019	36,611	$ 4	$ 602,425	$ (59)	$ (115,317)	$ 487,053
Exercise of stock options	141	—	1,367	—	—	1,367
Stock-based compensation expense	—	—	7,099	—	—	7,099
Unrealized gain on available-for-sale securities, net	—	—	—	530	—	530
Net loss	—	—	—	—	(1,457)	(1,457)
Balances at June 30, 2019	36,752	$ 4	$ 610,891	$ 471	$ (116,774)	$ 494,592

	Six Months Ended June 30, 2020					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income	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	282	—	3,979	—	—	3,979
Issuance of common stock under employee stock purchase plan	17	—	607	—	—	607
Stock-based compensation expense	—	—	16,333	—	—	16,333
Unrealized gain on available-for-sale securities, net	—	—	—	545	—	545
Net loss	—	—	—	—	(73,800)	(73,800)
Balances at June 30, 2020	37,291	$ 4	$ 648,729	$ 750	$ (251,622)	$ 397,861

	Six Months Ended June 30, 2019					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2018	36,120	$ 4	$ 592,580	$ (720)	$ (83,016)	$ 508,848
Adoption of ASU 2016-02 (Topic 842)	—	—	—	—	(33)	(33)
Adoption of ASU 2018-02	—	—	—	40	(40)	—
Exercise of stock options	622	—	5,129	—	—	5,129
Issuance of common stock under employee stock purchase plan	10	—	365	—	—	365
Stock-based compensation expense	—	—	12,817	—	—	12,817
Unrealized gain on available-for-sale securities, net	—	—	—	1,151	—	1,151
Net loss	—	—	—	—	(33,685)	(33,685)
Balances at June 30, 2019	36,752	$ 4	$ 610,891	$ 471	$ (116,774)	$ 494,592

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

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

	Six Months Ended June 30,	
	2020	2019
Cash flows from operating activities		
Net loss	$ (73,800)	$ (33,685)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	16,333	12,817
Net amortization of premiums and accretion of discounts on marketable debt securities	205	(788)
Depreciation and amortization	4,126	3,363
Net realized gains on sales and maturities of marketable securities	(7,075)	—
Unrealized losses (gains) on marketable equity securities	7,779	(31,656)
Imputed interest income from licensing	(1,900)	(1,375)
Other non-cash adjustments	373	442
Changes in operating assets and liabilities		
Accounts receivable	(2,711)	(1,419)
Prepaid expenses	(3,274)	(534)
Other current assets	(3,072)	1,419
Operating lease right-of-use assets	1,443	1,156
Other assets	1,355	(1,350)
Accounts payable	4,078	1,774
Accrued expenses and other current liabilities	1,434	(957)
Deferred revenue	—	(600)
Operating lease liabilities	(1,197)	(1,171)
Other liabilities	(1,166)	(482)
Net cash used in operating activities	(57,069)	(53,046)
Cash flows from investing activities		
Purchases of marketable debt securities	(70,665)	(106,119)
Maturities of marketable debt securities	146,353	141,262
Sales of marketable debt securities	2,287	—
Sales of marketable equity securities	7,124	—
Purchases of property and equipment	(7,908)	(8,010)
Net cash provided by investing activities	77,191	27,133
Cash flows from financing activities		
Proceeds from exercise of stock options	3,979	5,129
Proceeds from issuance of common stock under employee stock purchase plan	607	365
Net cash provided by financing activities	4,586	5,494
Net increase (decrease) in cash and cash equivalents and restricted cash	24,708	(20,419)
Cash and cash equivalents and restricted cash		
Beginning of period	70,844	76,614
End of period	$ 95,552	$ 56,195
Supplemental disclosures of non-cash investing and financing activities		
Non-cash consideration received for licenses granted	$ 1,123	$ —

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's proprietary adeno-associated virus (AAV) gene delivery platform (NAV Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. The NAV® Technology Platform is being applied by the Company, as well as by third-party licensees (NAV Technology Licensees), in the development of a broad pipeline of product candidates in multiple therapeutic areas and in one commercially available product, Zolgensma®, which is marketed by a NAV Technology Licensee. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

Liquidity and Risks

As of June 30, 2020, the Company had generated an accumulated deficit of $251.6 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 June 30, 2020, the Company had cash, cash equivalents and marketable securities of $339.2 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.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, development by the Company or its competitors of technological innovations, risks of failure of clinical trials, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to transition from clinical manufacturing to the commercial production of products.

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, 2019 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2019, which was filed with the SEC on February 26, 2020. 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, 2019, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2019.

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 financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Actual results could differ materially from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these consolidated financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process

may result in actual results differing materially from those estimated amounts used in the preparation of the consolidated financial statements. Significant estimates are used in the following areas, among others: license and royalty revenue, stock-based compensation expense, accrued research and development expenses and other accrued liabilities, 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):

	June 30, 2020	June 30, 2019
Cash and cash equivalents	$ 94,222	$ 55,142
Restricted cash	1,330	1,053
Total cash and cash equivalents and restricted cash	$ 95,552	$ 56,195

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 doubtful accounts, 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 doubtful accounts is recorded against accounts receivable such that the carrying value of accounts receivable reflects the net amount expected to be collected. The Company did not record an allowance for doubtful accounts as of June 30, 2020 and December 31, 2019.

Marketable Securities

Marketable securities consist of available-for-sale debt securities and equity securities and are carried at fair value. Marketable debt securities with remaining maturity dates exceeding 12 months which are not intended to be sold prior to maturity for use in current operations are classified as non-current assets. Marketable equity securities are classified as current assets.

Unrealized gains and losses on available-for-sale debt securities, net of any related tax effects, are excluded from results of operations and are included in other comprehensive income and reported as a separate component of stockholders' equity until realized. 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 income. Purchase premiums and discounts on marketable debt securities are amortized or accreted into the cost basis over the life of the related security as adjustments to the yield using the effective-interest method. Interest income is recognized when earned. Unrealized gains and losses on marketable equity securities are included in results of operations as investment income. 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.

At each reporting date, the Company evaluates available-for-sale debt securities which have an amortized cost basis in excess of the fair value of the security to determine if the unrealized loss or any potential credit losses should be recognized in results of operations. If the Company does not have the intent and ability to hold the security until recovery of the unrealized loss, the difference between the fair value and amortized cost basis of the security is charged to results of operations resulting in a new amortized cost basis of the security. If the Company has the intent and ability to hold the security until recovery of the unrealized loss, the security is evaluated for potential credit losses. If a credit loss is deemed to exist, the credit loss is recognized in results of operations and an allowance for credit losses is recorded against the amortized cost basis of the security. In determining whether a credit loss exists related to impaired available-for-sale debt securities, the Company considers, among other factors, the extent of the unrealized loss relative to the amortized cost basis, the credit rating of the issuer and any recent changes thereto, current and expected future economic conditions, and any adverse events or other changes in circumstances that have occurred which may indicate a potential credit loss. The Company did not record an allowance for credit losses on its available-for-sale debt securities as of June 30, 2020.

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 income 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.

Recent Accounting Pronouncements

Recently Adopted Accounting Pronouncements

In June 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2016-13, *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*, which amends the accounting for credit losses for most financial assets and certain other instruments. The standard requires that entities holding financial assets that are not accounted for at fair value through net income be presented at the net amount expected to be collected by recording an allowance for credit losses. The allowance for credit losses will be a valuation account that will be deducted from the amortized cost basis of the financial asset to present the net carrying value at the amount expected to be collected on the financial asset. The standard also amends the impairment model for available-for-sale debt securities, requiring credit losses on impaired debt securities to be included in results of operations. The Company adopted this standard effective January 1, 2020 using a modified retrospective transition method, which requires a cumulative-effect adjustment, if any, to opening accumulated deficit on the adoption date. The adoption of this standard primarily impacts the Company's methodology used to assess credit losses on its accounts receivable, contract assets and available-for-sale debt securities. Based on the composition of the Company's accounts receivable, contract assets and available-for-sale debt securities, the adoption of this standard required no cumulative-effect adjustments and did not have a material impact on the Company's financial position or results of operations. Please refer to the significant accounting policies above for a description of the Company's accounting policies for accounts receivable and marketable securities upon the adoption of this standard.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*, which modifies certain disclosure requirements regarding fair value measurements. The Company adopted this standard effective January 1, 2020. The adoption of this standard did not have a material impact on the Company's financial statement disclosures.

In August 2018, the FASB issued ASU 2018-15, *Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*. The standard aligns the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software (and hosting arrangements that include an internal-use software license). The Company adopted this standard effective January 1, 2020 on a prospective basis. The Company has various cloud-based software applications accounted for as service contracts, the most significant of which is the Company's enterprise resource planning (ERP) system for which implementation was in progress on the adoption date of this standard. The adoption of this standard resulted in the capitalization of certain costs during the six months ended June 30, 2020 related to the implementation of the ERP system and other cloud-based software applications which would have been expensed as incurred prior to the adoption of this standard. The adoption of this standard did not have a material impact on the Company's financial position or results of operations.

In December 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740)—Simplifying the Accounting for Income Taxes*, which simplifies the current accounting for income taxes. Among other changes, the standard removes the exception to the incremental approach for intraperiod tax allocation when there is a loss from continuing operations and income or a gain from other items such as other comprehensive income. The Company early adopted this standard effective January 1, 2020, with certain aspects of the standard applied using the modified retrospective transition method and other aspects of the standard applied on a prospective basis. The adoption of this standard required no cumulative-effect adjustments and did not have a material impact on the Company's financial position or results of operations.

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 / Cost	Unrealized Gains	Unrealized Losses	Fair Value
June 30, 2020				
U.S. government and federal agency securities	$ 34,315	$ 173	$ —	$ 34,488
Certificates of deposit	6,311	82	—	6,393
Corporate bonds	178,494	1,399	(43)	179,850
Equity securities	282	24,005	—	24,287
	$ 219,402	$ 25,659	$ (43)	$ 245,018

	Amortized Cost / Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2019				
U.S. government and federal agency securities	$ 62,637	$ 215	$ (5)	$ 62,847
Certificates of deposit	8,506	77	—	8,583
Corporate bonds	226,137	808	(29)	226,916
Equity securities	351	31,784	—	32,135
	$ 297,631	$ 32,884	$ (34)	$ 330,481

As of June 30, 2020 and December 31, 2019, no available-for-sale debt securities had remaining maturities greater than three years. The amortized cost of marketable debt securities is adjusted for amortization of premiums and accretion of discounts to maturity, or to the earliest call date for callable debt securities purchased at a premium.

As of June 30, 2020 and December 31, 2019, the balance in the Company's accumulated other comprehensive income consisted solely of net unrealized gains and losses on available-for-sale debt securities, net of income tax effects and reclassification adjustments for realized gains and losses. During the three and six months ended June 30, 2020, the Company recognized net unrealized gains on available-for-sale debt securities of $1.3 million and $0.6 million, respectively, and income tax expense of zero in other comprehensive income for the period. The Company recognized net realized gains (losses) of less than $(0.1) million and less than $0.1 million on the sale or maturity of available-for-sale debt securities during the three and six months ended June 30, 2020, which were reclassified out of accumulated other comprehensive income during the period and were included in investment income in the consolidated statements of operations and comprehensive loss. During the three and six months ended June 30, 2019, the Company recognized net unrealized gains on available-for-sale debt securities of $0.8 million and $1.8 million, respectively, and income tax expense of $0.3 million and $0.7 million, respectively, in other comprehensive income for the period. The Company recognized net realized gains of zero and less than $0.1 million on the sale or maturity of available-for-sale debt securities during the three and six months ended June 30, 2019, which were reclassified out of accumulated other comprehensive income during the period and were included in investment income in the consolidated statements of operations and comprehensive loss.

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

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
June 30, 2020						
Corporate bonds	$ 42,913	$ (43)	$ —	$ —	$ 42,913	$ (43)
	$ 42,913	$ (43)	$ —	$ —	$ 42,913	$ (43)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2019						
U.S. government and federal agency securities	$ 12,562	$ (5)	$ —	$ —	$ 12,562	$ (5)
Corporate bonds	48,556	(29)	—	—	48,556	(29)
	$ 61,118	$ (34)	$ —	$ —	$ 61,118	$ (34)

As of June 30, 2020, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 11 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 has not identified any credit losses associated with its available-for-sale debt securities. The Company did not recognize any impairment or credit losses on available-for-sale debt securities during the three and six months ended June 30, 2020.

Marketable equity securities held by the Company as of June 30, 2020 and December 31, 2019 consisted solely of common stock of Prevail Therapeutics Inc. (Prevail). The Company acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its initial public offering (IPO) in June 2019. Prior to Prevail's IPO, the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of $0.4 million. Upon Prevail's IPO in June 2019, the securities were reclassified to marketable securities and are measured at fair value. During the three and six months ended June 30, 2020, the Company recognized net realized and unrealized gains (losses) of $4.4 million and $(0.7) million, respectively, related to its marketable equity securities of Prevail. During the three and six months ended June 30, 2019, the Company recognized unrealized gains of $31.7 million and did not recognize any realized gains or losses related to its marketable equity securities of Prevail.

4. Fair Value of Financial Instruments

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

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
June 30, 2020				
Cash equivalents:				
Money market mutual funds	$ —	$ 84,473	$ —	$ 84,473
Total cash equivalents	—	84,473	—	84,473
Marketable securities:				
U.S. government and federal agency securities	—	34,488	—	34,488
Certificates of deposit	—	6,393	—	6,393
Corporate bonds	—	179,850	—	179,850
Equity securities	24,287	—	—	24,287
Total marketable securities	24,287	220,731	—	245,018
Total cash equivalents and marketable securities	$ 24,287	$ 305,204	$ —	$ 329,491

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2019				
Cash equivalents:				
Money market mutual funds	$ —	$ 56,058	$ —	$ 56,058
Total cash equivalents	—	56,058	—	56,058
Marketable securities:				
U.S. government and federal agency securities	—	62,847	—	62,847
Certificates of deposit	—	8,583	—	8,583
Corporate bonds	—	226,916	—	226,916
Equity securities	32,135	—	—	32,135
Total marketable securities	32,135	298,346	—	330,481
Total cash equivalents and marketable securities	$ 32,135	$ 354,404	$ —	$ 386,539

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 significantly different from those that would be used as of June 30, 2020 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 June 30, 2020, non-marketable equity securities had a carrying value of $1.1 million and were included in other assets on the consolidated balance sheet. As of December 31, 2019, the Company did not hold any non-marketable equity securities. No remeasurements or impairment losses were recorded on non-marketable equity securities during the three and six months ended June 30, 2020 and 2019.

5. Property and Equipment, Net

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

	June 30, 2020	December 31, 2019
Lab equipment	$ 21,315	$ 19,663
Computer equipment and software	3,028	2,545
Furniture and fixtures	2,897	2,188
Leasehold improvements	23,884	18,915
Total property and equipment	51,124	43,311
Accumulated depreciation and amortization	(18,428)	(14,338)
Property and equipment, net	$ 32,696	$ 28,973

6. License and Royalty Revenue

As of June 30, 2020, the Company's NAV Technology Platform was being applied by NAV Technology Licensees in one commercial product, Zolgensma, and in the development of more than 20 product candidates. 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 June 30, 2020, 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 $293.4 million, including (i) $0.3 million upon the submission of preclinical regulatory filings, (ii) $26.6 million upon the commencement of various stages of clinical trials, (iii) $26.0 million upon the submission of regulatory approval filings, (iv) $103.5 million upon the approval of commercial products by regulatory agencies and (v) $137.0 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.

The following tables present changes in the balances of the Company's receivables, contract assets and contract liabilities during the periods presented (in thousands):

	Balance at Beginning of Period	Additions	Deductions	Balance at End of Period
Three Months Ended June 30, 2020				
Receivables and contract assets:				
Accounts receivable, current and non-current	$ 48,895	$ 18,834	$ (21,235)	$ 46,494
Contract assets	$ 350	$ —	$ —	$ 350
Contract liabilities:				
Deferred revenue, current and non-current	$ 3,333	$ 1,124	$ —	$ 4,457

	Balance at Beginning of Period	Additions	Deductions	Balance at End of Period
Six Months Ended June 30, 2020				
Receivables and contract assets:				
Accounts receivable, current and non-current	$ 42,303	$ 36,976	$ (32,785)	$ 46,494
Contract assets	$ —	$ 350	$ —	$ 350
Contract liabilities:				
Deferred revenue, current and non-current	$ 3,333	$ 1,124	$ —	$ 4,457

	Balance at Beginning of Period	Additions	Deductions	Balance at End of Period
Three Months Ended June 30, 2019				
Receivables and contract assets:				
Accounts receivable, current and non-current	$ 31,130	$ 9,312	$ (6,808)	$ 33,634
Contract assets	$ 1,000	$ —	$ (1,000)	$ —
Contract liabilities:				
Deferred revenue, current and non-current	$ 3,933	$ —	$ (600)	$ 3,333

	Balance at Beginning of Period	Additions	Deductions	Balance at End of Period
Six Months Ended June 30, 2019				
Receivables and contract assets:				
Accounts receivable, current and non-current	$ 31,599	$ 11,049	$ (9,014)	$ 33,634
Contract assets	$ 750	$ 1,000	$ (1,750)	$ —
Contract liabilities:				
Deferred revenue, current and non-current	$ 3,933	$ —	$ (600)	$ 3,333

Additions to accounts receivable during the three and six months ended June 30, 2020 primarily consisted of royalties on net sales of Zolgensma of $11.9 million and $21.9 million, respectively, receivables recorded related to new licenses granted by the Company, amounts billed upon the achievement of development milestones by licensees during the periods, and interest income recognized during the periods related to significant financing components. Additions to accounts receivable during the three and six months ended June 30, 2019 primarily consisted of receivables recorded related to new licenses granted by the Company, amounts billed upon the achievement of development milestones by licensees during the periods, and interest income recognized during the periods related to significant financing components. Deductions to accounts receivable during the three and six months ended June 30, 2020 and 2019 primarily consisted of amounts collected from licensees during the periods.

The changes in the balances of contract assets during the three and six months ended June 30, 2020 and 2019 consist of development milestones deemed probable of achievement by licensees during the period, offset by the subsequent achievement of such milestones and billing of the associated milestone payments by the Company.

As of June 30, 2020, the Company had recorded deferred revenue of $4.5 million which represents consideration received from licensees for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations consist of (i) options granted to licensees that provide material rights to the licensee to acquire additional licenses from the Company, and (ii) research and development services to be performed by the Company related to licensed products. These performance obligations will be satisfied, and underlying revenue will be recognized, upon the exercise or expiration of the options or performance of the research and development services. The Company did not recognize any revenue during the three and six months ended June 30, 2020 that was included in deferred revenue at the beginning of the period. During the three and six months ended June 30, 2019, the Company recognized $0.6 million of revenue that was included in deferred revenue at the beginning of the period as a result of options exercised by licensees during the period.

During the three and six months ended June 30, 2020, the Company recognized revenue of $16.4 million and $26.8 million, respectively, from performance obligations satisfied in prior periods as a result of changes in the transaction prices of its license agreements as well as royalties on sales of licensed products and sublicense fees. During the three and six months ended June 30, 2019, the Company recognized revenue of $4.2 million and $5.0 million, respectively, from performance obligations satisfied in prior periods as a result of changes in the transaction prices of its licenses agreements as well as royalties on sales of licensed products and sublicense fees. Changes in transaction prices during the periods were primarily attributable to development milestones achieved or deemed probable of achievement during the period that were previously not considered probable of achievement.

As of June 30, 2020, the Company had recorded total current and non-current accounts receivable of $46.5 million, of which $30.0 million had been billed to customers and $16.5 million was billable to customers in future periods. As of December 31, 2019, the Company had recorded total current and non-current accounts receivable of $42.3 million, of which $0.4 million had been billed to customers and $41.9 million was billable to customers in future periods. Based on the Company's evaluation of the credit quality and financial condition of its significant customers, history of collections and evaluation of current and future expected economic conditions, no credit losses were recognized on accounts receivable or contract assets during the three and six months ended June 30, 2020.

AveXis March 2014 License

In March 2014, the Company entered into an exclusive license agreement, as amended in January 2018 (the March 2014 License) with AveXis, Inc. (AveXis). Under the March 2014 License, the Company granted AveXis 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. AveXis launched commercial sales of Zolgensma in the second quarter of 2019, which is a licensed product under the March 2014 License. Upon the commencement of commercial sales in the second quarter of 2019, the Company began recognizing royalty revenue on net sales of Zolgensma.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
License revenue	$ 3,500	$ 3,500	$ 3,500	$ 3,500
Zolgensma royalty revenue	11,945	924	21,924	924
Total license and royalty revenue	$ 15,445	$ 4,424	$ 25,424	$ 4,424
Interest income from licensing	$ 6	$ 7	$ 13	$ 15

As of June 30, 2020, the Company had recorded $12.1 million of accounts receivable from AveXis under the March 2014 License, of which $11.9 million were included in current assets and $0.2 million were included in non-current assets. As of December 31, 2019, the Company had recorded $11.0 million of accounts receivable from AveXis under the March 2014 License, of which $10.8 million were included in current assets and $0.2 million were included in non-current assets.

Abeona Therapeutics Inc.

Accounts receivable as of June 30, 2020 and December 31, 2019 included $28.8 million and $26.3 million, respectively, related to the license agreement entered into in November 2018 between the Company and Abeona Therapeutics Inc. (Abeona), as amended in November 2019 (the November 2018 License) for the development and commercialization of treatments for various diseases, all of which were included in current assets.

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 written demand for payment and breach notice. 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 July 31, 2020, 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. The Company recognized interest income from licensing of $0.8 million during the three and six months ended June 30, 2020 related to the unpaid license fees from Abeona under the November 2018 License, which is included in the $28.8 million of accounts receivable due from Abeona recorded as of June 30, 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 has filed a counterclaim in arbitration demanding payment of the $28.0 million of unpaid fees from Abeona, plus accrued interest. As of June 30, 2020, the Company had not recorded any liabilities related to this matter as, based on its evaluation of the merits of Abeona's claims, the Company believes its risk of loss is remote. Additionally, the Company evaluated the collectability of the $28.8 million due from Abeona and determined that no allowance for doubtful accounts should be recorded as of June 30, 2020, as the Company intends to enforce the collection of all amounts due from Abeona and, based on its evaluation of the merits of Abeona's claims, the Company expects to receive payment in full upon the completion of arbitration. However, the duration of the arbitration and timing of payment from Abeona are unpredictable. In accordance with its interest accrual policy, the Company will continue to accrue interest income on the unpaid balance due from Abeona under the November 2018 License until payment has been received or is otherwise no longer expected to be collected.

7. Stock-based Compensation

In January 2020, the Board of Directors authorized an additional 1,479,696 shares to be issued under the 2015 Equity Incentive Plan (the 2015 Plan). As of June 30, 2020, the total number of shares of common stock authorized for issuance under the 2015 Plan and the 2014 Stock Plan (the 2014 Plan) was 12,412,917, of which 2,309,229 remained available for future grants under the 2015 Plan.

Stock-based Compensation Expense

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Stock options	$ 8,086	$ 6,844	$ 15,865	$ 12,296
Restricted stock units	—	69	—	136
Employee stock purchase plan	230	186	468	385
	$ 8,316	$ 7,099	$ 16,333	$ 12,817

As of June 30, 2020, the Company had $77.3 million of unrecognized stock-based compensation expense related to stock options and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 2.7 years.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Research and development	$ 4,284	$ 3,526	$ 8,331	$ 5,873
General and administrative	4,032	3,573	8,002	6,944
	$ 8,316	$ 7,099	$ 16,333	$ 12,817

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, 2019	5,544	$ 28.79	7.5	$ 86,509
Granted	1,448	$ 38.68		
Exercised	(281)	$ 14.14		
Cancelled or forfeited	(215)	$ 45.93		
Outstanding at June 30, 2020	6,496	$ 31.06	7.6	$ 64,890
Exercisable at June 30, 2020	3,387	$ 21.79	6.4	$ 60,687
Vested and expected to vest at June 30, 2020	6,496	$ 31.06	7.6	$ 64,890

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

The weighted-average grant date fair value per share of options granted during the six months ended June 30, 2020 was $24.35. During the six months ended June 30, 2020, the total number of stock options exercised was 281,468, resulting in total proceeds of $4.0 million. The total intrinsic value of options exercised during the six months ended June 30, 2020 was $7.5 million.

Employee Stock Purchase Plan

In January 2020, the Board of Directors authorized an additional 369,924 shares to be issued under the 2015 ESPP. As of June 30, 2020, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 623,924, of which 486,068 remained available for future issuance. During the six months ended June 30, 2020, 17,442 shares of common stock were issued under the 2015 ESPP.

8. 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 a three-year cumulative loss position as of June 30, 2020 and December 31, 2019, 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 June 30, 2020 and December 31, 2019.

In response to the COVID-19 pandemic, the Coronavirus Aid, Relief and Economic Security Act (the CARES Act) was signed into law in March 2020. The CARES Act (i) lifts certain deduction limitations originally imposed by the Tax Cuts and Jobs Act of 2017 (the TCJA), (ii) allows corporate taxpayers to carryback net operating losses (NOLs) originating during 2018 through 2020 for up to five years, which was not previously allowed under the TCJA, (iii) eliminates the 80% of taxable income limitations on NOL utilization imposed by the TCJA, allowing corporate entities to fully utilize NOL carryforwards to offset taxable income in 2018, 2019 or 2020, and (iv) enacts various other changes to corporate taxation. Also included in the CARES Act was a change to the TCJA related to qualified improvement property, retroactively allowing for a 15-year recovery period and bonus depreciation. As a result of this change, the Company recorded current income tax benefit of $0.5 million during the three and six months ended June 30, 2020 related to a reduction of state taxes associated with additional depreciation deductions allowed for the 2018 tax year. Overall, the enactment of the CARES Act, including the change for qualified improvement property, did not result in any material adjustments to the Company's income tax provision for the three and six months ended June 30, 2020, or to the Company's net deferred tax assets as of June 30, 2020.

9. 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 services provided and increase the monthly fee, and the amended agreement expires in December 2020. Expenses incurred under the agreements with FOXKISER for the three and six months ended June 30, 2020 were $1.2 million and $2.4 million, respectively. Expenses incurred under the agreements with FOXKISER for the three and six months ended June 30, 2019 were $0.9 million and $1.7 million, respectively. Expenses incurred under the agreements with FOXKISER were recorded as research and development expenses in the consolidated statements of operations and comprehensive loss.

10. Net Loss Per Share

Since the Company incurred net losses for the three and six months ended June 30, 2020 and 2019, 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 and Six Months Ended June 30,	
	2020	2019
Stock options issued and outstanding	6,496	5,454
Unvested restricted stock units outstanding	—	40
Employee stock purchase plan	38	26
	6,534	5,520

11. Supplemental Disclosures

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

	June 30, 2020	December 31, 2019
Accrued personnel costs	$ 9,356	$ 10,903
Accrued external research and development expenses	6,832	5,791
Accrued sublicense fees and royalties	6,602	4,542
Accrued external general and administrative expenses	2,069	2,053
Accrued purchases of property and equipment	887	1,328
Other accrued expenses and current liabilities	93	229
	$ 25,839	$ 24,846

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, 2019, which we filed with the SEC on February 26, 2020. 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, 2019 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.

  We have enrolled 42 patients in the Phase I/IIa clinical trial of RGX-314 for the treatment of wet AMD and have reported data for all five dose level cohorts. We expect to initiate a pivotal program for the subretinal delivery of RGX-314 for the treatment of wet AMD in the second half of 2020.

  We expect to dose the first patient in a Phase II trial for the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet AMD (AAVIATE) in the third quarter of 2020 and plan to report interim data from the first cohort of the trial by the end of 2020. Additionally, we expect to initiate a Phase II trial of the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of DR in the second half of 2020, and expect to report interim data 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 the second half of 2020.

- **AAV-Mediated Antibody Expression for the Treatment of Neurodegenerative Diseases:** We continue to collaborate with Neurimmune AG (Neurimmune) to jointly develop 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 the second half of 2020.

Gene therapy programs for the potential treatment of rare monogenic diseases

- **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 (IDS), an enzyme that is responsible for breakdown of cellular waste products. Initial data from the first cohort demonstrated consistent and sustained reduction in heparan sulfate (HS) in the cerebral spinal fluid (CSF) and available data support early signs of neurocognitive stability. We have completed enrollment in the second cohort and expect to report interim data from both cohorts and a program update in the second half of 2020.
- **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. Recruitment and patient screening are ongoing in the Phase I/II clinical trial for RGX-111. We expect to provide a program update by the end of 2020.
- **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. We expect to submit an Investigational New Drug (IND) application for the intracisternal delivery of RGX-181 by the end of 2020, and plan to initiate enrollment in a Phase I/II trial in the first half of 2021.
- **RGX-381:** RGX-381 is a new program targeting the ocular manifestations of CLN2 disease in patients and is designed to use the AAV9 vector to deliver the TPP1 gene directly to the retina. We believe that one-time administration of RGX-381 could provide a durable source of TPP1 activity in the retina, thereby potentially preventing visual decline. There is currently no available treatment for ocular manifestations of CLN2 disease. We expect to submit an IND application for a Phase I/II study of RGX-381 in patients with CLN2 disease in the second half of 2020 and initiate enrollment in the first half of 2021.
- **RGX-501:** We have discontinued internal clinical development of RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH) and plan to evaluate strategic alternatives to support the continued advancement of this program.
- **Gene Therapy Research Program for the Treatment of Neuromuscular Disorders:** We expect to announce plans for the clinical development of a potential treatment for a neuromuscular disorder in the second half of 2020.

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.

RGX-314 Interim Update

In our Phase I/IIa trial for RGX-314, all 42 patients with wet AMD had received a single administration of RGX-314 across five dose cohorts. To qualify for inclusion in the trial, participants were required to have a history of frequent anti-vascular endothelial growth factor (VEGF) treatments (including at least four anti-VEGF injections in the eight months preceding trial enrollment) and a documented history of response to anti-VEGF therapy. The trial design included doses of 3×10^9 (Cohort 1), 1×10^{10} (Cohort 2), 6×10^{10} (Cohort 3), 1.6×10^{11} (Cohort 4) and 2.5×10^{11} (Cohort 5) genome copies (GC)/eye. Patients will be assessed every four weeks to the six-month primary endpoint, with long-term follow-up continuing for two years. Below is a summary of the interim results of our Phase I/IIa trial as of July 13, 2020:

- RGX-314 was generally well-tolerated at all dose levels, with one possibly drug-related serious adverse event (SAE) of significant decrease in vision reported in Cohort 5.
- In Cohorts 4 and 5, durable treatment effect was observed, with stable to improved visual acuity and retinal thickness, and meaningful reductions in anti-VEGF treatment burden were demonstrated over one year.
- Patients in Cohort 4 and Cohort 5 at one year after administration of RGX-314 demonstrated stable visual acuity with a mean BCVA change of +4 letters and -2 letters from baseline, respectively, as well as decreased retinal thickness, with a mean change in CRT of -61 µm and -79 µm, respectively.
- Patients in Cohort 4 received a mean of 4.1 injections over one year following administration of RGX-314, a 61% reduction in treatment burden. Patients in Cohort 5 received a mean of 1.4 injections over one year following administration of RGX-314, a reduction in treatment burden of 85%.

- In Cohort 4, three out of twelve (25%) patients received no anti-VEGF injections over one year, and these patients demonstrated a mean BCVA improvement of +6 letters and a mean reduction in CRT of -62 µm at one year. In Cohort 5, eight out of the eleven (73%) patients observed through one year have received no anti-VEGF injections after administration of RGX-314 and these patients demonstrated a stable mean BCVA change of 0 letters and a mean reduction in CRT of -95 µm at one year.
- Intraocular RGX-314 protein levels at one year demonstrated dose-dependent expression across cohorts.

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively license the NAV Technology Platform to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of June 30, 2020, our NAV Technology Platform was being applied in one commercially approved product (Zolgensma®), and many partnered product candidates are in development, several of which are in active clinical development. 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 could require us to delay or prevent us from proceeding with our clinical trials and other business initiatives, such as preclinical development and manufacturing operations. For example, the ongoing construction of our future corporate, manufacturing and research headquarters in Rockville, Maryland is expected to be delayed 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 and six months ended June 30, 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 at this time. Please refer to the “Risk Factors” section of this Quarterly Report on Form 10-Q 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 of royalties on net sales of Zolgensma, which is marketed by AveXis, Inc. (AveXis), a wholly owned subsidiary of Novartis AG (Novartis), for the treatment of spinal muscular atrophy (SMA). Zolgensma is a licensed product under our March 2014 License with AveXis 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, stock-based compensation and travel, 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/IIa clinical trial and a planned pivotal program to evaluate the safety and efficacy of the subretinal delivery of RGX-314 for the treatment of wet AMD;
- planned 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;
- a Phase I/II clinical trial 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 to treat neuromuscular disorders;
- completion of a long-term follow-up study for patients dosed in the Phase I/II clinical trial for RGX-501 as we evaluate strategic alternatives to support the continued advancement of this program;
- 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 and six months ended June 30, 2020 and 2019 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Direct Expenses				
RGX-314	$ 3,584	$ 3,582	$ 9,717	$ 5,986
RGX-121	2,340	299	4,680	2,073
RGX-181	440	3,007	1,158	5,303
RGX-501	1,628	1,066	2,364	2,065
Other product candidates	4,821	767	6,900	1,608
Total direct expenses	12,813	8,721	24,819	17,035
Unallocated Expenses				
Platform and new technologies	6,246	5,459	12,178	8,855
Personnel-related	16,162	12,241	32,021	22,815
Facilities and depreciation expense	2,670	2,290	5,322	4,496
Other unallocated	220	772	806	1,485
Total unallocated expenses	25,298	20,762	50,327	37,651
Total research and development	$ 38,111	$ 29,483	$ 75,146	$ 54,686

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 employee travel, 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

Interest Income from Licensing

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

Investment Income

Investment income consists of interest income earned and gains and losses realized from our cash equivalents 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.

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 financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our financial statements, as well as the reported revenues and expenses during the reported periods. We evaluate these estimates and assumptions on an ongoing basis. 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 that are not readily apparent from other sources. Actual results may differ 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 which are included in our Annual Report on Form 10-K for the year ended December 31, 2019. There have been no significant changes in our critical accounting policies since December 31, 2019.

Recent Accounting Pronouncements

See Note 2 "Recent Accounting Pronouncements" in the notes to the accompanying unaudited consolidated financial statements for a full description of accounting pronouncements which we have recently adopted and the impact to our financial statements upon adoption.

Results of Operations

	Three Months Ended June 30,			Six Months Ended June 30,		
	2020	2019	Change	2020	2019	Change
	(in thousands)					
Revenues						
License and royalty revenue	$ 16,566	$ 7,881	$ 8,685	$ 34,210	$ 8,765	$ 25,445
Total revenues	16,566	7,881	8,685	34,210	8,765	25,445
Operating Expenses						
Cost of revenues	4,684	1,927	2,757	8,093	1,956	6,137
Research and development	38,111	29,483	8,628	75,146	54,686	20,460
General and administrative	15,554	13,405	2,149	30,387	24,963	5,424
Other operating expenses (income)	50	(62)	112	117	(62)	179
Total operating expenses	58,399	44,753	13,646	113,743	81,543	32,200
Loss from operations	(41,833)	(36,872)	(4,961)	(79,533)	(72,778)	(6,755)
Other Income						
Interest income from licensing	1,849	762	1,087	2,697	1,375	1,322
Investment income	5,722	34,524	(28,802)	2,536	37,519	(34,983)
Total other income	7,571	35,286	(27,715)	5,233	38,894	(33,661)
Loss before income taxes	(34,262)	(1,586)	(32,676)	(74,300)	(33,884)	(40,416)
Income Tax Benefit	500	129	371	500	199	301
Net loss	$ (33,762)	$ (1,457)	$ (32,305)	$ (73,800)	$ (33,685)	$ (40,115)

Comparison of the Three Months Ended June 30, 2020 and 2019

License and Royalty Revenue. License and royalty revenue increased by $8.7 million, from $7.9 million for the three months ended June 30, 2019 to $16.6 million for the three months ended June 30, 2020. The increase was primarily attributable to an $11.0 million increase in Zolgensma royalty revenue. Commercial sales of Zolgensma commenced in the second quarter of 2019, and we are eligible to receive a milestone payment of $80.0 million from AveXis upon the achievement of $1.0 billion in cumulative net sales of Zolgensma. Cumulative net sales of Zolgensma through June 30, 2020 were more than $735.0 million.

The increase in license and royalty revenue for the three months ended June 30, 2020 was partially offset by non-recurring revenue recognized during the three months ended June 30, 2019 resulting from license options exercised by licensees during the period.

Research and Development Expense. Research and development expenses increased by $8.6 million, from $29.5 million for the three months ended June 30, 2019 to $38.1 million for the three months ended June 30, 2020. The increase was primarily attributable to the following:

- an increase of $3.9 million for personnel-related costs as a result of increased headcount of research and development personnel, including a $0.8 million increase in stock-based compensation expense;
- an increase of $2.2 million for external costs associated with clinical trial and regulatory activities;
- an increase of $1.5 million for laboratory costs and facilities used by research and development personnel, including a $0.3 million increase in depreciation expense allocated to research and development functions;
- an increase of $1.1 million for external costs associated with manufacturing-related services to support the ongoing development of our product candidates and process development activities; and
- an increase of $0.6 million for external costs associated with preclinical studies and other early-stage research and development.

General and Administrative Expense. General and administrative expenses increased by $2.1 million, from $13.4 million for the three months ended June 30, 2019 to $15.6 million for the three months ended June 30, 2020. The increase was primarily attributable to the following:

- an increase of $1.4 million for professional services, primarily related to commercial consulting and legal services; and

- an increase of $0.7 million for personnel-related costs as a result of increased headcount of general and administrative personnel, including a $0.5 million increase in stock-based compensation expense.

Investment Income. Investment income decreased by $28.8 million, from $34.5 million for the three months ended June 30, 2019 to $5.7 million for the three months ended June 30, 2020. The decrease was primarily attributable to an unrealized gain of $31.7 million recognized during the three months ended June 30, 2019 related to our marketable equity securities of Prevail Therapeutics Inc. (Prevail). We acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its initial public offering (IPO) in June 2019. Prior to Prevail's IPO, the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of $0.4 million. Upon Prevail's IPO in June 2019, the securities were reclassified to marketable securities and are measured at fair value. The decrease in investment income was partially offset by net realized and unrealized gains of $4.4 million recognized during the three months ended June 30, 2020 related to our marketable equity securities of Prevail. As of June 30, 2020, our marketable equity securities of Prevail had a fair value of $24.3 million. Significant fluctuations in the fair value of the securities may continue to occur from period to period.

Comparison of the Six Months Ended June 30, 2020 and 2019

License and Royalty Revenue. License and royalty revenue increased by $25.4 million, from $8.8 million for the six months ended June 30, 2019 to $34.2 million for the six months ended June 30, 2020. The increase was primarily attributable to a $21.0 million increase in Zolgensma royalty revenue. Commercial sales of Zolgensma commenced in the second quarter of 2019, and we are eligible to receive a milestone payment of $80.0 million from AveXis upon the achievement of $1.0 billion in cumulative net sales of Zolgensma. Cumulative net sales of Zolgensma through June 30, 2020 were more than $735.0 million.

Research and Development Expense. Research and development expenses increased by $20.5 million, from $54.7 million for the six months ended June 30, 2019 to $75.1 million for the six months ended June 30, 2020. The increase was primarily attributable to the following:

- an increase of $9.1 million for personnel-related costs as a result of increased headcount of research and development personnel, including a $2.5 million increase in stock-based compensation expense;
- an increase of $3.6 million for external costs associated with manufacturing-related services to support the ongoing development of our product candidates and process development activities;
- an increase of $3.6 million for external costs associated with clinical trial and regulatory activities;
- an increase of $3.2 million for laboratory costs and facilities used by research and development personnel, including a $0.5 million increase in depreciation expense allocated to research and development functions; and
- an increase of $1.4 million for external costs associated with preclinical studies and other early-stage research and development.

General and Administrative Expense. General and administrative expenses increased by $5.4 million, from $25.0 million for the six months ended June 30, 2019 to $30.4 million for the six months ended June 30, 2020. The increase was primarily attributable to the following:

- an increase of $2.9 million for professional services, primarily related to commercial consulting and legal services; and
- an increase of $1.9 million for personnel-related costs as a result of increased headcount of general and administrative personnel, including a $1.1 million increase in stock-based compensation expense.

Investment Income. Investment income decreased by $35.0 million, from $37.5 million for the six months ended June 30, 2019 to $2.5 million for the six months ended June 30, 2020. The decrease was primarily attributable to an unrealized gain of $31.7 million recognized during the six months ended June 30, 2019 related to our marketable equity securities of Prevail. We acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its IPO in June 2019. Prior to Prevail's IPO, the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of $0.4 million. Upon Prevail's IPO in June 2019, the securities were reclassified to marketable securities and are measured at fair value. As of June 30, 2020, our marketable equity securities of Prevail had a fair value of $24.3 million. Significant fluctuations in the fair value of the securities may continue to occur from period to period.

Liquidity and Capital Resources

As of June 30, 2020, we had cash, cash equivalents and marketable securities of $339.2 million, which were primarily derived from the sale of our common stock as well as revenues generated from the licensing of our NAV Technology Platform. We expect that our cash, cash equivalents and marketable securities as of June 30, 2020 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.

Commercial sales of Zolgensma commenced in the second quarter of 2019, upon which we began recognizing royalty revenue on net sales of the licensed product. We are eligible to receive a milestone payment of $80.0 million from AveXis upon the achievement of $1.0 billion in cumulative net sales of Zolgensma. Cumulative net sales of Zolgensma through June 30, 2020 were more than $735.0 million. However, there is no guarantee that the net sales milestone will be achieved.

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. 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 uncertainty and rapidly changing 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

	Six Months Ended June 30,	
	2020	**2019**
	(in thousands)	
Net cash used in operating activities	$ (57,069)	$ (53,046)
Net cash provided by investing activities	77,191	27,133
Net cash provided by financing activities	4,586	5,494
Net increase (decrease) in cash and cash equivalents and restricted cash	$ 24,708	$ (20,419)

Cash Flows from Operating Activities

Our net cash used in operating activities for the six months ended June 30, 2020 increased by $4.0 million from the six months ended June 30, 2019. The increase was primarily attributable to an increase in operating expenses of $32.2 million during the six months ended June 30, 2020, offset primarily by an increase in license and royalty payments received during this period. The increase in operating expenses during the period was primarily attributable to increased employee headcount and external research and development expenses as we continue the development and advancement of our lead product candidates and other research programs.

For the six months ended June 30, 2020, our net cash used in operating activities of $57.1 million consisted of a net loss of $73.8 million and changes in working capital of $3.1 million, offset by $19.8 million in adjustments for non-cash items. The changes in working capital include an increase in accounts receivable of $2.7 million which was largely driven by an increase in unbilled Zolgensma royalties during the period. Other changes in working capital were incurred in the normal course of business, primarily as a result of the timing of invoices from and payments to suppliers, prepayments to suppliers, and accrued liabilities for unbilled goods and services from suppliers and personnel-related costs. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of $16.3 million, depreciation and amortization expense of $4.1 million, and an unrealized loss on our marketable equity securities of Prevail, net of realized gains, of $0.7 million, and were partially offset by imputed interest earned from our license agreements of $1.9 million.

For the six months ended June 30, 2019, our net cash used in operating activities of $53.0 million consisted of a net loss of $33.7 million, $17.2 million in adjustments for non-cash items and changes in working capital of $2.2 million. Adjustments for non-cash items primarily consisted of an unrealized gain on our marketable equity securities of Prevail of $31.7 million, imputed interest earned from our license agreements of $1.4 million and net accretion of discounts on marketable debt securities of $0.8 million, and were partially offset by stock-based compensation expenses of $12.8 million and depreciation and amortization expense of $3.4 million. The changes in working capital include an increase in accounts receivable of $1.4 million and a decrease in deferred revenue of $0.6 million, which were largely driven by new licenses we granted as a result of license options exercised by licensees during the period. Other changes in working capital were incurred in the normal course of business, primarily as a result of the timing of invoices

from and payments to suppliers, prepayments to suppliers, and accrued liabilities for unbilled goods and services from suppliers and personnel-related costs.

Cash Flows from Investing Activities

For the six months ended June 30, 2020, net cash provided by investing activities consisted of $155.8 million in sales and maturities of marketable securities, offset by $70.7 million to purchase marketable securities and $7.9 million to purchase property and equipment. We expect capital expenditures to increase in the second half of 2020 and in 2021 as a result of the buildout of our future corporate, manufacturing and research headquarters at 9804 Medical Center Drive in Rockville, Maryland. Total remaining capital expenditures related to the build out of the facility at 9804 Medical Center Drive, net of amounts to be reimbursed by the landlord under our tenant improvement allowance, are expected to be in the upper double-digit millions (USD) and are expected to be incurred through 2022. However, the actual amount and timing of these capital expenditures are uncertain and may differ materially from our current estimates.

For the six months ended June 30, 2019, net cash provided by investing activities consisted of $141.3 million in sales and maturities of marketable securities, offset by $106.1 million to purchase marketable securities and $8.0 million to purchase property and equipment.

Cash Flows from Financing Activities

For the six months ended June 30, 2020, net cash provided by financing activities consisted of $4.6 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

For the six months ended June 30, 2019, net cash provided by financing activities consisted of $5.5 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 $251.6 million as of June 30, 2020. 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 increase significantly in the future for costs associated with building out additional office, laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

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

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;
- the results of our preclinical studies for our product candidates and any subsequent clinical trials;
- our planned expansion of the licensing of our NAV Technology Platform;

- 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 impact of the COVID-19 pandemic on our business, operations and preclinical and clinical development timelines and plans;
- 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 June 30, 2020 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, 2019.

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 most recent Annual Report on Form 10-K for the year ended December 31, 2019. There have been no material changes to our exposure to market risk during the six months ended June 30, 2020.

Item 4. Controls and Procedures.

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2020. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of June 30, 2020, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the quarter ended June 30, 2020 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.

Pursuant to the terms of the License Agreement dated November 4, 2018 between the Company and Abeona Therapeutics Inc. (Abeona), as amended on November 4, 2019 (the License Agreement), Abeona was required to make a payment of $8.0 million to us no later than April 1, 2020. Abeona failed to make this payment and we therefore delivered to Abeona a written demand for payment and breach notice in April 2020. Upon expiration of the applicable cure period under the License Agreement, which expiration occurred on May 2, 2020, the License Agreement was terminated. Upon termination, all rights and licenses granted to Abeona under the License Agreement terminated and an additional $20.0 million fee that would have otherwise been due to us in November 2020 became payable within 15 days of the termination date. We have not yet received payment for any portion of the fees due from Abeona.

In May 2020, Abeona filed a claim in arbitration alleging we breached certain responsibilities to communicate with Abeona regarding our prosecution of licensed patents under the License Agreement. We dispute Abeona's claim and have filed a counterclaim in arbitration for the $28.0 million total unpaid fees, plus interest, which accrues at a rate of 1.5% per month under the License Agreement. We have not recorded any liabilities related to this matter, as we believe our risk of loss is remote. Additionally, we intend to enforce the collection of all amounts owed from Abeona and, based on our evaluation of the merits of the claims, we expect to receive payment in full upon the completion of arbitration. However, we may ultimately receive less than the full amount we believe we are owed, and the duration of the arbitration and the timing of payment from Abeona, if any, are unpredictable. Any such adverse result or delay in payment may have a material adverse effect on our business, financial condition, results of operations or cash flows.

For more information, refer to Note 6, "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, 2019. There have been no material changes from the risk factors previously disclosed in such filing, except as follows:

Risks Related to our NAV Technology Platform and the Development of Our Product Candidates

Our business, operations and preclinical and clinical development timelines and plans could be adversely affected by the effects of the COVID-19 pandemic, including a possible resurgence or second wave of infections, and other public health crises.

The COVID-19 pandemic and other public health crises in regions where we have clinical trial sites or other business operations could have a material adverse effect on our business, operations and preclinical and clinical development timelines and plans, and could significantly constrain or disrupt the operations of third parties upon which we rely, including contract research organizations (CROs) and contract manufacturing organizations (CMOs).

In response to the COVID-19 pandemic, federal, state, local and foreign governments have put in place quarantines, executive orders, shelter-in-place orders, guidelines and other similar orders and restrictions intended to control the spread of the disease. Such orders and restrictions have resulted in business closures, work stoppages, delays, work-from-home policies, travel restrictions and cancellations of events, among other effects that could negatively impact productivity and disrupt our business and operations. Our offices, laboratories, clinical trial sites, prospective clinical trial sites, CROs, CMOs and other collaborators and partners are located in jurisdictions where such orders and restrictions have been enforced. 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 could require us to delay or prevent us from proceeding with our clinical trials. For instance, our clinical trial site initiation and subject enrollment could be delayed or suspended due to closures and prioritization of resources toward the COVID-19 pandemic. In addition, some subjects may not be able or willing to comply with clinical trial protocols, and the ability to conduct follow-up visits with treated subjects may be limited if travel or healthcare services are impeded. Similarly, our ability to recruit and retain principal investigators and other clinical trial personnel could be adversely affected. While we have not experienced significant disruptions in our supply chain to date due to the COVID-19 pandemic, it may impact our ability to procure resources, raw materials or components necessary for our research studies and preclinical and clinical development. Additionally, required inspections and reviews by regulatory authorities may be delayed due to a focus of resources on COVID-19 as well as continued travel and other restrictions. Significant delays in the timing and completion of our research studies and preclinical and clinical development would be costly and could adversely affect our ability to obtain marketing approval from regulatory authorities for the commercialization of our product candidates.

The construction of our new headquarters, including our current good manufacturing practice (cGMP) production facility, is expected to be delayed due to various government orders and restrictions relating to the COVID-19 pandemic. The potential impact of any such delay is unpredictable but may include significant additional costs and disruptions to our operations.

The spread of COVID-19 has caused a broad impact globally and may materially affect our business, financial condition and results of operations, as well as continue to increase the volatility and adversely affect the value of our common stock. While the full extent of the economic impact and duration of the COVID-19 pandemic may be difficult to assess or predict, the continuation of prolonged adverse economic conditions (including due to any resurgence or second wave of COVID-19 infections) may reduce our ability to access capital and adversely affect our liquidity. 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.

Scientific and economic analyses of the COVID-19 pandemic continue to evolve and we will continue to monitor the situation closely. The ultimate impact of the COVID-19 pandemic and other public health crises is highly unpredictable and subject to change. We are not yet certain about the full extent of the potential impact of COVID-19 on our business, operations and preclinical and clinical development. To the extent COVID-19 adversely affects our business, financial condition and results of operations, as well as global economic conditions more generally, it may also heighten many of the other risk factors described in our Annual Report on Form 10-K for the year ended December 31, 2019.

Risks Related to Third Parties

We have in the past, and in the future may, enter into licensing agreements or collaborations with third parties licensing parts of our NAV Technology Platform for the development of product candidates. If these licensing arrangements or collaborations are not successful, our business could be harmed.

We have entered into agreements involving the licensing of parts of our NAV Technology Platform and relating to the development and commercialization of certain product candidates and plan to enter into additional licensing agreements or collaborations in the future. We have limited control over the amount and timing of resources that our current and future licensees and collaborators, including our NAV Technology Licensees, dedicate to the development or commercialization of product candidates or of products utilizing licensed components of our NAV Technology Platform. Our ability to generate revenues from these arrangements will depend on our and our licensees' and collaborators' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our licensees and collaborators have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our licensee or collaborator is responsible could be harmful to the public perception and prospects of our NAV Technology Platform or product candidates.

Any current or future licensing agreements or future collaborations we enter into may pose additional risks, including the following:

- subjects in clinical trials undertaken by licensees or future collaborators, including our NAV Technology Licensees, may suffer adverse effects, including death;
- licensees or collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the licensees' or collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;
- licensees or collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the licensees or collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates developed in collaboration with us may be viewed by our licensees or collaborators as competitive with their own product candidates or products, which may cause licensees or collaborators to cease to devote resources to the commercialization of our product candidates;
- a licensee or collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;
- licensees or collaborators may breach their reporting, payment, intellectual property or other obligations to us, which could prevent us from complying with our contractual obligations to GSK and Penn;

- disagreements with licensees or collaborators, including disagreements over intellectual property and other proprietary rights, payment obligations, contract interpretation or the preferred course of development of any product candidates, may cause delays or termination of the research, development or commercialization of such product candidates, may lead to additional responsibilities for us with respect to such product candidates or may result in litigation or arbitration, any of which would be time-consuming and expensive and could potentially lessen the value of such agreements and collaborations;
- licensees or collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- disputes may arise with respect to the ownership of our other rights to intellectual property developed pursuant to our licensing agreements or collaborations;
- licensees or collaborators may infringe or otherwise violate the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- licensing agreements or collaborations may be terminated for the convenience of the licensee or collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If our licensing agreements or collaborations do not result in the successful development and commercialization of products, or if one of our licensees or collaborators terminates its agreement with us, we may not receive any future milestone or royalty payments, as applicable, under the license agreement or collaboration. If we do not receive the payments we expect under these agreements, our development of product candidates could be delayed and we may need additional resources to develop our product candidates. In addition, if one of our licensees or collaborators terminates its agreement with us, we may find it more difficult to attract new licensees or collaborators and the perception of us in the business and financial communities could be harmed. Each of our licensees and collaborators is subject to similar risks with respect to product development, regulatory approval and commercialization, and any such risk could result in its business being harmed, which could adversely affect our collaboration.

For example, we are currently in arbitration with Abeona Therapeutics Inc. (Abeona) regarding a dispute under the License Agreement dated November 4, 2018 between the Company and Abeona, as amended on November 4, 2019 (the License Agreement), Abeona was required to make a payment of $8.0 million to us no later than April 1, 2020. Abeona failed to make this payment and we therefore delivered to Abeona a written demand for payment and breach notice in April 2020. Upon expiration of the applicable cure period under the License Agreement, which expiration occurred on May 2, 2020, the License Agreement was terminated. Upon termination, all rights and licenses granted to Abeona under the License Agreement terminated and an additional $20.0 million fee that would have otherwise been due to us in November 2020 became payable within 15 days of the termination date. We have not yet received payment for any portion of the fees due from Abeona.

In May 2020, Abeona filed a claim in arbitration alleging that we breached certain responsibilities to communicate with Abeona regarding our prosecution of licensed patents under the License Agreement. We dispute Abeona's claim and have filed a counterclaim in arbitration for the $28.0 million total unpaid fees, plus interest, which accrues at a rate of 1.5% per month under the License Agreement. We have not recorded any liabilities related to this matter, as we believe our risk of loss is remote. Additionally, we intend to enforce the collection of all amounts owed from Abeona and, based on our evaluation of the merits of the claims, we expect to receive payment in full upon the completion of arbitration. However, we may ultimately receive less than the full amount we believe we are owed, and the duration of the arbitration and the timing of payment from Abeona, if any, are unpredictable. Any such adverse result or delay in payment may have a material adverse effect on our business, financial condition, results of operations or cash flows.

We may in the future decide to partner or collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of our product candidates. These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Our ability to reach a definitive licensing agreement or collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a variety of factors. If we license rights to product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate the licensed product candidates with our existing operations.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
3.1	Restated Certificate of Incorporation	8-K	3.1	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 June 30, 2020 formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (ii) Consolidated Statements of Operations and Comprehensive Loss (iii) Consolidated Statements of Stockholders' Equity (iv) Consolidated Statements of Cash Flows (v) Notes to Consolidated Financial Statements				X
104	The cover page from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended June 30, 2020 formatted in Inline XBRL (included in Exhibit 101)				

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

SIGNATURES

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

REGENXBIO Inc.

Dated: August 6, 2020

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

Dated: August 6, 2020

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

EXHIBIT 31.1

CERTIFICATION

I, Kenneth T. Mills, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting.

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

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

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

Date: August 6, 2020

/s/ Kenneth T. Mills

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

EXHIBIT 31.2

CERTIFICATION

I, Vittal Vasista, certify that:

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

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting.

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

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

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

Date: August 6, 2020

/s/ Vittal Vasista

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

EXHIBIT 32.1

CERTIFICATION

In connection with the Quarterly Report of REGENXBIO Inc. (the "Registrant") on Form 10-Q for the quarter ended June 30, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Kenneth T. Mills, President, Chief Executive Officer and Director of the Registrant, and Vittal Vasista, Chief Financial Officer of the Registrant, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to their respective knowledge:

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

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

Date: August 6, 2020

/s/ Kenneth T. Mills

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

Date: August 6, 2020

/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.