Table of Contents

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 September 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 **(Address of principal executive offices)**	**20850** **(Zip Code)**

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

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

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

Title of each class	Trading symbol(s)	Name of each exchange on which registered
Common Stock, $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 October 30, 2020, there were 37,408,448 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 SEPTEMBER 30, 2020

TABLE OF CONTENTS

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 and our ability to recover such unpaid fees;
- 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)

	September 30, 2020	December 31, 2019
Assets		
Current assets		
Cash and cash equivalents	$ 93,220	$ 69,514
Marketable securities	148,305	226,696
Accounts receivable, net	122,116	38,148
Prepaid expenses	16,112	6,475
Other current assets	7,197	4,199
Total current assets	386,950	345,032
Marketable securities	48,272	103,785
Accounts receivable, net	3,564	4,155
Property and equipment, net	38,871	28,973
Operating lease right-of-use assets	57,827	10,078
Restricted cash	1,330	1,330
Other assets	4,360	4,555
Total assets	$ 541,174	$ 497,908
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 15,244	$ 6,409
Accrued expenses and other current liabilities	44,356	24,846
Deferred revenue	449	—
Operating lease liabilities	3,564	2,421
Total current liabilities	63,613	33,676
Deferred revenue	3,895	3,333
Operating lease liabilities	57,461	8,874
Other liabilities	545	1,828
Total liabilities	125,514	47,711
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at September 30, 2020 and December 31, 2019	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at September 30, 2020 and December 31, 2019; 37,404 and 36,992 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	4	4
Additional paid-in capital	658,224	627,810
Accumulated other comprehensive income	263	205
Accumulated deficit	(242,831)	(177,822)
Total stockholders' equity	415,660	450,197
Total liabilities and stockholders' equity	$ 541,174	$ 497,908

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Revenues				
License and royalty revenue	$ 98,912	$ 14,700	$ 133,122	$ 23,465
Total revenues	98,912	14,700	133,122	23,465
Operating Expenses				
Cost of revenues	17,364	2,494	25,457	4,450
Research and development	43,968	35,692	119,114	90,378
General and administrative	15,859	12,402	46,246	37,365
Provision for credit losses and other	7,770	8	7,887	(54)
Total operating expenses	84,961	50,596	198,704	132,139
Income (loss) from operations	13,951	(35,896)	(65,582)	(108,674)
Other Income (Loss)				
Interest income from licensing	1,444	716	4,141	2,091
Investment income (loss)	(6,607)	431	(4,071)	37,950
Total other income (loss)	(5,163)	1,147	70	40,041
Income (loss) before income taxes	8,788	(34,749)	(65,512)	(68,633)
Income Tax Benefit	3	165	503	364
Net income (loss)	$ 8,791	$ (34,584)	$ (65,009)	$ (68,269)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net	(487)	(108)	58	1,043
Total other comprehensive income (loss)	(487)	(108)	58	1,043
Comprehensive income (loss)	$ 8,304	$ (34,692)	$ (64,951)	$ (67,226)
Net income (loss) per share:				
Basic	$ 0.24	$ (0.94)	$ (1.75)	$ (1.86)
Diluted	$ 0.23	$ (0.94)	$ (1.75)	$ (1.86)
Weighted-average common shares outstanding:				
Basic	37,342	36,813	37,234	36,618
Diluted	38,877	36,813	37,234	36,618

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 September 30, 2020					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at June 30, 2020	37,291	$ 4	$ 648,729	$ 750	$ (251,622)	$ 397,861
Exercise of stock options	74	—	268	—	—	268
Issuance of common stock under employee stock purchase plan	38	—	1,192	—	—	1,192
Stock-based compensation expense	—	—	8,035	—	—	8,035
Unrealized loss on available-for-sale securities, net	—	—	—	(487)	—	(487)
Net income	—	—	—	—	8,791	8,791
Balances at September 30, 2020	37,404	$ 4	$ 658,224	$ 263	$ (242,831)	$ 415,660

	Three Months Ended September 30, 2019					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at June 30, 2019	36,752	$ 4	$ 610,891	$ 471	$ (116,774)	$ 494,592
Exercise of stock options	62	—	384	—	—	384
Issuance of common stock under employee stock purchase plan	26	—	949	—	—	949
Stock-based compensation expense	—	—	7,162	—	—	7,162
Unrealized loss on available-for-sale securities, net	—	—	—	(108)	—	(108)
Net loss	—	—	—	—	(34,584)	(34,584)
Balances at September 30, 2019	36,840	$ 4	$ 619,386	$ 363	$ (151,358)	$ 468,395

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

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

	Nine Months Ended September 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	356	—	4,247	—	—	4,247
Issuance of common stock under employee stock purchase plan	55	—	1,799	—	—	1,799
Stock-based compensation expense	—	—	24,368	—	—	24,368
Unrealized gain on available-for-sale securities, net	—	—	—	58	—	58
Net loss	—	—	—	—	(65,009)	(65,009)
Balances at September 30, 2020	37,404	$ 4	$ 658,224	$ 263	$ (242,831)	$ 415,660

	Nine Months Ended September 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	684	—	5,513	—	—	5,513
Issuance of common stock under employee stock purchase plan	36	—	1,314	—	—	1,314
Stock-based compensation expense	—	—	19,979	—	—	19,979
Unrealized gain on available-for-sale securities, net	—	—	—	1,043	—	1,043
Net loss	—	—	—	—	(68,269)	(68,269)
Balances at September 30, 2019	36,840	$ 4	$ 619,386	$ 363	$ (151,358)	$ 468,395

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

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

	Nine Months Ended September 30,	
	2020	2019
Cash flows from operating activities		
Net loss	$ (65,009)	$ (68,269)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	24,368	19,979
Depreciation and amortization	6,302	5,229
Provision for credit losses	7,678	—
Net amortization of premiums (accretion of discounts) on marketable debt securities	586	(1,085)
Net realized and unrealized losses (gains) on marketable securities	8,207	(29,440)
Imputed interest income from licensing	(2,033)	(2,091)
Other non-cash adjustments	263	357
Changes in operating assets and liabilities		
Accounts receivable	(89,442)	(10,699)
Prepaid expenses	(9,637)	(1,418)
Other current assets	(2,998)	1,286
Operating lease right-of-use assets	1,986	1,712
Other assets	1,318	(2,150)
Accounts payable	9,907	5,339
Accrued expenses and other current liabilities	18,400	1,990
Deferred revenue	—	(600)
Operating lease liabilities	(2,222)	(1,832)
Other liabilities	(1,203)	(507)
Net cash used in operating activities	(93,529)	(82,199)
Cash flows from investing activities		
Purchases of marketable debt securities	(79,374)	(127,925)
Maturities of marketable debt securities	189,882	218,019
Sales of marketable debt securities	2,287	—
Sales of marketable equity securities	12,374	—
Purchases of property and equipment	(13,980)	(10,689)
Net cash provided by investing activities	111,189	79,405
Cash flows from financing activities		
Proceeds from exercise of stock options	4,247	5,513
Proceeds from issuance of common stock under employee stock purchase plan	1,799	1,314
Net cash provided by financing activities	6,046	6,827
Net increase in cash and cash equivalents and restricted cash	23,706	4,033
Cash and cash equivalents and restricted cash		
Beginning of period	70,844	76,614
End of period	$ 94,550	$ 80,647
Supplemental disclosures of non-cash investing and financing activities		
Additions to property and equipment through accounts payable and accrued expenses	$ 46	$ —
Non-cash additions to property and equipment through tenant improvement allowance	$ 2,217	$ —
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 September 30, 2020, the Company had generated an accumulated deficit of $242.8 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 September 30, 2020, the Company had cash, cash equivalents and marketable securities of $289.8 million, which management believes is sufficient to fund operations for at least the next 12 months from the date these consolidated financial statements were issued.

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, the allowance for credit losses, 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):

	September 30, 2020	September 30, 2019
Cash and cash equivalents	$ 93,220	$ 79,594
Restricted cash	1,330	1,053
Total cash and cash equivalents and restricted cash	$ 94,550	$ 80,647

Accounts Receivable

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

Accounts receivable are stated net of an allowance for credit losses, if deemed necessary based on the Company's evaluation of collectability and potential credit losses. Management assesses the collectability of its accounts receivable using the specific identification of account balances, and considers the credit quality and financial condition of its significant customers, historical information regarding credit losses and the Company's evaluation of current and expected future economic conditions. If necessary, an allowance for credit losses is recorded against accounts receivable such that the carrying value of accounts receivable reflects the net amount expected to be collected. Accounts receivable balances are written off against the allowance for credit losses when the potential for collectability is considered remote. Please refer to Note 7 for further information regarding the allowance for credit losses related to accounts receivable.

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 September 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 Income (Loss) Per Share

Basic net income (loss) per share is calculated by dividing net income (loss) applicable to common stockholders by the weighted-average common shares outstanding during the period, without consideration for common stock equivalents. Diluted net income (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 income (loss) per share calculation, common stock equivalents are excluded from the calculation of diluted net income (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 three and nine months ended September 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
September 30, 2020				
U.S. government and federal agency securities	$ 20,802	$ 77	$ —	$ 20,879
Certificates of deposit	3,416	51	—	3,467
Corporate bonds	159,732	1,007	(11)	160,728
Equity securities	195	11,308	—	11,503
	$ 184,145	$ 12,443	$ (11)	$ 196,577

	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 September 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 September 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 nine months ended September 30, 2020, the Company recognized net unrealized gains (losses) on available-for-sale debt securities of $(0.5) million and $0.1 million, respectively, and income tax expense of zero in other comprehensive income (loss) for the period. The Company recognized net realized gains of less than $0.1 million and $0.1 million on the sale or maturity of available-for-sale debt securities during the three and nine months ended September 30, 2020, respectively, which were reclassified out of accumulated other comprehensive income during the period and were included in investment income (loss) in the consolidated statements of operations and comprehensive income (loss). During the three and nine months ended September 30, 2019, the Company recognized net unrealized gains (losses) on available-for-sale debt securities of $(0.1) million and $1.7 million, respectively, and income tax benefit (expense) of less than $0.1 million and $(0.6) million, respectively, in other comprehensive income (loss) for the period. The Company recognized net realized gains of less than $0.1 million on the sale or maturity of available-for-sale debt securities during the three and nine months ended September 30, 2019, which were reclassified out of accumulated other comprehensive income during the period and were included in investment income (loss) in the consolidated statements of operations and comprehensive income (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
September 30, 2020						
Corporate bonds	$ 13,728	$ (11)	$ —	$ —	$ 13,728	$ (11)
	$ 13,728	$ (11)	$ —	$ —	$ 13,728	$ (11)

	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 September 30, 2020, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of five investment grade security positions. The Company has the intent and ability to hold such securities until recovery, and due to the credit quality of the issuers and low severity of each unrealized loss position relative to its amortized cost basis, the Company did not identify any credit losses associated with its available-for-sale debt securities. The Company did not recognize any impairment or credit losses on available-for-sale debt securities during the three and nine months ended September 30, 2020.

Marketable equity securities held by the Company as of September 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 nine months ended September 30, 2020, the Company recognized net realized and unrealized losses of $7.5 million and $8.3 million, respectively, related to its marketable equity securities of Prevail. During the three and nine months ended September 30, 2019, the Company recognized unrealized gains (losses) of $(2.2) million and $29.4 million, respectively, 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
September 30, 2020				
Cash equivalents:				
Money market mutual funds	$ —	$ 70,805	$ —	$ 70,805
Total cash equivalents	—	70,805	—	70,805
Marketable securities:				
U.S. government and federal agency securities	—	20,879	—	20,879
Certificates of deposit	—	3,467	—	3,467
Corporate bonds	—	160,728	—	160,728
Equity securities	11,503	—	—	11,503
Total marketable securities	11,503	185,074	—	196,577
Total cash equivalents and marketable securities	$ 11,503	$ 255,879	$ —	$ 267,382

	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 September 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 September 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 nine months ended September 30, 2020 and 2019.

5. Property and Equipment, Net

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

	September 30, 2020	December 31, 2019
Laboratory and manufacturing equipment	$ 23,162	$ 19,663
Computer equipment and software	3,272	2,545
Furniture and fixtures	2,898	2,188
Leasehold improvements	30,109	18,915
Total property and equipment	59,441	43,311
Accumulated depreciation and amortization	(20,570)	(14,338)
Property and equipment, net	$ 38,871	$ 28,973

6. Leases

9804 Medical Center Drive

In November 2018, the Company entered into an operating lease, as amended in April 2019 and November 2019, for approximately 177,000 square feet of office, laboratory and manufacturing facilities in a new building to be constructed at 9804 Medical Center Drive in Rockville, Maryland (the 9804 Medical Center Drive Lease). The new facility will serve as the Company's future corporate, research and manufacturing headquarters. The initial construction of the building was performed by the landlord, and the lease commenced in September 2020 upon the delivery of leased premises to the Company to make additional improvements to the building. Monthly payments under the lease begin in September 2021 and escalate annually in accordance with the lease agreement. The lease expires in September 2036, subject to extension and termination options held by the Company. The Company has the option to extend the term of the lease for up to 10 additional years and the option to terminate the lease, with payment of an early termination fee, after 12 years from the delivery of the leased premises to the Company. The Company's extension and termination options under the 9804 Medical Center Drive Lease have been excluded from the measurement of the right-of-use assets and lease liabilities for the lease as they were not reasonably certain of exercise. As required by the lease agreement, the Company has provided the landlord with an irrevocable letter of credit of $1.1 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease.

Pursuant to the 9804 Medical Center Drive Lease, the Company received a $19.5 million tenant improvement allowance from the landlord to perform improvements to the leased premises. The tenant improvement allowance has been recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of lease expense over the term of the lease. As of September 30, 2020, the Company had unreimbursed amounts remaining under the tenant improvement allowance of $17.2 million, which were deemed in-substance lease payments and recorded as a reduction of the lease liability. As of September 30, 2020, the Company had recorded property and equipment of $17.3 million related to the buildout of the facility at 9804 Medical Center Drive, which have not yet been placed in service.

The Company recorded the right-of-use assets and lease liabilities related to the 9804 Medical Center Drive Lease upon its commencement in September 2020. As of September 30, 2020, the Company had recorded right-of-use assets of $50.6 million and lease liabilities of $52.5 million related to the 9804 Medical Center Drive Lease.

9712 Medical Center Drive

In March 2015, the Company entered into an operating lease for office space at 9712 Medical Center Drive in Rockville, Maryland (the 9712 Medical Center Drive Lease). The lease term commenced in April 2015. Monthly payments under the lease began in October 2015 and escalate annually in accordance with the lease agreement.

In September 2015, November 2015, July 2017 and April 2018, the Company amended the 9712 Medical Center Drive Lease to include additional office and laboratory space at 9714 Medical Center Drive, and ultimately extend the term of the lease to September 2021. The Company had options to extend the term of the 9712 Medical Center Drive Lease for up to six additional years. Additionally, upon the commencement of the 9804 Medical Center Drive Lease in September 2020, the Company had the option to terminate the 9712 Medical Center Drive Lease with six months' notice. The Company's extension and termination options under the 9712 Medical Center Drive Lease were excluded from the measurement of the right-of-use assets and lease liabilities for the lease as they were not reasonably certain of exercise. The Company received a $0.4 million tenant improvement allowance from the landlord which has been recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of lease expense over the term of the lease.

In October 2020, the Company amended the 9712 Medical Center Drive Lease to extend the term of the lease to February 2027. Pursuant to the amendment, the Company has an option to extend the term of the lease for three additional years, as well as an option to extend the lease term to be coterminous with the 9804 Medical Center Drive Lease, which expires in September 2036. Total additional lease payments under the 9712 Medical Center Drive Lease as a result of the October 2020 amendment were $8.8 million, excluding any lease payments contingent upon the Company's option to extend the term of the lease.

9600 Blackwell Road

In January 2016, the Company entered into an operating lease for its corporate headquarters at 9600 Blackwell Road in Rockville, Maryland (the Blackwell Road Lease). The lease commenced in February 2016 and expires in September 2023. In November 2017, the Blackwell Road Lease was amended to include additional office space for the remainder of the lease term. Monthly payments under the lease began in September 2016 and escalate annually in accordance with the lease agreement. The Company received a $0.8 million tenant improvement allowance from the landlord which has been recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of lease expense over the term of the lease.

The Company had an option to extend the term of the Blackwell Road Lease for up to five additional years and the option to terminate the lease, with payment of an early termination fee, after 67 months from the lease commencement date. During the three months ended September 30, 2020, the Company reassessed the term of the Blackwell Road Lease and determined that as of September 30, 2020, it was reasonably certain that the Company will exercise its termination option under the lease. Accordingly, the measurement of the right-of-use assets and lease liabilities for the Blackwell Road Lease were reduced by $0.7 million during the three months ended September 30, 2020, to reflect the payment of the early termination fee and the revised lease term through September 2021.

In November 2020, the Company exercised its termination option under the Blackwell Road Lease. As a result of the termination, the lease will expire in September 2021 and the Company is obligated to pay an early termination fee of $0.4 million.

400 Madison Avenue

In May 2016, the Company entered into an operating lease for office space at 400 Madison Avenue in New York, New York (the 400 Madison Lease). The lease commenced in July 2016 and monthly payments under the lease began in October 2016 and escalate annually in accordance with the lease agreement. In May 2019, the 400 Madison Lease was amended to include additional office space and extend the term of the lease from October 2020 to April 2027. The Company received a $0.7 million tenant improvement allowance from the landlord which has been recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of lease expense over the term of the lease. As required by the lease agreement, the Company has provided the landlord with an irrevocable letter of credit of $0.2 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease.

Other Leases

The Company leases additional office and laboratory facilities, laboratory equipment and other equipment under operating leases with various expiration dates through 2028, including leases which have been executed but have not yet commenced.

Operating Lease Information

All of the Company's leases are classified as operating leases. The following table summarizes the Company's lease costs and supplemental cash flow information related to its operating leases (in thousands):

	Three Months Ended September 30, 2020	Nine Months Ended September 30, 2020
Operating lease cost	$ 997	$ 2,793
Variable lease cost	174	575
Total lease cost	$ 1,171	$ 3,368
Cash paid for amounts included in operating lease liabilities	$ 988	$ 2,482
Right-of-use assets acquired through operating lease liabilities	$ 49,734	$ 49,734

Right-of-use assets acquired through operating lease liabilities for the three and nine months ended September 30, 2020 include a reduction of $0.7 million related to the Company's change in estimate regarding the exercise of its termination rights under the Blackwell Road Lease. Short-term lease expense for the three and nine months ended September 30, 2020 was not material and is included in operating lease cost in the table above. Variable lease cost under the Company's operating leases includes items such as common area maintenance, utilities, taxes and other charges.

The weighted-average remaining lease term and weighted-average discount rate of the Company's operating leases were as follows:

	As of September 30, 2020
Weighted-average remaining lease term (years)	13.8
Weighted-average discount rate	5.4%

The following table presents a reconciliation of the undiscounted future minimum lease payments remaining the Company's operating leases to the amounts reported as operating lease liabilities on the consolidated balance sheet as of September 30, 2020 (in thousands):

	As of September 30, 2020
Undiscounted future minimum lease payments:	
2020 (remainder of year)	$ 1,117
2021	3,964
2022	5,055
2023	6,980
2024	8,000
Thereafter	99,184
Total undiscounted future minimum lease payments	$ 124,300
Amount representing imputed interest	(45,843)
Tenant improvement allowance not yet received	(17,432)
Total operating lease liabilities	61,025
Current portion of operating lease liabilities	(3,564)
Operating lease liabilities, non-current	$ 57,461

The table above excludes future minimum lease payments for leases which were executed but had not yet commenced as of September 30, 2020, the total of which were not material.

7. License and Royalty Revenue

As of September 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 September 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 $213.4 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) $57.0 million upon the achievement of specified sales targets for licensed products. To the extent the milestone payments are realized by the Company, the Company will be obligated to pay sublicense fees to licensors based on a specified percentage of the fees earned by the Company. The achievement of milestones by licensees is highly dependent on the successful development and commercialization of licensed products and it is at least reasonably possible that some or all of the milestone fees will not be realized by the Company.

Accounts Receivable, Contract Assets and Deferred Revenue

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Accounts receivable, current and non-current:				
Balance, beginning of period	$ 46,494	$ 33,634	$ 42,303	$ 31,599
Additions	100,243	15,526	137,220	26,575
Deductions	(21,057)	(5,640)	(53,843)	(14,654)
Balance, end of period	$ 125,680	$ 43,520	$ 125,680	$ 43,520
Contract assets:				
Balance, beginning of period	$ 350	$ —	$ —	$ 750
Additions	—	—	350	1,000
Deductions	—	—	—	(1,750)
Balance, end of period	$ 350	$ —	$ 350	$ —
Deferred revenue, current and non-current:				
Balance, beginning of period	$ 4,457	$ 3,333	$ 3,333	$ 3,933
Additions	—	—	1,124	—
Deductions	(113)	—	(113)	(600)
Balance, end of period	$ 4,344	$ 3,333	$ 4,344	$ 3,333
Revenue recognized during the period from:				
Amounts included in deferred revenue at beginning of period	$ 113	$ —	$ —	$ 600
Performance obligations satisfied in previous periods	$ 98,799	$ 10,072	$ 125,555	$ 15,037

Additions to accounts receivable during the periods presented consisted primarily of royalties on net sales of Zolgensma, billed and unbilled receivables recorded for the achievement of milestones by licensees during the period, receivables recorded related to new licenses granted by the Company, and interest income recognized related to significant financing components. Deductions to accounts receivable during the periods presented primarily consisted of amounts collected from licensees and increases in the allowance for credit losses. Additions to contract assets during the periods presented consisted of development milestones deemed probable of achievement by licensees during the periods. Deductions to contract assets during the periods presented consisted of the achievement of such milestones and billing of the associated milestone payments by the Company.

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

	September 30, 2020	December 31, 2019
Current accounts receivable:		
Billed to customers	$ 30,086	$ 376
Unbilled	99,708	37,772
Allowance for credit losses	(7,678)	—
Current accounts receivable, net	122,116	38,148
Non-current accounts receivable:		
Unbilled	3,564	4,155
Allowance for credit losses	—	—
Non-current accounts receivable, net	3,564	4,155
Total accounts receivable, net	$ 125,680	$ 42,303

The following table presents the changes in the allowance for credit losses related to accounts receivable and contract assets for the nine months ended September 30, 2020 (in thousands):

	Accounts Receivable	Contract Assets
Balance at December 31, 2019	$ —	$ —
Provision for credit losses	7,678	—
Write-offs	—	—
Balance at September 30, 2020	$ 7,678	$ —

The Company's allowance for credit losses as of September 30, 2020 was related solely to accounts receivable from Abeona Therapeutics Inc. (Abeona). Please refer to the section below, Abeona Therapeutics Inc., for further information regarding amounts due from Abeona and the associated allowance for credit losses. The Company's provision for credit losses for the three and nine months ended September 30, 2020 was $7.7 million and was related solely to changes in estimates regarding the allowance for credit losses associated with the accounts receivable from Abeona. No provision for credit losses was recorded for the three or nine months ended September 30, 2019.

As of September 30, 2020, the Company had recorded deferred revenue of $4.3 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, which will be satisfied upon the exercise or expiration of the options and (ii) research and development services to be performed by the Company related to licensed products, which will be satisfied as the research and development services are performed.

Revenue recognized from performance obligations satisfied in previous periods was primarily attributable to royalty and sublicense revenues as well as changes in transaction prices of the Company's license agreements during the periods. Changes in transaction prices were primarily attributable to development milestones achieved or deemed probable of achievement during the periods, which were previously not considered probable of achievement.

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, a licensed product under the March 2014 License, in the second quarter of 2019, upon which the Company began recognizing royalty revenue on net sales of the licensed product.

Pursuant to the March 2014 License, AveXis was obligated to pay a sales-based milestone fee of $80.0 million to the Company upon the achievement of $1.0 billion in cumulative net sales of licensed products. AveXis achieved cumulative net sales of Zolgensma of $1.0 billion in third quarter of 2020, upon which the Company recognized revenue of $80.0 million related to the sales-based milestone fee. The $80.0 milestone fee was recorded as accounts receivable as of September 30, 2020, and the Company received payment of the $80.0 million milestone fee from AveXis in October 2020.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
License revenue	$ —	$ —	$ 3,500	$ 3,500
Royalties on net sales of Zolgensma	18,799	9,182	40,723	10,106
Achievement of sales-based milestone for Zolgensma	80,000	—	80,000	—
Total license and royalty revenue	$ 98,799	$ 9,182	$ 124,223	$ 13,606
Interest income from licensing	$ 6	$ 7	$ 20	$ 22

As of September 30, 2020, the Company had recorded total accounts receivable of $98.9 million from AveXis under the March 2014 License, of which $98.8 million were included in current assets and $0.1 million were included in non-current assets. As of December 31, 2019, the Company had recorded total accounts receivable of $11.0 million 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.

In November 2018, the Company entered into a license agreement with Abeona, as amended in November 2019 (the November 2018 License), for the development and commercialization of various diseases using the NAV Technology Platform. Pursuant to the November 2018 License, Abeona was required to pay a license fee of $8.0 million to the Company no later than April 1, 2020. Abeona failed to make this payment, and in April 2020, the Company delivered to Abeona a notice of its breach of the license agreement and written demand for payment. Upon expiration of the applicable cure period in May 2020, the license agreement was terminated. As a result of the termination, Abeona was required to pay a $20.0 million license fee to the Company within 15 days of the termination date, which otherwise would have been due to the Company in November 2020. As of October 30, 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. During the three and nine months ended September 30, 2020, the Company recognized interest income from licensing of $1.3 million and $2.1 million, respectively, related to the unpaid license fees from Abeona under the November 2018 License. Total accounts receivable from Abeona recorded as of September 30, 2020 was $30.1 million, consisting of the unpaid license fees and associated accrued interest.

In May 2020, subsequent to the termination of the November 2018 License, Abeona filed a claim in arbitration alleging that the Company had breached certain responsibilities to communicate with Abeona regarding the Company's prosecution of licensed patents under the November 2018 License. The Company disputes Abeona's claim and has filed a counterclaim in arbitration demanding payment of the $28.0 million of unpaid fees from Abeona, plus accrued interest. Based on its evaluation of the merits of Abeona's claims, the Company had not recorded any liabilities related these claims as of September 30, 2020, and the Company currently expects that its demand for payment in full will be upheld in arbitration. The Company intends to enforce the full collection of all amounts due from Abeona upon completion of arbitration, which is currently scheduled to occur in March 2021. However, the duration and outcome of arbitration and timing of payment from Abeona are unpredictable and uncertain at this time.

While the Company currently expects its demand for payment in full will be upheld in arbitration, the Company assessed the collectability of the $30.1 million due from Abeona as of September 30, 2020 as it relates to credit risk. In performing this assessment, the Company evaluated Abeona's credit profile and financial condition, as well its expectations regarding Abeona's future cash flows and ability to satisfy this obligation upon the completion of arbitration in 2021. Additionally, the Company considered Abeona's continued failure to remit payment to the Company, as well as events which occurred during the three months ended September 30, 2020 impacting Abeona's business and credit profile, specifically the departure of key members of Abeona's management and board of directors and subsequent decline in market capitalization. As a result of this analysis, the Company recorded an allowance for credit losses of $7.7 million as of September 30, 2020 related to the accounts receivable due from Abeona. However, management intends to enforce the full collection of all amounts due from Abeona upon the completion of arbitration. In accordance with the Company's interest accrual policy, the Company will cease the recognition of interest income accrued under the license agreement subsequent to the recognition of the allowance for credit losses unless and until such amounts are deemed to be collectable.

8. 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 September 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 2,321,306 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 September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Stock options	$ 7,880	$ 6,965	$ 23,745	$ 19,261
Restricted stock units	—	69	—	206
Employee stock purchase plan	155	128	623	512
	$ 8,035	$ 7,162	$ 24,368	$ 19,979

As of September 30, 2020, the Company had $68.6 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 2.5 years.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	$ 4,110	$ 3,657	$ 12,442	$ 9,530
General and administrative	3,925	3,505	11,926	10,449
	$ 8,035	$ 7,162	$ 24,368	$ 19,979

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	1,542	$ 38.25		
Exercised	(356)	$ 11.92		
Cancelled or forfeited	(321)	$ 45.11		
Outstanding at September 30, 2020	6,409	$ 31.18	7.4	$ 37,396
Exercisable at September 30, 2020	3,581	$ 23.34	6.3	$ 36,885
Vested and expected to vest at September 30, 2020	6,409	$ 31.18	7.4	$ 37,396

(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 nine months ended September 30, 2020 was $24.08. During the nine months ended September 30, 2020, the total number of stock options exercised was 356,302, resulting in total proceeds of $4.2 million. The total intrinsic value of options exercised during the nine months ended September 30, 2020 was $9.4 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 September 30, 2020, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 623,924, of which 448,011 remained available for future issuance. During the nine months ended September 30, 2020, 55,499 shares of common stock were issued under the 2015 ESPP.

9. Income Taxes

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, including three-year cumulative loss positions as of September 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 September 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 nine months ended September 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 nine months ended September 30, 2020, or to the Company's net deferred tax assets as of September 30, 2020.

10. 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. Effective August 2020, the agreement was further amended to extend the term of the agreement by two years through December 2022. The agreement may be terminated by either party with six months' advance written notice. Expenses incurred under the agreements with FOXKISER for the three and nine months ended September 30, 2020 were $1.2 million and $3.6 million, respectively. Expenses incurred under the agreements with FOXKISER for the three and nine months ended September 30, 2019 were $1.2 million and $2.9 million, respectively. Expenses incurred under the agreements with FOXKISER were recorded as research and development expenses in the consolidated statements of operations and comprehensive income (loss).

11. Net Income (Loss) Per Share

The computations of basic and diluted net income (loss) per share were as follows (in thousands, except per share data):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Basic net income (loss) per share:				
Net income (loss)	$ 8,791	$ (34,584)	$ (65,009)	$ (68,269)
Shares used in computation:				
Weighted-average common shares outstanding	37,342	36,813	37,234	36,618
Basic net income (loss) per share	$ 0.24	$ (0.94)	$ (1.75)	$ (1.86)
Diluted net income (loss) per share:				
Net income (loss)	$ 8,791	$ (34,584)	$ (65,009)	$ (68,269)
Shares used in computation:				
Weighted-average common shares outstanding	37,342	36,813	37,234	36,618
Stock options	1,529	—	—	—
Employee stock purchase plan	6	—	—	—
Weighted-average diluted common shares	38,877	36,813	37,234	36,618
Diluted net income (loss) per share	$ 0.23	$ (0.94)	$ (1.75)	$ (1.86)

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Stock options issued and outstanding	4,072	5,530	6,409	5,530
Unvested restricted stock units outstanding	—	40	—	40
Employee stock purchase plan	—	16	21	16
	4,072	5,586	6,430	5,586

12. Supplemental Disclosures

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

	September 30, 2020	December 31, 2019
Accrued sublicense fees and royalties	$ 19,285	$ 4,542
Accrued personnel costs	10,380	10,903
Accrued external research and development expenses	9,163	5,791
Accrued external general and administrative expenses	2,873	2,053
Accrued purchases of property and equipment	2,438	1,328
Other accrued expenses and current liabilities	217	229
	$ 44,356	$ 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 for the subretinal delivery 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 first quarter of 2021.

  We have begun dosing patients in a Phase II trial for the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet AMD (AAVIATE). We expect to complete enrollment of the first cohort by the end of 2020, and report initial safety data in early 2021. Additionally, the Phase II trial of the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of DR (ALTITUDE) is active and we expect to begin enrolling patients by the end of 2020. We expect to report interim data from this trial in 2021.
- **AAV-Mediated Antibody Expression for the Treatment of Hereditary Angioedema (HAE):** We are developing a novel, one-time treatment utilizing a NAV Vector to deliver a gene encoding for a therapeutic antibody that targets and binds to plasma kallikrein, a key protein left unregulated in patients with HAE. HAE is a chronic and severe disease characterized by recurring severe swelling (angioedema), most commonly in the face, airway, intestines and limbs. We expect to provide a program update in 2021.
- **AAV-Mediated Antibody Expression for the Treatment of Neurodegenerative Diseases:** We 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 2021.

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.

  On September 30, 2020, we announced the expansion of the RGX-121 program for MPS II. Eight patients have now been dosed across two dose cohorts in the ongoing Phase I/II trial of RGX-121 in severe MPS II patients under the age of 5 years old. The first two patients in the expanded Cohort 2 were dosed in October 2020 via intracisternal delivery of RGX-121 at a dose of $6.5x10^{10}$ genome copies per gram (GC/g) of brain mass. We anticipate further updates from this trial by the end of 2020.

  In addition, we plan to begin a second Phase I/II multicenter, open-label trial of RGX-121 for the treatment of pediatric patients with severe MPS II over the age of 5 years old. Up to six patients may be enrolled, and RGX-121 will be administered at a dose level of 6.5x1010 GC/g of brain mass. We also announced a new prospective observational study designed to provide detailed characterization of neurocognitive development and key biomarkers in patients with severe MPS II.

- **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 in the first quarter of 2021, and we 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 by the end of 2020 and initiate enrollment in the first half of 2021.

- **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 2021.

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

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively license the NAV Technology Platform to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of September 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 from our original estimates due to various government orders and restrictions relating to the COVID-19 pandemic. In addition, if the business and operations of our licensees are adversely affected by the COVID-19 pandemic, our revenues could in turn be adversely affected. We are proactively taking measures to mitigate or reduce any adverse impact of the COVID-19 pandemic on the progress of our clinical trials and other business initiatives.

Our results of operations for the three and nine months ended September 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. 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 agreement 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;
- Phase II clinical trials to evaluate the safety and efficacy of the suprachoroidal delivery of RGX-314 using the SCS Microinjector for the treatment of wet AMD (AAVIATE) and DR (ALTITUDE);
- 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 Phase I/II clinical trial for RGX-181 for the treatment of CLN2 disease;
- preclinical research and development and a planned Phase I/II 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 for the treatment of homozygous familial hypercholesterolemia (HoFH) 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 nine months ended September 30, 2020 and 2019 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Direct Expenses				
RGX-314	$ 6,472	$ 9,170	$ 16,189	$ 15,156
RGX-121	2,897	775	7,577	2,848
RGX-111	683	645	1,584	2,253
RGX-181	369	1,448	1,527	6,751
RGX-381	1,398	—	2,431	—
RGX-501	339	1,325	2,704	3,390
Other product candidates	4,237	—	9,204	—
Total direct expenses	16,395	13,363	41,216	30,398
Unallocated Expenses				
Platform and new technologies	8,731	5,631	20,910	14,486
Personnel-related	15,731	13,345	47,752	36,159
Facilities and depreciation expense	2,829	2,422	8,151	6,918
Other unallocated	282	931	1,085	2,417
Total unallocated expenses	27,573	22,329	77,898	59,980
Total research and development	$ 43,968	$ 35,692	$ 119,114	$ 90,378

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.

We have discontinued internal clinical development of RGX-501 for the treatment of HoFH and plan to evaluate strategic alternatives to support the continued advancement of this program. Planned future research and development costs related to RGX-501 primarily relate to the completion of a long-term follow-up study for patients dosed to date.

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 (Loss)

Interest Income from Licensing

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

Investment Income (Loss)

Investment income 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 September 30,			Nine Months Ended September 30,		
	2020	2019	Change	2020	2019	Change
	(in thousands)					
Revenues						
License and royalty revenue	$ 98,912	$ 14,700	$ 84,212	$ 133,122	$ 23,465	$ 109,657
Total revenues	98,912	14,700	84,212	133,122	23,465	109,657
Operating Expenses						
Cost of revenues	17,364	2,494	14,870	25,457	4,450	21,007
Research and development	43,968	35,692	8,276	119,114	90,378	28,736
General and administrative	15,859	12,402	3,457	46,246	37,365	8,881
Provision for credit losses and other	7,770	8	7,762	7,887	(54)	7,941
Total operating expenses	84,961	50,596	34,365	198,704	132,139	66,565
Income (loss) from operations	13,951	(35,896)	49,847	(65,582)	(108,674)	43,092
Other Income (Loss)						
Interest income from licensing	1,444	716	728	4,141	2,091	2,050
Investment income (loss)	(6,607)	431	(7,038)	(4,071)	37,950	(42,021)
Total other income (loss)	(5,163)	1,147	(6,310)	70	40,041	(39,971)
Income (loss) before income taxes	8,788	(34,749)	43,537	(65,512)	(68,633)	3,121
Income Tax Benefit	3	165	(162)	503	364	139
Net income (loss)	$ 8,791	$ (34,584)	$ 43,375	$ (65,009)	$ (68,269)	$ 3,260

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

License and Royalty Revenue. License and royalty revenue increased by $84.2 million, from $14.7 million for the three months ended September 30, 2019 to $98.9 million for the three months ended September 30, 2020. The increase was primarily attributable to the following:

- an increase of $9.6 million in Zolgensma royalty revenue, from $9.2 million for the third quarter of 2019 to $18.8 million for the third quarter of 2020, as sales of Zolgensma for the third quarter of 2020 increased by 82% as compared to the third quarter of 2019; and
- an $80.0 million milestone fee recognized as revenue during the three months ended September 30, 2020 as a result of the achievement of $1.0 billion in cumulative net sales of Zolgensma in the third quarter of 2020. Upon the achievement of this milestone, there are no further development or sales-based milestones remaining under the March 2014 license agreement with AveXis.

The increase in license and royalty revenue for the three months ended September 30, 2020 was partially offset by non-recurring revenue recognized during the three months ended September 30, 2019 resulting from new licenses we granted to licensees during the period.

Research and Development Expense. Research and development expenses increased by $8.3 million, from $35.7 million for the three months ended September 30, 2019 to $44.0 million for the three months ended September 30, 2020. The increase was primarily attributable to the following:

- an increase of $3.8 million for external costs associated with preclinical studies and other early-stage research and development;
- an increase of $2.3 million for personnel-related costs as a result of increased headcount of research and development personnel, including a $0.5 million increase in stock-based compensation expense;
- an increase of $1.7 million for external costs associated with clinical trial and regulatory activities; and
- an increase of $0.9 million for external costs associated with manufacturing-related services to support the ongoing development of our product candidates and process development activities.

General and Administrative Expense. General and administrative expenses increased by $3.5 million, from $12.4 million for the three months ended September 30, 2019 to $15.9 million for the three months ended September 30, 2020. The increase was primarily attributable to the following:

- an increase of $3.0 million for professional services, primarily related to commercial consulting and legal services; and
- an increase of $0.5 million for personnel-related costs as a result of increased headcount of general and administrative personnel, including a $0.4 million increase in stock-based compensation expense.

Provision for Credit Losses and Other. Provision for credit losses and other increased by $7.8 million during the three months ended September 30, 2020 as compared to the three months ended September 30, 2019. The increase was primarily attributable to a provision for credit losses of $7.7 million recognized during the three months ended September 30, 2020 related to our accounts receivable from Abeona Therapeutics Inc. (Abeona). As of September 30, 2020, we had recorded total accounts receivable from Abeona of $30.1 million and a related allowance for credit losses of $7.7 million. For further information regarding the provision for credit losses recognized during the three months ended September 30, 2020, refer to Note 7, “License and Royalty Revenue—Abeona Therapeutics Inc.” to the accompanying unaudited consolidated financial statements.

Investment Income (Loss). Investment loss was $6.6 million for the three months ended September 30, 2020 as compared to investment income of $0.4 million for the three months ended September 30, 2019, a change of $7.0 million. The change was primarily attributable to an increase in net realized and unrealized losses of $5.3 million related to our marketable equity securities of Prevail Therapeutics Inc. (Prevail), as well as a decrease of $1.8 million in investment income on marketable debt securities. As of September 30, 2020, our marketable equity securities of Prevail had a fair value of $11.5 million, and significant fluctuations in the fair value of the securities may continue to occur from period to period.

Comparison of the Nine Months Ended September 30, 2020 and 2019

License and Royalty Revenue. License and royalty revenue increased by $109.7 million, from $23.5 million for the nine months ended September 30, 2019 to $133.1 million for the nine months ended September 30, 2020. The increase was primarily attributable to the following:

- an increase of $30.6 million in Zolgensma royalty revenue, from $10.1 million for the nine months ended September 30, 2019 to $40.7 million for the nine months ended September 30, 2020, as commercial sales of Zolgensma did not commence until the second quarter of 2019; and
- an $80.0 million milestone fee recognized as revenue during the nine months ended September 30, 2020 as a result of the achievement of $1.0 billion in cumulative net sales of Zolgensma in the third quarter of 2020. Upon the achievement of this milestone, there are no further development or sales-based milestones remaining under the March 2014 license agreement with AveXis.

Research and Development Expense. Research and development expenses increased by $28.7 million, from $90.4 million for the nine months ended September 30, 2019 to $119.1 million for the nine months ended September 30, 2020. The increase was primarily attributable to the following:

- an increase of $11.5 million for personnel-related costs as a result of increased headcount of research and development personnel, including a $2.9 million increase in stock-based compensation expense;
- an increase of $5.4 million for external costs associated with clinical trial and regulatory activities;
- an increase of $5.3 million for external costs associated with preclinical studies and other early-stage research and development;
- an increase of $4.5 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 $3.4 million for laboratory costs and facilities used by research and development personnel, including a $0.7 million increase in depreciation expense allocated to research and development functions.

General and Administrative Expense. General and administrative expenses increased by $8.9 million, from $37.4 million for the nine months ended September 30, 2019 to $46.2 million for the nine months ended September 30, 2020. The increase was primarily attributable to the following:

- an increase of $5.9 million for professional services, primarily related to commercial consulting and legal services; and
- an increase of $2.5 million for personnel-related costs as a result of increased headcount of general and administrative personnel, including a $1.5 million increase in stock-based compensation expense.

Provision for Credit Losses and Other. Provision for credit losses and other increased by $7.9 million during the nine months ended September 30, 2020 as compared to the nine months ended September 30, 2019. The increase was primarily attributable to a provision for credit losses of $7.7 million recognized during the nine months ended September 30, 2020 related to our accounts receivable from Abeona. As of September 30, 2020, we had recorded total accounts receivable from Abeona of $30.1 million and a related allowance for credit losses of $7.7 million. For further information regarding the provision for credit losses recognized during the nine months ended September 30, 2020, refer to Note 7, “License and Royalty Revenue—Abeona Therapeutics Inc.” to the accompanying unaudited consolidated financial statements.

Investment Income (Loss). Investment loss was $4.1 million for the nine months ended September 30, 2020 as compared to investment income of $38.0 million for the nine months ended September 30, 2019, a change of $42.0 million. The change was primarily attributable to an unrealized gain of $29.4 million recognized during the nine months ended September 30, 2019 related to our marketable equity securities of Prevail, as well as net realized and unrealized losses of $8.3 million recognized during the nine months ended September 30, 2020 related to these securities. 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. As of September 30, 2020, our marketable equity securities of Prevail had a fair value of $11.5 million, and significant fluctuations in the fair value of the securities may continue to occur from period to period. The change in investment income (loss) also includes a decrease of $4.4 million in investment income on marketable debt securities.

Liquidity and Capital Resources

As of September 30, 2020, we had cash, cash equivalents and marketable securities of $289.8 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 September 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.

AveXis achieved cumulative net sales of Zolgensma of $1.0 billion in the third quarter of 2020, which triggered the payment of an $80.0 million milestone fee in accordance with our license agreement. We recorded the $80.0 million milestone fee as accounts receivable as of September 30, 2020, and received the payment in full from AveXis in October 2020. Upon the payment of this milestone fee, there are no further development or sales-based milestone payments remaining under the March 2014 license agreement with AveXis.

We intend to devote the majority of our current capital to clinical development, seeking regulatory approval of our product candidates and capital expenditures to build out additional office, laboratory and manufacturing capacity, including the buildout of our future corporate, manufacturing and research headquarters at 9804 Medical Center Drive in Rockville, Maryland. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the total amount of operating expenditures and capital outlays necessary to complete the development of our product candidates. Additionally, our estimates are based on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Furthermore, given the continuing uncertainty and volatile market and economic conditions caused by the COVID-19 pandemic, as well as potential for further effects due to a resurgence in COVID-19 infections, we will continue to monitor the nature and extent of the impact of the COVID-19 pandemic on our liquidity and capital resources.

Cash Flows

	Nine Months Ended September 30,	
	2020	2019
	(in thousands)	
Net cash used in operating activities	$ (93,529)	$ (82,199)
Net cash provided by investing activities	111,189	79,405
Net cash provided by financing activities	6,046	6,827
Net increase in cash and cash equivalents and restricted cash	$ 23,706	$ 4,033

Cash Flows from Operating Activities

Our net cash used in operating activities for the nine months ended September 30, 2020 increased by $11.3 million from the nine months ended September 30, 2019. The increase was primarily attributable to an increase in operating expenses of $66.6 million during the nine months ended September 30, 2020, offset primarily by an increase in Zolgensma 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 nine months ended September 30, 2020, our net cash used in operating activities of $93.5 million consisted of a net loss of $65.0 million and changes in working capital of $73.9 million, offset by $45.4 million in adjustments for non-cash items. The changes in working capital include an increase in accounts receivable of $89.4 million which was largely driven by an increase in unbilled receivables for Zolgensma royalties and an $80.0 million sales-based milestone fee earned during the third quarter which was recorded in accounts receivable at the end of 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 $24.4 million, depreciation and amortization expense of $6.3 million, an unrealized loss on our marketable equity securities of Prevail, net of realized gains, of $8.3 million, and a provision for credit losses on accounts receivable of $7.7 million.

For the nine months ended September 30, 2019, our net cash used in operating activities of $82.2 million consisted of a net loss of $68.3 million, $7.1 million in adjustments for non-cash items and changes in working capital of $6.9 million. Adjustments for non-cash items primarily consisted of an unrealized gain on our marketable equity securities of Prevail of $29.4 million, imputed interest earned from our license agreements of $2.1 million and net accretion of discounts on marketable debt securities of $1.1 million, and were partially offset by stock-based compensation expenses of $20.0 million and depreciation and amortization expense of $5.2 million. The changes in working capital include an increase in accounts receivable of $10.7 million which was largely driven by an increase in unbilled receivables for Zolgensma royalties. 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 nine months ended September 30, 2020, net cash provided by investing activities consisted of $204.5 million in sales and maturities of marketable securities, offset by $79.4 million to purchase marketable securities and $14.0 million to purchase property and equipment. We expect capital expenditures to increase for the remainder 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 nine months ended September 30, 2019, net cash provided by investing activities consisted of $218.0 million in sales and maturities of marketable securities, offset by $127.9 million to purchase marketable securities and $10.7 million to purchase property and equipment.

Cash Flows from Financing Activities

For the nine months ended September 30, 2020, net cash provided by financing activities consisted of $6.0 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

For the nine months ended September 30, 2019, net cash provided by financing activities consisted of $6.8 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

Future Funding Requirements

We have incurred cumulative losses since our inception and had an accumulated deficit of $242.8 million as of September 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, including the build out of our facility at 9804 Medical Center Drive as discussed above. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

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

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;
- the results of our preclinical studies for our product candidates and any subsequent clinical trials;
- 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 September 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 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 nine months ended September 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 September 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 September 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 September 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 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 currently expect our demand for payment in full to be upheld in arbitration. However, we have evaluated Abeona's credit profile and financial condition and have recorded an allowance for credit losses related to the accounts receivable due from Abeona. 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 7, "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 possible resurgences or multiple waves 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, and further resurgences or waves of infections may lead to similar orders and restrictions in the future. 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 ongoing construction of our new headquarters, including our current good manufacturing practice (cGMP) production facility, is expected to be delayed from our original estimates 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 further resurgences or waves 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), pursuant to which 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 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 currently expect our demand for payment in full to be upheld in arbitration. However, we have evaluated Abeona's credit profile and financial condition and have recorded an allowance for credit losses related to the accounts receivable due from Abeona. 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.

Amendment to 9712 Medical Center Drive Lease

On October 30, 2020, the Company and ARE-Maryland No. 45, LLC (the Medical Center Drive Landlord) entered into a Fifth Amendment (the Fifth Amendment) to the Lease dated as of March 6, 2015, by and between BMR-Medical Center Drive LLC, as the original landlord, and the Company, as the tenant (as previously amended by the First Amendment dated as of September 30, 2015, the Second Amendment dated as of November 23, 2015, the Third Amendment dated as of July 21, 2017 and the Fourth Amendment dated as of April 20, 2018, the Original Medical Center Drive Lease and, as amended by the Fifth Amendment, the Amended Medical Center Drive Lease).

The term of the Original Medical Center Drive Lease had been set to expire on September 30, 2021. Pursuant to the Amended Medical Center Drive Lease, the term has been extended for an additional period to commence on October 1, 2021 and expire on February 28, 2027 (the Extension Term). The Company has an option to further extend the Amended Medical Center Drive Lease until February 28, 2030 and an additional option to further extend the Amended Medical Center Drive Lease to be coterminous with the Lease dated as of November 1, 2018 between the Medical Center Drive Landlord's affiliate, ARE-Maryland No. 24, LLC, as the landlord, and the Company, as the tenant, for premises at 9804 Medical Center Drive, Rockville, Maryland.

Pursuant to the Amended Medical Center Drive Lease, the Company will lease approximately 10,833 square feet of office space at 9712 Medical Center Drive, Rockville, Maryland (the 9712 Medical Center Drive Premises) and approximately 39,421 square feet of office and laboratory space at 9714 Medical Center Drive, Rockville, Maryland (the 9714 Medical Center Drive Premises) from the Medical Center Drive Landlord.

Subject to the abatement described below, for the first year of the Extension Term, the base rent for the 9712 Medical Center Drive Premises will be $27,732.48 per month ($2.56 per square foot) and the base rent for the 9714 Medical Center Drive Premises will be $104,301.40 per month ($2.65 per square foot), and each will escalate thereafter as described in the Fifth Amendment. The Medical Center Drive Landlord has granted an abatement of all base rent payable under the Amended Medical Center Drive Lease for the first five months of the Extension Term.

The foregoing descriptions of certain terms of the Fifth Amendment and the Amended Medical Center Drive Lease do not purport to be complete and are qualified in their entirety by reference to the full text of the Fifth Amendment and the Amended Medical Center Drive Lease. The Fifth Amendment is included as Exhibit 10.3 to this Quarterly Report on Form 10-Q and incorporated by reference herein.

Termination of Blackwell Road Lease

On November 2, 2020, pursuant to the Lease dated as of January 28, 2016 between 9600 Blackwell II LLC (the Blackwell Landlord), as successor-in-interest to TNREF III 9600 Blackwell, LLC, and the Company (as amended by the First Amendment dated as of November 3, 2017, the Blackwell Lease), the Company provided notice to the Blackwell Landlord of the exercise of the Company's right to terminate the Blackwell Lease effective September 30, 2021. In accordance with the terms of the Blackwell Lease, the Company expects to pay an early termination fee of approximately $430,000 to the Blackwell Landlord.

The foregoing descriptions of certain terms of the Blackwell Lease do not purport to be complete and are qualified in their entirety by reference to the full text of the Blackwell Lease.

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	
10.1*	Compensation Program for Non-Employee Directors				X
10.2†	Fifth Amendment to License Agreement effective September 11, 2020 between the Company and The Trustees of the University of Pennsylvania				X
10.3	Fifth Amendment to Lease dated October 30, 2020 between the Company and ARE-Maryland No. 45, LLC				X
10.4	Third Amendment to Lease dated October 30, 2020 between the Company and ARE-Maryland No. 24, LLC				X
31.1	Certification of the Chief Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
31.2	Certification of the Chief Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
32.1	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350				X
101	The following materials from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended September 30, 2020 formatted in Inline XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (ii) Consolidated Statements of Operations and Comprehensive Income (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 September 30, 2020 formatted in Inline XBRL (included in Exhibit 101)				

* Management contract or compensatory plan or arrangement.
† Portions of this exhibit have been omitted.

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

SIGNATURES

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

REGENXBIO Inc.

Dated: November 4, 2020

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

Dated: November 4, 2020

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

REGENXBIO INC.
COMPENSATION PROGRAM FOR NON-EMPLOYEE DIRECTORS

(as adopted by the Board of Directors on September 22, 2015 and amended on October 29, 2020)

A. Cash Compensation

1. Board retainer: $40,000 per year for each non-employee director, paid in quarterly installments in arrears.

2. Chairman of the Board retainer: $30,000 per year, paid in quarterly installments in arrears.

3. Lead Independent Director retainer: $25,000 per year, paid in quarterly installments in arrears.

4. Committee chair retainer: $18,000 per year for the Audit Committee chair, $15,000 per year for the Compensation Committee chair, and $8,500 per year for the Nominating and Corporate Governance Committee chair, paid in quarterly installments in arrears.

5. Committee member retainer: $9,000 per year for each non-chair member of the Audit Committee, $6,000 per year for each non-chair member of the Compensation Committee, and $5,000 per year for each non-chair member of the Nominating and Corporate Governance Committee, paid in quarterly installments in arrears.

B. Equity Compensation

1. Initial stock option grant: stock option for each non-employee director to purchase 20,000 shares of REGENXBIO Inc. (the "Company") common stock. The per share price of each option shall equal 100% of the Fair Market Value (as defined in the 2015 Equity Incentive Plan (the "EIP")) of a share of common stock of the Company on the date the option is granted. The option shall vest in equal monthly installments over the 36 months following the grant date, with immediate full vesting in the event of a Change in Control (as defined in the EIP). The option will be granted by the Compensation Committee under the EIP in conjunction with the director's initial appointment or election to the Board.

2. Annual stock option grant: stock option for each non-employee director to purchase 10,000 shares of the Company's common stock. The per share price of each option shall equal 100% of the Fair Market Value of a share of common stock of the Company on the date the option is granted. The option shall vest in equal monthly installments over the 12 months following the grant date, with immediate full vesting in the event of a Change in Control. The option will be

granted by the Compensation Committee under the EIP following the election of such director at the Company's annual meeting of stockholders.

C. **Other Items**

1. Non-employee directors will be reimbursed for reasonable out-of-pocket expenses incurred to attend Board and Committee meetings.

2. Customary director and officer insurance is provided for non-employee directors.

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

UNIVERSITY *of* PENNSYLVANIA

Fifth Amendment to License Agreement

This Fifth Amendment to License Agreement (this "*Fifth Amendment*") effective as of September 11, 2020 (this "*Fifth Amendment Effective Date*"), is made by and between The Trustees of the University of Pennsylvania ("*Penn*") and REGENXBIO Inc. ("*Company*") (collectively, the "*Parties*") and amends the License Agreement between the Parties, which was effective as of February 24, 2009, as subsequently amended by a First Amendment dated March 6, 2009, a Second Amendment dated September 9, 2014, a Third Amendment dated April 29, 2016, and a Fourth Amendment dated April 4, 2019 (the "*License Agreement*"). All capitalized terms used but not defined herein shall have the meaning set forth in the License Agreement.

BACKGROUND

WHEREAS, the Parties desire to amend the License Agreement to clarify the division of recoveries in infringement litigation, to correct a scrivener's error in Section 8.5 and to update payment and notice addresses;

NOW, THEREFORE, in consideration of the mutual covenants and promises contained herein, the Parties, intending to be bound, hereby mutually agree to the following:

1. Section 8.5 of the License Agreement is hereby amended and restated in its entirety:

"8.5 Recoveries from Litigation. Except as expressly provided in this Section 8.5 or as otherwise agreed between the parties, if Company prosecutes any infringement claims, Company will use the financial recoveries from such claims, if any, (a) first, to reimburse [****] for its litigation expenditures; and (b) second, to retain any remainder but to treat the remainder as [****] for the purpose of determining [****]. If Company prosecutes any infringement claims with Penn joined as a voluntary party, unless otherwise agreed between the parties, then Company will use the financial recoveries from such claims, if any, (a) first, to reimburse [****] and [****] for their respective litigation expenditures on a dollar-for-dollar basis; and (b) second, to retain any remainder but to [****]. If Penn prosecutes any infringement claims independent of [****], then Penn will prosecute such infringement at [****] expense and will retain any financial recoveries [****]."

2. For clarity, there are no sections 3.6 or 3.7 in the License Agreement.

3. Section 4.6 of the License Agreement is hereby amended and restated in its entirety and replaced by the following:

"4.6 Place of Payment. All payments by Company to Penn hereunder shall be made by deposit of USD in the requisite amount to the "The Trustees of the University of Pennsylvania" and will be made by delivery to any one of the following:

By ACH/Wire:
Wells Fargo Bank, N.A.
ABA #[****] (domestic wires)
SWIFT CODE: [****]
(international wires only)
Account Number: [****]
Payment should include the necessary amount to cover any bank charges incurred.

By Check (lockbox):
The Trustees of the
University of Pennsylvania
c/o Penn Center for Innovation
PO Box 785546
Philadelphia, PA 19178-5546

4. The Notice Addresses of the parties under 13.6 of the License Agreement are hereby updated by the Notice Addresses listed on the Signature Page.

5. This Fifth Amendment amends the terms of the License Agreement and is deemed incorporated into, and governed by all other terms of, the License Agreement. To the extent that the License Agreement is explicitly amended by this Fifth Amendment, the terms of this Fifth Amendment will control where the terms of the License Agreement are contrary to or conflict with the terms of this Fifth Amendment. All other terms and conditions of the License Agreement not explicitly amended by this Fifth Amendment shall remain in full force and effect. The License Agreement, as previously amended, shall, together with this Fifth Amendment, be read and construed as a single instrument.

6. Signatures on this Fifth Amendment may be communicated by facsimile or e-mail transmission and shall be binding upon the Parties upon receipt by transmitting the same by facsimile or e-mail transmission, which signatures shall be deemed originals. If executed in counterparts, the Fifth Amendment shall be effective as if simultaneously executed.

[Intentionally left blank]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this Fifth Amendment to be executed by their duly authorized representatives.

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: /s/ John S. Swartley
Name: John S. Swartley, PhD
Title: Associate Vice Provost for Research and Executive Director, PCI
Date: September 11, 2020

Address:
Penn Center for Innovation
University of Pennsylvania
3600 Civic Center Blvd.,
9th Floor
Philadelphia, PA 19104-4310 Attention: Managing Director

Required copy to:
University of Pennsylvania
Office of General Counsel
2929 Walnut Street, Suite 400
Philadelphia, PA 19104-5509
Officer Attention: General Counsel

REGENXBIO INC.

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President and CEO
Date: 9/11/20

Address:
9600 Blackwell Road
Suite 210
Rockville, MD 20850
Attention: CEO

9600 Blackwell Road
Suite 210
Rockville, MD 20850
Attention: Chief Legal

FIFTH AMENDMENT TO LEASE

THIS FIFTH AMENDMENT TO LEASE ("**this Fifth Amendment**") is made as of this 30th day of October, 2020 ("**Effective Date**"), between **ARE-MARYLAND NO. 45, LLC**, a Delaware limited liability company, having an address at 26 North Euclid Avenue, Pasadena, California 91101 ("**Landlord**"), and **REGENXBIO INC**., a Delaware corporation, having an address at 9712 Medical Center Drive, Rockville, Maryland 20850 ("**Tenant**").

RECITALS

A. BMR-Medical Center Drive LLC, a Delaware limited liability company ("**Original Landlord**"), and Tenant are parties to that certain Lease dated as of March 6, 2015 ("**Original Lease**"), as affected by that certain Acknowledgement of Term Commencement Date and Term Expiration Date dated as of April 17, 2015, given by Tenant in favor of Original Landlord, as amended by that certain First Amendment to Lease dated as of September 30, 2015 ("**First Amendment**"), as further amended by that certain Second Amendment to Lease dated as of November 23, 2015 ("**Second Amendment**"), as further amended by that certain Third Amendment to Lease dated as of July 21, 2017 ("**Third Amendment**"), and as further amended by that certain Fourth Amendment to Lease dated as of April 20, 2018 ("**Fourth Amendment**"; collectively, as amended, the "**Existing Lease**"), whereby Tenant leases certain premises containing an aggregate of 49,360 rentable square feet located (i) in the building at 9712 Medical Center Drive, Rockville, Maryland ("**9712 Building**" and the premises leased therein, "**9712 Building Premises**"), and (ii) in the building at 9714 Medical Center Drive in Rockville, Maryland ("**9714 Building**" and the premises leased therein, "**9714 Building Premises**") (collectively, the "**Premises**").

B. Original Landlord and Tenant are parties to that certain Space License Agreement dated as of February 22, 2016, as amended by that certain First Amendment to Space License Agreement dated as of July 21, 2017 ("**License Agreement**"), whereby Tenant licenses certain storage space comprising approximately 894 square feet located in the 9714 Building ("**License Area**").

C. Original Landlord sold the 9712 Building and 9714 Building to Landlord, as evidenced by a Special Warranty Deed dated as of May 8, 2018 and recorded on May 24, 2018 among the Land Records of Montgomery County, Maryland in Book 56100, Page 90.

D. Landlord and Tenant desire to terminate the License Agreement effective as of September 30, 2021 and, effective as of October 1, 2021, to amend the Rentable Area of Tenant's Premises at the 9714 Building to include the License Area, hereinafter referred to as the "**Storage Area Premises**."

E. Landlord and Tenant further desire to amend the Lease, among other things, to extend the Term and abate certain Base Rent and to expand the Premises to include the License Area.

AGREEMENT

Now, therefore, in consideration of the foregoing Recitals, the mutual promises and conditions contained herein, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Landlord and Tenant hereby agree that the Lease is amended as follows:

1. **Definitions; Recitals**. Terms used in this Fifth Amendment but not otherwise defined shall have the meanings set forth in the Existing Lease. The Recitals form an integral part of this Fifth Amendment and are hereby incorporated by reference.

2. **Termination of License Agreement**. The License Agreement is hereby terminated effective as of midnight Eastern Time on September 30, 2021, and Landlord and Tenant shall have no

further obligations under the License Agreement, except with respect to those provisions that expressly survive the expiration or earlier termination of the License Agreement (including, but not limited to, Section 21 [Indemnification]).

3. **Lease of Storage Area Premises**. Landlord hereby leases to Tenant, and Tenant hereby leases from Landlord, as of October 1, 2021, the Storage Area Premises. From and after October 1, 2021, the term "Premises," as used in the Lease and in this Fifth Amendment, shall mean the 9712 Premises and/or the 9714 Premises and/or the Suite 1114 Premises and/or the Suite 1214 Premises and/or the Additional First Floor Premises and/or the Storage Area Premises, individually and collectively, as the context dictates; the "9714 Building Premises" shall include the 9714 Premises, the Suite 1114 Premises, the Suite 1214 Premises, the Additional First Floor Premises and the Storage Area Premises and Exhibit A to the Existing Lease shall be deleted in its entirety and replaced with **Exhibit A** to this Fifth Amendment. All provisions of the Lease: (a) applicable to the "9714 Premises" shall be applicable to the 9714 Premises as defined in the First Amendment, together with the Suite 1114 Premises and the Suite 1214 Premises, both as defined in the Third Amendment, the Additional First Floor Premises as defined in the Fourth Amendment, and the Storage Area Premises as defined herein, except to the extent otherwise specifically set forth herein or in the Third Amendment or in the Fourth Amendment, and (b) applicable to the "Premises" shall be applicable to the 9712 Premises, the 9714 Premises, the Suite 1114 Premises, the Suite 1214 Premises, the Additional First Floor Premises, and the Storage Area Premises, except to the extent otherwise specifically set forth herein or in the Third Amendment or in the Fourth Amendment.

4. **Rentable Area and Pro Rata Shares**. Effective as of October 1, 2021, the table in Section 3 of the Fourth Amendment shall be deleted in its entirety and replaced with the following:

Definition or Provision	**Means the Following (As of October 1, 2021)**
Premises in 9712 Building	
Approximate Rentable Area of 9712 Premises	10,833 square feet
Premises in 9714 Building	
Approximate Rentable Area of 9714 Premises	19,056 square feet
Approximate Rentable Area of Suite 1114 Premises	5,319 square feet
Approximate Rentable Area of Suite 1214 Premises	11,000 square feet
Approximate Rentable Area of Additional First Floor Premises	3,152 square feet
Approximate Rentable Area of Storage Area Premises	894 square feet
Approximate Rentable Area for all Premises in the 9714 Building (total)	*39,421 square feet*
Building, Campus, Project Size	
Approximate Rentable Area of 9712 Building	22,907 square feet
Approximate Rentable Area of 9714 Building	39,421 square feet

Definition or Provision	**Means the Following (As of the October 1, 2021)**
Approximate Rentable Area of South Campus	93,019 square feet
Approximate Rentable Area of Project	215,619 square feet
Pro Rata Shares	
Tenant's Pro Rata Share of 9712 Building	47.29%
Tenant's Pro Rata Share of 9714 Building for 9714 Premises	48.34%
Tenant's Pro Rata Share of 9714 Building for Suite 1114 Premises	13.49%
Tenant's Pro Rata Share of 9714 Building for Suite 1214 Premises	27.90%
Tenant's Pro Rata Share of 9714 Building for Additional First Floor Premises	8.00%
Tenant's Pro Rata Share of 9714 Building for Storage Area Premises	2.27%
Tenant's Pro Rata Share of 9714 Building (total)	*100.00%*
Tenant's Pro Rata Share of South Campus for 9712 Premises	11.65%
Tenant's Pro Rata Share of South Campus for 9714 Premises	20.49%
Tenant's Pro Rata Share of South Campus for Suite 1114 Premises	5.72%
Tenant's Pro Rata Share of South Campus for Suite 1214 Premises	11.83%
Tenant's Pro Rata Share of South Campus for Additional First Floor Premises	3.39%
Tenant's Pro Rata Share of South Campus for Storage Area Premises	0.96
Tenant's Pro Rata Share of South Campus for all Premises in the 9714 Building (total)	*42.39%*
Tenant's Pro Rata Share of Project for 9712 Premises	5.02%
Tenant's Pro Rata Share of Project for 9714 Premises	8.84%
Tenant's Pro Rata Share of Project for Suite 1114 Premises	2.47%
Tenant's Pro Rata Share of Project for Suite 1214 Premises	5.10%
Tenant's Pro Rata Share of Project for Additional First Floor Premises	1.46%
Tenant's Pro Rata Share of Project for Storage Area Premises	0.41%
Tenant's Pro Rata Share of Project for all Premises in the 9714 Building (total)	*18.28%*

5. **Permitted Use**. The term "Permitted Use" as used in the Lease shall mean, individually and collectively, as the context dictates, the Permitted Use for the 9712 Building and/or the 9714 Building and/or the Storage Premises and all provisions of the Lease applicable to the "Permitted Use" shall be applicable to each of the 9712 Premises, the 9714 Premises, the Suite 1114 Lab/Office Premises, the Suite 1214 Premises, the Storage Premises, the Additional First Floor Premises, and the Storage Area Premises, except to the extent otherwise specifically set forth herein or in the Third Amendment or the Fourth Amendment. From and after the Effective Date, Tenant shall have the right to use the Storage Area Premises, as a Permitted Use, for a freezer farm, laboratory, office, and storage.

6. **Term for Storage Area Premises**. The Term of the Lease for the Storage Area Premises ("**Storage Area Premises Term**") shall commence as of October 1, 2021, and be co-terminous with the Term for the Premises.

7. **Condition of Storage Area Premises**. Tenant acknowledges that (a) it is in possession of and fully familiar with the condition of the Storage Area Premises and, notwithstanding anything contained in the Lease to the contrary, agrees to take the same in its condition "as is" as of October 1, 2021, and (b) Landlord shall have no obligation to alter, repair, or otherwise prepare the Storage Area Premises for Tenant's occupancy or to pay for any improvements to the 9714 Building. Tenant's taking of possession of the Storage Area Premises shall conclusively establish that the Storage Area Premises were at such time in good, sanitary and satisfactory condition and repair; provided, however, that nothing in this grammatical sentence shall take away from Landlord's express obligations under the Lease, including, without limitation, Landlord's repair, maintenance, restoration, and legal compliance obligations expressly set forth in the Lease.

8. **Base Rent for Storage Area Premises**. From the Effective Date to September 30, 2021, Tenant shall continue to pay to Landlord the Fees when due and payable under the License Agreement. During the Storage Area Premises Term, Tenant shall pay to Landlord as Base Rent for the Storage Area Premises, commencing on October 1, 2021, as provided below in Section 14.

9. **Additional Rent**.

a. In addition to Base Rent for the Storage Area Premises, commencing on October 1, 2021 and continuing during the Second Extension Term, Tenant shall pay to Landlord as Additional Rent at times hereinafter specified in this Fifth Amendment (i) Tenant's Storage Area Premises Adjusted Share (as defined below) for the Storage Area Premises, of Operating Expenses (as defined in the Existing Lease) for (A) the 9714 Building, (B) the South Campus, and (C) the Project, (ii) the Property Management Fee (as defined in the Existing Lease), and (iii) any other amounts that Tenant assumes or agrees to pay under the provisions of the Lease that are owed to Landlord, including any and all other sums that may become due by reason of any default of Tenant or failure on Tenant's part to comply with the agreements, terms, covenants and conditions of the Lease to be performed by Tenant, after notice and the lapse of any applicable cure periods. Commencing on October 1, 2021 and continuing during the Second Extension Term, to the extent that Tenant uses more than Tenant's Pro Rata Share of any item of Operating Expenses related to the Storage Area Premises, Tenant shall pay Landlord for such excess in addition to Tenant's obligation to pay Tenant's Pro Rata Share of Operating Expenses (such excess, together with Tenant's Pro Rata Share, "**Tenant's Storage Area Premises Adjusted Share**").

b. Tenant shall pay to Landlord on the first day of each calendar month of the Term, as Additional Rent, commencing on October 1, 2021 and continuing during the Second Extension Term, (i) the Property Management Fee, and (ii) Landlord's good faith estimate of Tenant's Storage Area Premises Adjusted Share of Operating Expenses with respect to the 9714 Building, the South Campus, and the Project for such month.

c. Tenant's responsibility for Tenant's Storage Area Premises Adjusted Share of Operating

Expenses shall continue to the latest of (i) the date of termination of the Lease with respect to the Storage Area Premises, (ii) the date Tenant has fully vacated the Storage Area Premises, and (c) if termination of the Lease is due to a default by Tenant, the date of rental commencement of a replacement tenant. Operating Expenses for the calendar year in which Tenant's obligation to share therein commences and for the calendar year in which such obligation ceases shall be prorated on a basis reasonably determined by Landlord. Expenses such as taxes, assessments, and insurance premiums that are incurred for an extended time period shall be prorated based upon the time periods to which they apply so that the amounts attributed to the Storage Area Premises relate in a reasonable manner to the time period wherein Tenant has an obligation to share in Operating Expenses.

d. Notwithstanding anything in the Existing Lease to the contrary, in the event Tenant's Pro Rata Share of Operating Expenses, excluding Uncontrollable Operating Expenses (defined below), with respect to the Storage Area Premises increases by more than 5% annually, Tenant shall not be obligated to pay for any Tenant's Pro Rata Share of Operating Expenses, excluding Uncontrollable Operating Expenses (defined below), with respect to the Storage Area Premises in excess of such 5% increase (except to the extent that Tenant uses more than its Pro Rata Share of such Operating Expense, as provided in Section 9.a above). For the purposes of this Section, "**Uncontrollable Operating Expenses**" shall mean the costs of utilities, inspection fees and other costs required by any Government Authority for continued compliance with Applicable Laws or to comply with Applicable Laws that are first applicable to the 9712 Building, the 9714 Building, the South Campus and/or the Project (as applicable) after October 1, 2021 with respect to the Storage Area Premises, snow removal, Real Estate Taxes (as defined in the Existing Lease), and insurance.

10. **Utilities and Services for the Storage Area Premises**. The provisions of Section 11 of the First Amendment applicable to the 9714 Premises shall also apply in the same manner to the Storage Area Premises.

11. **Repairs and Maintenance of the Storage Area Premises**. The provisions of Section 12 of the First Amendment applicable to the 9714 Premises shall also apply in the same manner to the Storage Area Premises. Specifically, with respect to the 9714 Premises, the Suite 1114 Premises, the Suite 1214 Premises, the Additional First Floor Premises, and the Storage Area Premises, Landlord shall repair and maintain the structural and exterior portions and Common Area of the 9714 Building and the Project, including roofing and covering materials; foundations (excluding any architectural slabs, but including any structural slabs); exterior walls; plumbing; fire sprinkler systems (if any); HVAC systems; elevators; and electrical systems installed or furnished by Landlord.

12. **Parking**. Notwithstanding the provisions of the Existing Lease, Tenant shall not be entitled to any additional parking spaces related to the Storage Area Premises.

13. **Base Term Extension**. The Extension Term of the Existing Lease expires on September 30, 2021. The Base Term of the Lease with respect to the entire Premises shall be extended for a period of 65 months ("**Second Extension Term**"), beginning on October 1, 2021 and, unless otherwise extended or terminated in accordance with the terms of the Lease, expiring on February 28, 2027. The Options to extend the Term as set forth in Section 42 of the Original Lease (as affected by Section 15 of the Third Amendment) shall continue to be in full force and effect and shall apply to the entire Premises. In light of the expiration date of the Second Extension Term, corresponding changes are necessary for the expiration date of each of the Options. Accordingly, notwithstanding any contrary provision contained in the Lease, the Term following any exercise by Tenant of the first Option would be extended until February 28, 2030, and the Term following any exercise by Tenant of the second Option would be extended until the Termination Date (as defined in that certain Lease Agreement dated as of November 1, 2018 (as amended, the "**9804 MCD Lease**") between Landlord's affiliate, ARE-Maryland No. 24, LLC, a Delaware limited liability company, and Tenant, with respect to premises located at 9804 Medical Center Drive, Rockville, Maryland 20850. The expiration dates set forth in this paragraph are summarized in the table below:

Time Period	Expiration Date
Extension Term	September 30, 2021
Second Extension Term	February 28, 2027
First Option (if exercised by Tenant)	February 28, 2030
Second Option (if exercised by Tenant)	Termination Date (as defined in the 9804 MCD Lease)

14. **Base Rent During Second Extension Term**. Subject to the Base Rent Abatement (as defined below), the Base Rent for the entire Premises for the Second Extension Term shall be as set forth below, without any further escalation and any reference in the Existing Lease to escalation of Base Rent shall not apply to the Second Extension Term. Base Rent, as so adjusted, shall thereafter be due as provided in the Lease. Base Rent adjustments for any fractional calendar month shall be prorated.

Base Rent for the entire 9712 Building Premises during the Second Extension Term:

Dates	Square Feet of Rentable Area	Base Rent per Square Foot of Rentable Area	Annual Base Rent*	Monthly Base Rent*
10/1/2021– 09/30/2022	10,833	$30.72 annually	$332,789.76	$27,732.48
10/1/2022– 09/30/2023	10,833	$31.65 annually	$342,864.45	$28,572.04
10/1/2023– 09/30/2024	10,833	$32.60 annually	$353,155.80	$29,429.65
10/1/2024– 09/30/2025	10,833	$33.57 annually	$363,663.81	$30,305.32
10/1/2025– 09/30/2026	10,833	$34.58 annually	$374,605.14	$31,217.10
10/1/2026– 02/28/2027	10,833	$35.62 annually	$385,871.46	$32,155.96

Base Rent for the entire 9714 Building Premises during the Second Extension Term:

Dates	Square Feet of Rentable Area	Base Rent per Square Foot of Rentable Area	Annual Base Rent*	Monthly Base Rent*
10/1/2021– 09/30/2022	39,421	$31.75 annually	$1,251,616.75	$104,301.40
10/1/2022– 09/30/2023	39,421	$32.71 annually	$1,289,460.91	$107,455.08
10/1/2023– 09/30/2024	39,421	$33.69 annually	$1,328,093.49	$110,674.46
10/1/2024– 09/30/2025	39,421	$34.70 annually	$1,367,908.70	$113,992.39
10/1/2025– 09/30/2026	39,421	$35.74 annually	$1,408,906.54	$117,408.88
10/1/2026– 02/28/2027	39,421	$36.81 annually	$1,451,087.01	$120,923.92

15. **Base Rent Abatement**. Provided a Default does not then exist under the Lease, Landlord hereby grants Tenant an abatement ("**Base Rent Abatement**") of the Base Rent payable under the Lease with respect to the entire Premises for the 5 month period beginning on October 1, 2021 and ending on February 28, 2022. Thereafter, Tenant shall pay the full amount of Base Rent due in accordance with the provisions of the Lease. The Property Management Fee set forth in Section 9.2(w) of the Lease shall not be abated and shall be based on the amount of Base Rent that would have been payable but for the Base Rent Abatement.

16. **Miscellaneous**.

a. This Fifth Amendment is the entire agreement between the parties with respect to the subject matter hereof and supersedes all prior and contemporaneous oral and written agreements and

discussions. This Fifth Amendment may be amended only by an agreement in writing, signed by the parties hereto.

b. This Fifth Amendment is binding upon and shall inure to the benefit of the parties hereto, their respective members, agents, employees, representatives, officers, directors, divisions, subsidiaries, affiliates, assigns, heirs, successors in interest and shareholders.

c. This Fifth Amendment may be executed in 2 or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature process complying with the U.S. federal ESIGN Act of 2000, such as DocuSign) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes. Electronic signatures shall be deemed original signatures for purposes of this Fifth Amendment and all matters related thereto, with such electronic signatures having the same legal effect as original signatures.

d. Landlord and Tenant each represents and warrants that it has not dealt with any broker, agent or other person (collectively, "**Broker**") in connection with this Fifth Amendment and that no Broker brought about this Fifth Amendment, other than Cresa Global, Inc. ("**Cresa**"). Cresa, acting as Tenant's broker, shall be paid by Landlord pursuant to a separate agreement between Landlord and Cresa. Landlord and Tenant each hereby agree to indemnify and hold the other harmless from and against any claims by any Broker, other than Cresa, claiming a commission or other form of compensation by virtue of having dealt with Tenant or Landlord, as applicable, with regard to this leasing transaction.

e. Except as amended and/or modified by this Fifth Amendment, the Lease is hereby ratified and confirmed and all other terms of the Lease shall remain in full force and effect, unaltered and unchanged by this Fifth Amendment. In the event of any conflict between the provisions of this Fifth Amendment and the provisions of the Lease, the provisions of this Fifth Amendment shall prevail. Regardless of whether specifically amended by this Fifth Amendment, all of the terms and provisions of the Lease are hereby amended to the extent necessary to give effect to the purpose and intent of this Fifth Amendment.

[**END OF PAGE**]

IN WITNESS WHEREOF, the parties hereto have executed this Fifth Amendment under seal as of the day and year first above written.

TENANT:

REGENXBIO INC.,
a Delaware corporation

By: /s/ Ken Mills (SEAL)
Name: Ken Mills
Title: President & CEO

LANDLORD:

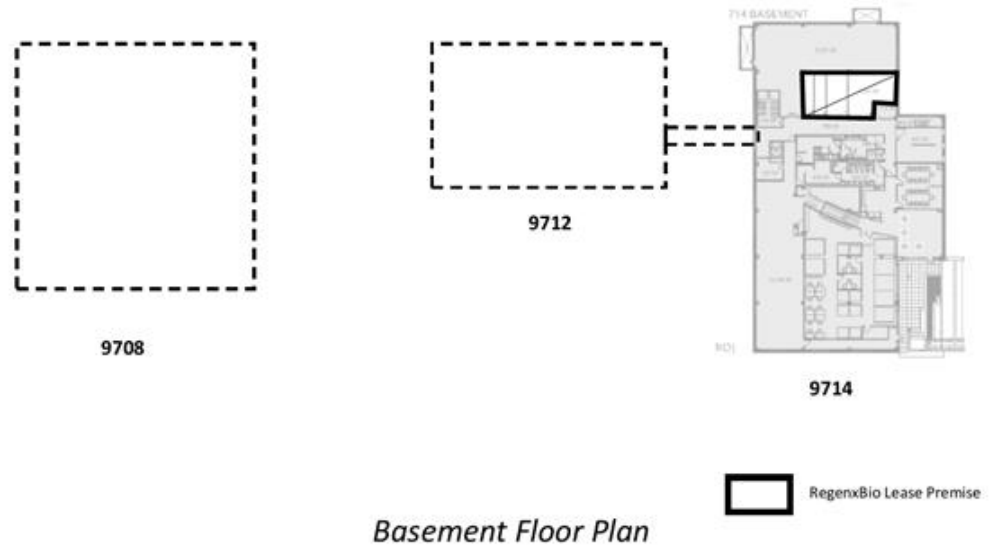
ARE-MARYLAND NO. 45, LLC,
a Delaware limited liability company

By: Alexandria Real Estate Equities, L.P.,
a Delaware limited partnership,
managing member

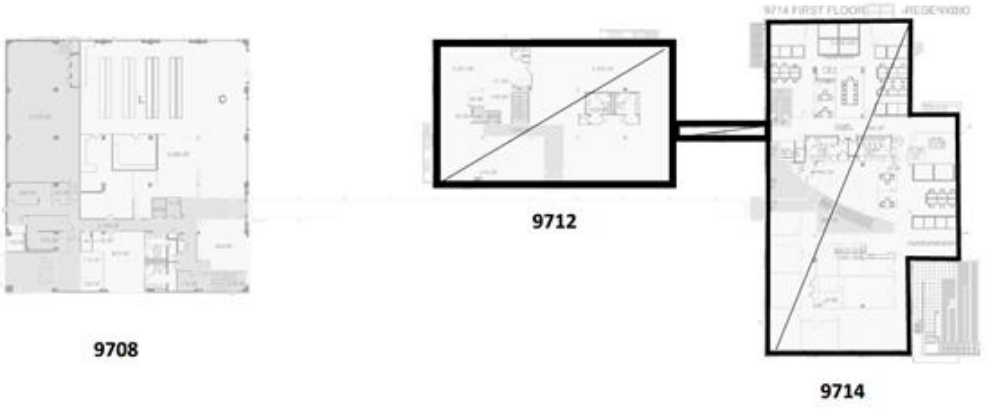
By: ARE-QRS CORP.,
a Maryland corporation,
general partner

By: /s/ Allison Grochola (SEAL)
Name: Allison Grochola
Title: Vice President RE Legal Affairs

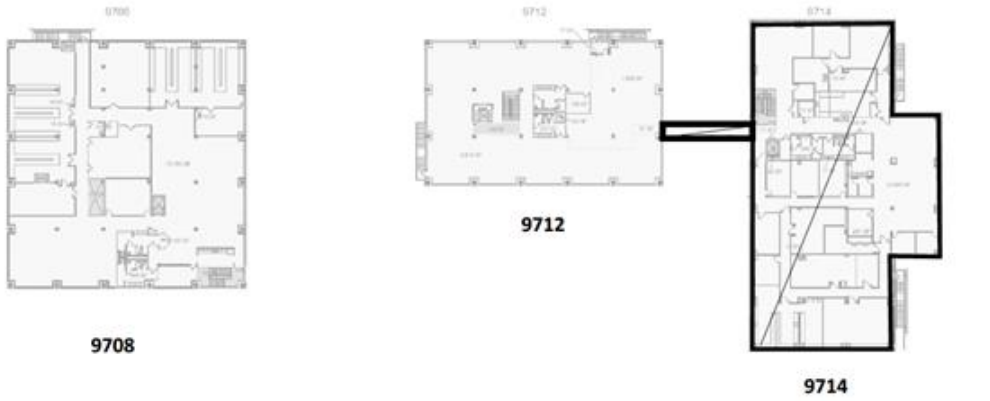
EXHIBIT A



Basement Floor Plan



First Floor Plan



Second Floor Plan

THIRD AMENDMENT TO LEASE AGREEMENT

THIS THIRD AMENDMENT TO LEASE AGREEMENT ("**this Third Amendment**") is dated as of October 30, 2020 ("**Effective Date**"), by and between **ARE-MARYLAND NO. 24, LLC**, a Delaware limited liability company, having an address at 26 North Euclid Avenue, Pasadena, California 91101 ("**Landlord**"), and **REGENXBIO INC.**, a Delaware corporation, having an address at Suite 210, 9600 Blackwell Road, Rockville, Maryland 20850 ("**Tenant**").

RECITALS

A. Landlord and Tenant have entered into that certain Lease Agreement dated as of November 1, 2018 and a letter agreement dated November 1, 2018 ("**Original Lease**"), as amended by a letter agreement dated April 12, 2019, a First Amendment to Lease Agreement dated April 23 2019 ("**First Amendment)**", a Second Amendment to Lease Agreement dated November 4, 2019 ("**Second Amendment**"), and a letter agreement dated as of November 4, 2019 (such letter agreement, together with the Original Lease, the earlier letter agreements, the First Amendment, and the Second Amendment, the "**Lease**"), wherein Landlord leased to Tenant certain premises containing approximately 176,832 rentable square feet ("**Premises**") located at Suite 100, Building F, 9800 Medical Center Drive, Rockville, Maryland 20850, as more particularly described in the Lease.

B. Landlord and Tenant desire to amend the Lease, among other things, to specify the Adjustment Date.

AGREEMENT

Now, therefore, in consideration of the foregoing Recitals, the mutual promises and conditions contained herein, and for other good and valuable consideration, the receipt and legal sufficiency of which are hereby acknowledged, Landlord and Tenant hereby agree that the Lease is amended as follows:

1. **Definitions; Recitals**. Terms used in this Third Amendment but not otherwise defined shall have the meanings set forth in the Lease. The Recitals form an integral part of this Third Amendment and are hereby incorporated by reference.

2. **Adjustment Date**. Notwithstanding any contrary provision contained in the Lease, the Adjustment Date under Section 4 of the Lease shall be September 24, and the first Adjustment Date shall occur on September 24, 2022.

3. **Miscellaneous**.

a. **Entire Agreement**. The Lease, as amended by this Third Amendment, is the entire agreement between the parties with respect to the subject matter hereof and supersedes all prior and contemporaneous oral and written agreements and discussions. The Lease, as so amended by this Third Amendment, may be amended only by an agreement in writing, signed by the parties hereto.

b. **Binding Effect**. This Third Amendment is binding upon and shall inure to the benefit of the parties hereto, their respective agents, employees, members, representatives, officers, directors, divisions, subsidiaries, affiliates, assigns, heirs, successors in interest and shareholders.

c. **Broker**. Landlord and Tenant each represents and warrants that it has not dealt with any broker, agent or other person (collectively, "**Broker**") in connection with this Third Amendment and that no Broker brought about this transaction, other than Tenant's broker, Cresa Global Inc. d/b/a Cresa ("**Cresa**"). Cresa shall be paid by Landlord pursuant to a separate agreement between Landlord and Cresa. Landlord and Tenant each hereby agree to indemnify, defend, and hold the other harmless from and against any claims by any Broker, other than Cresa, claiming a commission or other form of compensation by virtue of having dealt with Tenant or Landlord, as applicable, with regard to this Third Amendment.

d. **Counterparts**. This Third Amendment may be executed in 2 or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument. Counterparts may be delivered via facsimile, electronic mail (including pdf or any electronic signature process complying with the U.S. federal ESIGN Act of 2000, such as DocuSign) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes. Electronic signatures shall be deemed original signatures for purposes of this Third Amendment and all matters related thereto, with such electronic signatures having the same legal effect as original signatures.

e. **Ratification; Conflicts**. Except as amended and/or modified by this Third Amendment, the Lease is hereby ratified and confirmed and all other terms of the Lease shall remain in full force and effect, unaltered and unchanged by this Third Amendment. In the event of any conflict between the provisions of this Third Amendment and the provisions of the Lease, the provisions of this Third Amendment shall prevail. Regardless of whether specifically amended by this Third Amendment, all of the terms and provisions of the Lease are hereby amended to the extent necessary to give effect to the purpose and intent of this Third Amendment.

[**SIGNATURES APPEAR ON NEXT PAGE**]

IN WITNESS WHEREOF, the parties hereto have executed this Third Amendment under seal as of the day and year first above written.

TENANT:

REGENXBIO INC.,
a Delaware corporation

By: /s/ Kenneth Mills (SEAL)
Name: Kenneth Mills
Title: President & CEO

LANDLORD:

ARE-MARYLAND NO. 24, LLC,
a Delaware limited liability company

By: Alexandria Real Estate Equities, L.P.,
a Delaware limited partnership,
managing member

By: ARE-QRS CORP.,
a Maryland corporation,
general partner

By: /s/ Allison Grochola (SEAL)
Name: Allison Grochola
Title: Vice President, RE Legal Affairs

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: November 4, 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: November 4, 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 September 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: November 4, 2020

/s/ Kenneth T. Mills

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

Date: November 4, 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.