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, 2019

OR

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

For the transition period from ___ to ___

Commission File Number 001-37553

REGENXBIO Inc.

(Exact Name of Registrant as Specified in its Charter)

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

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

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

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

Title of each class	Ticker 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 November 1, 2019, there were 36,874,282 outstanding shares of the registrant's common stock, par value $0.0001 per share.

REGENXBIO INC.
QUARTERLY REPORT ON FORM 10-Q
FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2019

TABLE OF CONTENTS

PART I—FINANCIAL INFORMATION

Item 1.	Financial Statements (Unaudited)	3
	Consolidated Balance Sheets as of September 30, 2019 and December 31, 2018	3
	Consolidated Statements of Operations and Comprehensive Income (Loss) for the Three and Nine Months Ended September 30, 2019 and 2018	4
	Consolidated Statements of Stockholders' Equity for the Three and Nine Months Ended September 30, 2019 and 2018	5
	Consolidated Statements of Cash Flows for the Nine Months Ended September 30, 2019 and 2018	7
	Notes to Consolidated Financial Statements	8
Item 2.	Management's Discussion and Analysis of Financial Condition and Results of Operations	30
Item 3.	Quantitative and Qualitative Disclosures About Market Risk	43
Item 4.	Controls and Procedures	43

PART II—OTHER INFORMATION

Item 1.	Legal Proceedings	44
Item 1A.	Risk Factors	44
Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	46
Item 3.	Defaults Upon Senior Securities	46
Item 4.	Mine Safety Disclosures	46
Item 5.	Other Information	46
Item 6.	Exhibits	48
Signatures		49

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 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;
- our expectations regarding the imposition by the U.S. Food and Drug Administration (the FDA) of a partial clinical hold on our investigational new drug application for RGX-314 for the treatment of wet age-related macular degeneration, including our ability to resolve issues raised by the FDA and the timing of any lifting of the hold by the FDA;
- the ability to obtain and maintain regulatory approval of our product candidates, and the labeling for any approved 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 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 product candidates;
- our ability to establish and maintain development partnerships;
- our expectations regarding our expenses and revenue;
- 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, 2018 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, 2019	December 31, 2018
Assets		
Current assets		
Cash and cash equivalents	$ 79,594	$ 75,561
Marketable securities	252,125	244,200
Accounts receivable	19,175	8,587
Prepaid expenses	6,920	5,734
Other current assets	2,414	3,831
Total current assets	360,228	337,913
Marketable securities	85,408	150,819
Accounts receivable	24,345	23,012
Property and equipment, net	28,287	28,702
Operating lease right-of-use assets	5,905	—
Restricted cash	1,053	1,053
Other assets	4,011	2,315
Total assets	$ 509,237	$ 543,814
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$ 9,608	$ 4,412
Accrued expenses and other current liabilities	19,231	17,164
Deferred revenue	—	600
Operating lease liabilities	2,506	—
Total current liabilities	31,345	22,176
Deferred revenue	3,333	3,333
Operating lease liabilities	4,320	—
Deferred rent	—	1,098
Financing lease obligations	—	5,854
Other liabilities	1,844	2,505
Total liabilities	40,842	34,966
Commitments and contingencies (Note 7)		
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at September 30, 2019 and December 31, 2018	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at September 30, 2019 and December 31, 2018; 36,840 and 36,120 shares issued and outstanding at September 30, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	619,386	592,580
Accumulated other comprehensive income (loss)	363	(720)
Accumulated deficit	(151,358)	(83,016)
Total stockholders' equity	468,395	508,848
Total liabilities and stockholders' equity	$ 509,237	$ 543,814

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,	
	2019	2018	2019	2018
Revenues				
License and royalty revenue	$ 14,700	$ 5,306	$ 23,465	$ 177,728
Total revenues	14,700	5,306	23,465	177,728
Operating Expenses				
Cost of revenues	2,494	517	4,450	6,797
Research and development	35,692	18,508	90,378	59,544
General and administrative	12,402	9,008	37,365	25,706
Other operating expenses (income)	8	(2)	(54)	31
Total operating expenses	50,596	28,031	132,139	92,078
Income (loss) from operations	(35,896)	(22,725)	(108,674)	85,650
Other Income				
Interest income from licensing	716	109	2,091	8,362
Investment income	431	2,122	37,950	4,177
Total other income	1,147	2,231	40,041	12,539
Income (loss) before income taxes	(34,749)	(20,494)	(68,633)	98,189
Income Tax Benefit (Expense)	165	1,292	364	(2,558)
Net income (loss)	$ (34,584)	$ (19,202)	$ (68,269)	$ 95,631
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense	(108)	(103)	1,043	(159)
Total other comprehensive income (loss)	(108)	(103)	1,043	(159)
Comprehensive income (loss)	$ (34,692)	$ (19,305)	$ (67,226)	$ 95,472
Net income (loss) applicable to common stockholders	$ (34,584)	$ (19,202)	$ (68,269)	$ 95,631
Net income (loss) per share:				
Basic	$ (0.94)	$ (0.56)	$ (1.86)	$ 2.94
Diluted	$ (0.94)	$ (0.56)	$ (1.86)	$ 2.67
Weighted-average common shares outstanding:				
Basic	36,813	33,988	36,618	32,576
Diluted	36,813	33,988	36,618	35,875

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, 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 of reclassifications and income tax expense	—	—	—	(108)	—	(108)
Net loss	—	—	—	—	(34,584)	(34,584)
Balances at September 30, 2019	36,840	$ 4	$ 619,386	$ 363	$ (151,358)	$ 468,395

	Three Months Ended September 30, 2018					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at June 30, 2018	32,275	$ 3	$ 386,110	$ (771)	$ (68,120)	$ 317,222
Issuance of common stock upon public offering, net of transaction costs of $12,728	3,105	1	189,096	—	—	189,097
Exercise of stock options	307	—	2,053	—	—	2,053
Issuance of common stock under employee stock purchase plan	17	—	507	—	—	507
Stock-based compensation expense	—	—	4,483	—	—	4,483
Unrealized loss on available-for-sale securities, net of reclassifications and income tax expense	—	—	—	(103)	—	(103)
Net loss	—	—	—	—	(19,202)	(19,202)
Balances at September 30, 2018	35,704	$ 4	$ 582,249	$ (874)	$ (87,322)	$ 494,057

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, 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 of reclassifications and income tax expense	—	—	—	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

	Nine Months Ended September 30, 2018					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balances at December 31, 2017	31,295	$ 3	$ 371,497	$ (715)	$ (187,756)	$ 183,029
Adoption of ASU 2014-09 (Topic 606)	—	—	—	—	4,803	4,803
Issuance of common stock upon public offering, net of transaction costs of $12,728	3,105	1	189,096	—	—	189,097
Exercise of stock options	1,267	—	9,051	—	—	9,051
Issuance of common stock under employee stock purchase plan	37	—	850	—	—	850
Stock-based compensation expense	—	—	11,755	—	—	11,755
Unrealized loss on available-for-sale securities, net of reclassifications and income tax expense	—	—	—	(159)	—	(159)
Net income	—	—	—	—	95,631	95,631
Balances at September 30, 2018	35,704	$ 4	$ 582,249	$ (874)	$ (87,322)	$ 494,057

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,	
	2019	2018
Cash flows from operating activities		
Net income (loss)	$ (68,269)	$ 95,631
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities		
Stock-based compensation expense	19,979	11,755
Net amortization of premiums and accretion of discounts on marketable debt securities	(1,085)	889
Depreciation and amortization	5,229	2,671
Net realized losses (gains) on sales and maturities of marketable securities	(20)	15
Imputed interest income from licensing	(2,091)	(8,362)
Unrealized gains on marketable equity securities	(29,420)	—
Other non-cash adjustments	357	12
Changes in operating assets and liabilities		
Accounts receivable	(10,699)	6,986
Prepaid expenses	(1,418)	(974)
Other current assets	1,286	(3,072)
Operating lease right-of-use assets	1,712	—
Other assets	(2,150)	(788)
Accounts payable	5,339	156
Accrued expenses and other current liabilities	1,990	3,438
Deferred revenue	(600)	600
Operating lease liabilities	(1,832)	—
Deferred rent	—	(41)
Other liabilities	(507)	(128)
Net cash provided by (used in) operating activities	(82,199)	108,788
Cash flows from investing activities		
Purchases of marketable securities	(127,925)	(319,897)
Maturities of marketable securities	218,019	116,110
Purchases of property and equipment	(10,689)	(8,389)
Net cash provided by (used in) investing activities	79,405	(212,176)
Cash flows from financing activities		
Proceeds from exercise of stock options	5,513	9,051
Proceeds from issuance of common stock under employee stock purchase plan	1,314	850
Proceeds from public offerings of common stock, net of underwriting discounts and commissions	—	189,716
Issuance costs for public offerings of common stock	—	(462)
Net cash provided by financing activities	6,827	199,155
Net increase in cash and cash equivalents and restricted cash	4,033	95,767
Cash and cash equivalents and restricted cash		
Beginning of period	76,614	46,881
End of period	$ 80,647	$ 142,648
Supplemental disclosures of non-cash investing and financing activities		
Purchases of property and equipment in accounts payable and accrued expenses	$ —	$ 174
Issuance costs for public offerings of common stock in accounts payable and accrued expenses	$ —	$ 157

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 leading 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 Company's 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 candidates in multiple therapeutic areas. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

Liquidity and Risks

As of September 30, 2019, the Company had generated an accumulated deficit of $151.4 million since inception. As the Company has incurred cumulative losses since inception, transition to recurring profitability is dependent upon the successful development, approval and commercialization of its product candidates and achieving a level of revenues adequate to support the Company's cost structure. The Company may never achieve recurring profitability, and unless and until it does, the Company will continue to need to raise additional capital. As of September 30, 2019, the Company had cash, cash equivalents and marketable securities of $417.1 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, 2018 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on February 27, 2019. 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, 2018, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

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

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Actual results could differ materially from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these consolidated financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the consolidated financial statements. Significant estimates are used in the following areas, among others: license and royalty revenue, stock-based compensation expense, accrued research and development expenses and other accrued liabilities, income taxes and the fair value of financial instruments.

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, 2019	September 30, 2018
Cash and cash equivalents	$ 79,594	$ 142,423
Restricted cash	1,053	225
Total cash and cash equivalents and restricted cash	$ 80,647	$ 142,648

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 (loss) 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 (loss). 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.

A decline in the fair value below cost of available-for-sale debt securities that is deemed other-than-temporary is charged to results of operations, resulting in the establishment of a new cost basis for the security. The Company regularly evaluates whether declines in the fair value of its debt securities below their cost are other-than-temporary. The evaluation includes consideration of the cause of the impairment, including the creditworthiness of the security issuers, the number of securities in an unrealized loss position, the severity and duration of the unrealized losses, whether the Company has the intent to sell the securities and whether it is more likely than not that the Company will be required to sell the securities before the recovery of their amortized cost basis. The Company has not recorded any impairment of available-for-sale debt securities which was deemed to be to be other-than-temporary.

Leases

Effective January 1, 2019, the Company adopted Accounting Standards Update (ASU) 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in Accounting Standards Codification (ASC) 840, *Leases* (Topic 840). Please refer to Recent Accounting Pronouncements below for additional information on the adoption of Topic 842 and the impact upon adoption to the Company's consolidated financial statements.

Under Topic 842, the Company classifies its leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the Company. Lease classification is evaluated at the inception of the lease agreement. Regardless of classification, the Company records a right-of-use asset and a lease liability for all leases with a term greater than 12 months. All of the Company's leases as of September 30, 2019 have been classified as operating leases. Operating lease expense is recognized on a straight-line basis over the term of the lease, with the exception of variable lease expenses which are recognized as incurred.

The Company identifies leases in its contracts if the contract conveys the right to control the use of identified property, plant or equipment for a period of time in exchange for consideration. The Company does not allocate lease consideration between lease and nonlease components and records a lease liability equal to the present value of the remaining fixed consideration under the lease. The interest rates implicit in the Company's leases are generally not readily determinable. Accordingly, the Company uses its estimated incremental borrowing rate at the commencement date of the lease to determine the present value discount of the lease liability. The Company estimates its incremental borrowing rate for each lease based on an evaluation of its expected credit rating and the prevailing market rates for collateralized debt in a similar economic environment with similar payment terms and maturity dates commensurate with the term of the lease. The right-of-use asset for each lease is equal to the lease liability, adjusted for unamortized initial direct costs and lease incentives and prepaid or accrued rent. Initial direct costs of entering into a lease are included in the right-of-use asset and amortized as lease expense over the term of the lease. Lease incentives, such as tenant improvements allowances, are recorded as a reduction of the right-of-use asset and amortized as a reduction of lease expense over the term of the lease. The Company excludes options to extend or terminate leases from the calculation of the lease liability unless it is reasonably certain the option will be exercised.

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. 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.

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* (Topic 606). Topic 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of Topic 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

The Company applies the five-step model to contracts that are within the scope of Topic 606 only when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, for contracts within the scope of Topic 606, the Company assesses the goods or services promised within each contract and determine those that are performance obligations and whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

The Company evaluates its contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, the Company evaluates the credit profile of the customer and prevailing market interest rates and selects an interest rate in which it believes would be charged to the customer in a separate financing arrangement over a similar financing term.

License and Royalty Revenue

The Company licenses its 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 Company's 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 payable 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.

The Company's license agreements are accounted for as contracts with customers within the scope of Topic 606. At the inception of each license agreement, the Company determines the contract term for purposes of applying the requirements of Topic 606. Licenses are generally terminable at the option of the licensee with advance notice to the Company. For each license, the Company evaluates these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options. The determination of whether a substantive termination penalty is associated with the termination rights requires significant judgment. In making this determination, the Company considers, among other things, the nature of the intellectual property rights that would be returned to the Company upon termination, including the exclusivity of the licensed rights and the stage of development of the licensed products, the payment terms, including the amount and timing of non-refundable or guaranteed payments, and the business purpose of the termination rights granted to the licensee. Generally, the most significant judgment in determining whether a substantive termination penalty exists is the related to amount of any up-front or guaranteed non-refundable payments relative to the amount of annual payments that may be avoided by the licensee upon termination of the license. The Company considers all of the facts and circumstances relevant to each license when making this determination.

Performance obligations under the Company's license agreements may include (i) the delivery of intellectual property licenses and (ii) options granted to licensees to acquire additional licenses to the extent the options represent material rights to the licensee. At the inception of each license agreement which contains options for the licensee to acquire additional licenses, or contract renewal options, the Company evaluates the options to determine whether they provide material rights to the licensee. In making this determination, the Company considers whether the options are priced at a discount to the standalone selling price for the underlying licenses. If an option is priced at a discount to the standalone selling price for the underlying license, the option is considered to be a material right to the licensee and is accounted for as a separate performance obligation under the current license agreement.

The Company evaluates the transaction price of its license agreements at the inception of each agreement and at each reporting date. The transaction price includes the fixed consideration payable to the Company during the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the license agreements includes up-front and annual fees payable during the contract term. Variable consideration under the license agreements includes development and sales-based milestone payments, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by a licensee is excluded from the transaction price and not accounted for as part of the license agreement until the option is exercised.

The transaction price for each license agreement is allocated to the underlying performance obligations and recognized as revenue when the performance obligations are satisfied. Consideration allocated to performance obligations for the delivery of an intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. Consideration allocated to performance obligations for license options is recognized as revenue in full upon the earlier of the option exercise or expiration. The exercise of a license option by a licensee is accounted for as a new license for revenue recognition purposes.

Up-front and annual licenses fees payable to the Company over the contract term of each license are included in the transaction price, and the portion of this consideration that is allocated to the performance obligation for the delivery of the intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. If annual license fees are payable to the Company in periods beyond 12 months from the delivery of the license, a significant financing component is deemed to exist which provides a financing benefit to the licensee. If a significant financing component is identified, the Company adjusts the transaction price for the license to include only the present value of the annual license fees payable to the Company over the contract term. The discounted portion of the license fees is recognized as interest income from licensing over the financing period of the license.

Development milestone payments are payable to the Company upon the achievement of specified development milestones by licensees. At the inception of each license agreement that contains development milestone payments, the Company evaluates whether the milestones are considered probable of achievement and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price and recognized as revenue upon the delivery of the license. Milestone payments contingent on the achievement of development milestones that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, the Company re-evaluates the probability of achievement of outstanding development milestones and, if necessary, adjusts the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. Any such adjustments are recorded on a cumulative catch-up basis and recognized as revenue in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

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). Zolgensma is a licensed product under the Company's March 2014 license agreement, as amended, with AveXis for the development and commercialization of treatments for spinal muscular atrophy (SMA). The Company recognizes royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by AveXis, which in certain cases may require the Company to estimate royalty revenue for periods of net sales which have not yet been reported to the Company. Sales-based milestone payments related to net sales of Zolgensma are recognized as royalty revenue in the period in which the milestone is achieved.

The Company receives payments from licensees based on the billing schedules established in each license agreement. Amounts recognized as revenue which have not yet been received from licensees, including unbilled royalties, are recorded as accounts receivable when the Company's rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from licensees are recorded as contract assets when the Company's rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets. 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 consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of license revenue in the period of the termination. Amounts received by the Company prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations by the Company. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

Cost of Revenues

Cost of revenues consists primarily of sublicense fees, milestone payments and royalties on net sales of licensed products as specified in the Company's agreements with its licensors. Sublicense fees are based on a percentage of license fees received by the Company from NAV Technology Licensees and are recognized in the period that the underlying 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. Amounts which are payable to licensors in periods beyond 12 months from the reporting date are recorded as non-current liabilities on the consolidated balance sheets.

Collaborative Arrangements

The Company evaluates its collaboration arrangements to determine whether they are within the scope of ASC 808, *Collaborative Arrangements* (Topic 808). Such arrangements are within the scope of Topic 808 if they involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This evaluation is performed throughout the life of the arrangement based on any changes in the roles and responsibilities of the parties under the arrangement. For arrangements within the scope of Topic 808, the Company identifies the various transactions with the counterparty and determines if any unit of account is more reflective of a transaction with a customer and therefore should be accounted for within the scope of Topic 606. For transactions that are accounted for pursuant to Topic 808, an appropriate method of recognition and presentation is determined and consistently applied. Amounts that are owed to collaboration partners for development activities are recognized as research and development expenses as incurred by the collaboration partner. Amounts received from collaboration partners for development activities are recognized as a reduction of research and development expenses as incurred by the Company. The Company does not have any collaborative arrangements containing transactions with customers accounted for under Topic 606.

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

Adoption of ASU 2016-02, Leases

In February 2016, the Financial Accounting Standards Board (FASB) issued ASU 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in ASC 840, *Leases* (Topic 840). Effective January 1, 2019, the Company adopted Topic 842 using the modified retrospective transition method. Under this method, the Company applied Topic 842 to all leases in effect as of, or entered into after, January 1, 2019 and recorded the cumulative impact of the adoption as an adjustment to its accumulated deficit on January 1, 2019. The Company's consolidated financial statements for periods ending after January 1, 2019 are presented in accordance with the requirements of Topic 842, while comparative prior period amounts have not been adjusted and continue to be reported in accordance with Topic 840. Please refer to Leases above for a description of the Company's lease accounting policies upon the adoption on Topic 842.

The Company elected certain practical expedients allowed by Topic 842 for transition purposes, including the package of practical expedients which permitted the Company to not reassess lease identification, classification and initial direct costs under Topic 842 for leases that commenced prior to January 1, 2019. Additionally, the Company elected the practical expedient allowed for transition purposes to use hindsight in determining the terms of leases that commenced prior to January 1, 2019.

Upon the adoption of Topic 842, the Company recorded operating lease right-of-use assets of $7.4 million and operating lease liabilities of $8.4 million for its leases which were in effect and had commenced prior to January 1, 2019 and had original lease terms of more than 12 months. The Company also derecognized current and non-current deferred rent liabilities of $1.4 million and prepaid expenses, other current assets and other assets of $0.4 million upon the adoption of Topic 842. Additionally, upon the adoption of Topic 842, the Company derecognized $5.9 million of property and equipment and $5.9 million of financing lease obligations related to construction-in-progress at 9800 Medical Center Drive, as the Company does not control the building during the construction period under the requirements of Topic 842. The lease term for the facility at 9800 Medical Center Drive does not commence until certain construction is completed by the landlord and the building is delivered to the Company. The right-of-use assets and lease liabilities related to the facility at 9800 Medical Center Drive will not be recognized on the Company's consolidated balance sheets until the commencement date of the lease, which is expected to occur in 2020.

The cumulative impact of the adoption of Topic 842 resulted in an increase in accumulated deficit of less than $0.1 million on January 1, 2019. The adoption of Topic 842 did not have a material impact on the Company's results of operations for the three and nine months ended September 30, 2019, nor does the Company believe it will have a material impact on future results of operations based on its current leasing arrangements.

Other Recently Adopted Accounting Pronouncements

In November 2018, the FASB issued ASU 2018-18, *Collaborative Arrangements (Topic 808): Clarifying the Interaction Between Topic 808 and Topic 606*. The standard clarifies that certain transactions between participants of a collaborative arrangement should be accounted for as revenue under Topic 606 when the counterparty in the collaborative arrangement is a customer in the context of a unit of account. Additionally, the standard precludes entities from presenting consideration received from a participant in a collaborative arrangement with revenue recognized under Topic 606 if the participant is not a customer. The Company early adopted this standard effective July 1, 2019 and applied the standard retrospectively to all relevant contracts that were not completed as of the date of adoption. As of July 1, 2019, the Company had no contracts within the scope of Topic 808, thus 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.

In February 2018, the FASB issued ASU 2018-02, *Income Statement—Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income*, which amends the previous guidance on comprehensive income to provide an option for an entity to reclassify the stranded tax effects of the Tax Cuts and Jobs Act of 2017 (the TCJA) that was signed into law in December 2017 from accumulated other comprehensive income directly to retained earnings. The stranded tax effects result from the remeasurement of deferred tax assets and liabilities which were originally recorded in comprehensive income but whose remeasurement is reflected in the income statement. The Company adopted this standard effective January 1, 2019, and upon adoption recorded a cumulative adjustment of less than $0.1 million to reclassify the stranded tax effects of unrealized gains and losses on available-for-sale securities from accumulated other comprehensive income (loss) to accumulated deficit. The adoption of this standard did not have a material impact on the Company's financial position or results of operations.

In April 2017, the FASB issued ASU 2017-08, *Receivables—Nonrefundable Fees and Other Costs (Subtopic 310-20)*, which amends the required amortization period for certain purchased callable debt securities held at a premium by shortening the amortization period for the premium to the earliest call date. The Company adopted this standard effective January 1, 2019. 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.

Recent Accounting Pronouncements Not Yet Adopted

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 standard is effective for the Company beginning January 1, 2020, with early adoption permitted upon issuance, and may be applied either retrospectively or prospectively to all implementation costs incurred after the date of adoption. The Company is evaluating the application of this standard but has not yet determined the potential effects it may have on its consolidated financial statements.

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 standard is effective for the Company beginning January 1, 2020, with early adoption permitted upon issuance. The Company does not believe the application of this standard will have a material impact on its financial statement disclosures.

In June 2016, the FASB issued 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. An 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 is effective for the Company beginning January 1, 2020, with early adoption permitted for annual and interim periods beginning January 1, 2019. The Company does not believe the application of this standard will have a material impact on its 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	Unrealized Gains	Unrealized Losses	Fair Value
September 30, 2019				
U.S. government and federal agency securities	$ 78,093	$ 296	$ (3)	$ 78,386
Certificates of deposit	7,529	87	—	7,616
Corporate bonds	220,694	1,018	(21)	221,691
Equity securities	420	29,420	—	29,840
	$ 306,736	$ 30,821	$ (24)	$ 337,533

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2018				
U.S. government and federal agency securities	$ 103,410	$ 93	$ (37)	$ 103,466
Certificates of deposit	8,992	—	—	8,992
Corporate bonds	282,902	36	(377)	282,561
	$ 395,304	$ 129	$ (414)	$ 395,019

As of September 30, 2019 and December 31, 2018, 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, 2019 and December 31, 2018, the balance in the Company's accumulated other comprehensive income (loss) 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, 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 periods. 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 (loss) during the periods and were included in investment income in the consolidated statements of operations and comprehensive income (loss). During the three and nine months ended September 30, 2018, the Company recognized net unrealized losses on available-for-sale debt securities of $0.1 million and $0.2 million, respectively, and income tax expense of zero in other comprehensive income (loss) for the periods. The Company recognized net realized losses 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, 2018, which were reclassified out of accumulated other comprehensive income (loss) during the periods and were included in investment income in the consolidated statements of operations and comprehensive income (loss). Realized gains and losses from the sale or maturity of available-for-sale debt securities are determined based on the specific identification method.

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, 2019						
U.S. government and federal agency securities	$ 4,497	$ (3)	$ —	$ —	$ 4,497	$ (3)
Corporate bonds	23,354	(21)	—	—	23,354	(21)
	$ 27,851	$ (24)	$ —	$ —	$ 27,851	$ (24)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2018						
U.S. government and federal agency securities	$ 53,124	$ (37)	$ —	$ —	$ 53,124	$ (37)
Corporate bonds	245,283	(354)	12,424	(23)	257,707	(377)
	$ 298,407	$ (391)	$ 12,424	$ (23)	$ 310,831	$ (414)

As of September 30, 2019, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 7 investment grade security positions. The Company has the intent and ability to hold such securities until recovery and has determined that none of its available-for-sale debt securities were other-than-temporarily impaired as of September 30, 2019 or December 31, 2018.

Marketable equity securities held by the Company as of September 30, 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. The Company recognized unrealized gains (losses) of $(2.2) million and $29.4 million during the three and nine months ended September 30, 2019, respectively, related to the marketable equity securities of Prevail, which are included in investment income in the consolidated statements of operations and comprehensive income (loss). Pursuant to a lock-up agreement executed in connection with Prevail's IPO, the Company is restricted from selling its common stock of Prevail prior to December 2019. As of September 30, 2019, no amounts have been realized by the Company related to its marketable securities of Prevail as there have been no sales of the securities.

4. Fair Value of Financial Instruments

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

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
September 30, 2019				
Cash equivalents:				
Money market mutual funds	$ —	$ 72,634	$ —	$ 72,634
Corporate bonds	—	5,905	—	5,905
Total cash equivalents	—	78,539	—	78,539
Marketable securities:				
U.S. government and federal agency securities	—	78,386	—	78,386
Certificates of deposit	—	7,616	—	7,616
Corporate bonds	—	221,691	—	221,691
Equity securities	29,840	—	—	29,840
Total marketable securities	29,840	307,693	—	337,533
Total cash equivalents and marketable securities	$ 29,840	$ 386,232	$ —	$ 416,072

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2018				
Cash equivalents:				
Money market mutual funds	$ —	$ 75,542	$ —	$ 75,542
Total cash equivalents	—	75,542	—	75,542
Marketable securities:				
U.S. government and federal agency securities	—	103,466	—	103,466
Certificates of deposit	—	8,992	—	8,992
Corporate bonds	—	282,561	—	282,561
Total marketable securities	—	395,019	—	395,019
Total cash equivalents and marketable securities	$ —	$ 470,561	$ —	$ 470,561

There were no transfers of financial instruments between levels of the fair value hierarchy during the nine months ended September 30, 2019.

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, 2019 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 December 31, 2018, non-marketable equity securities had a carrying value of $0.4 million and were included in other assets on the consolidated balance sheets. The Company did not identify any observable price changes or changes in circumstances that would have had an adverse effect on the fair value of the securities as of December 31, 2018. During the nine months ended September 30, 2019, all of the Company’s non-marketable equity securities were reclassified to marketable securities and as of September 30, 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, 2019 and 2018.

5. Property and Equipment, Net

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

	September 30, 2019	December 31, 2018
Lab equipment	$ 18,492	$ 14,417
Computer equipment and software	2,448	2,002
Furniture and fixtures	1,960	1,915
Leasehold improvements	17,831	11,751
Construction-in-progress	—	5,854
Total property and equipment	40,731	35,939
Accumulated depreciation and amortization	(12,444)	(7,237)
Property and equipment, net	$ 28,287	$ 28,702

Construction-in-progress reported in the table above as of December 31, 2018 consisted of certain costs incurred and reported by the Company's landlord at 9800 Medical Center Drive. Upon the adoption of Topic 842 on January 1, 2019, the Company derecognized the cumulative amount of construction costs incurred by the landlord of $5.9 million. Please refer to Note 2 for further information on the Company's adoption of Topic 842 and Note 6 for further information on the Company's lease at 9800 Medical Center Drive.

6. Leases

9800 Medical Center Drive

In November 2018, the Company entered into a lease agreement, as amended in April 2019, for approximately 139,000 square feet of office and laboratory facilities in a new building to be constructed at 9800 Medical Center Drive in Rockville, Maryland (the 9800 Medical Center Drive Lease). The initial construction of the building is being conducted by the landlord and is expected to be completed in 2020, after which the leased premises will be delivered to the Company to make additional improvements to the building. Pursuant to the amended lease agreement, the Company will receive a $15.3 million tenant improvement allowance from the landlord to construct additional improvements to the leased premises. The lease expires approximately 16 years from delivery of the leased premises to the Company, subject to certain 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 after 12 years from the delivery of the leased premises to the Company. If the Company elects to terminate the lease, it will be subject to a termination fee equal to the unamortized tenant improvement allowance, rent abatement and landlord commissions as of the termination date, bearing interest at 5% per annum, plus four months of base rent and operating expenses. Additionally, after the delivery of the leased premises under the 9800 Medical Center Drive Lease, the Company will have the option to terminate its lease at 9712 Medical Center Drive with six months' notice. Monthly payments under the 9800 Medical Center Drive Lease begin approximately 12 months from the delivery of the leased premises to the Company and escalate annually in accordance with the lease agreement. As required by the lease agreement, the Company has provided the landlord with an irrevocable letter of credit of $0.8 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease.

The Company is involved in the construction project for the leased premises at 9800 Medical Center Drive, including having the responsibility to pay for a portion of the costs of non-normal tenant improvements such as finish work, mechanical, electrical and plumbing elements of the building, among other items. As of December 31, 2018, under the requirements of Topic 840, the Company was deemed the owner of the leased premises during the construction period for accounting purposes and certain estimated construction costs incurred and reported by the landlord were recorded as property and equipment, with a corresponding financing lease obligation, on the consolidated balance sheet. The Company has determined that it does not control the building during the construction period under the requirements of Topic 842. Accordingly, upon the adoption of Topic 842 on January 1, 2019, the Company derecognized property and equipment of $5.9 million for the cumulative costs of construction incurred by the landlord as well as the associated $5.9 million financing lease obligation. As of September 30, 2019, the Company had recorded $5.4 million of costs related to construction-in-progress at 9800 Medical Center Drive, which have been recorded as leasehold improvements within property and equipment on the consolidated balance sheets.

As of September 30, 2019, the right-of-use assets and lease liabilities related to the 9800 Medical Center Drive Lease have not been recorded on the Company's consolidated balance sheets and will be measured and recognized on the commencement date of the lease, which is expected to occur in 2020 when the landlord delivers the newly constructed building to the Company.

In November 2019, the Company amended the 9800 Medical Center Drive Lease to expand the leased premises to include the entire rentable square footage of the building (the November 2019 Amendment). The November 2019 Amendment increased the leased square footage by approximately 38,000 square feet, expanding the total leased premises from approximately 139,000 square feet to approximately 177,000 square feet. Total additional lease payments related to the November 2019 Amendment are estimated to be $22.6 million over the lease term, which are subject to adjustment based on the actual square footage of the facility once constructed. Additionally, the November 2019 Amendment resulted in an increase in the Company's tenant improvement allowance from $15.3 million to $19.5 million.

Other Leases

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 has options to extend the term of the 9712 Medical Center Drive Lease for up to six additional years. Additionally, upon the commencement of the 9800 Medical Center Drive Lease, the Company will have 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 have been excluded from the measurement of the right-of-use assets and lease liabilities for the lease as they are 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 rent expense over the term of the lease.

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 has an option to extend the term of the Blackwell Road Lease for up to five additional years and the option to terminate the lease after 67 months from the lease commencement date. If the Company elects to terminate the lease, it will be subject to a termination fee equal to the unamortized tenant improvement allowance, rent abatement and landlord costs and commissions as of the termination date, bearing interest at 8% per annum. The Company's extension and termination options under the Blackwell Road Lease have been excluded from the measurement of the right-of-use assets and lease liabilities for the lease as they are not reasonably certain of exercise. 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 rent expense over the term of the lease.

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. 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.

In May 2019, the 400 Madison Lease was amended to include additional office space and extend the term of the lease. Prior to the amendment, the lease was to expire in October 2020. Pursuant to the amendment, the Company vacated the original leased premises during the third quarter of 2019, upon which the landlord began the demolition of the original premises as well as the additional premises under the amended lease. Upon completion of the demolition, the landlord will deliver the entire expanded premises to the Company to make tenant improvements. The amended lease will expire approximately 7.5 years from the date the expanded premises are delivered to the Company, which occurred in October 2019. The Company will receive a $0.7 million tenant improvement allowance from the landlord to construct improvements to the leased premises.

As a result of the amendment in May 2019, the expiration of the lease term for the original premises under the 400 Madison Lease was adjusted from October 2020 to August 2019. Accordingly, upon the execution of the amendment in May 2019, the right-of-use assets and lease liabilities for the original premises were reduced by $0.4 million to account for the modification of the lease term. As of September 30, 2019, the Company did not have control of the original or additional leased premises under the amended lease and no right-of-use assets and lease liabilities under the 400 Madison Lease were recorded on the Company's consolidated balance sheets. The right-of-use assets and lease liabilities related to the expanded premises for the extended lease term will be measured and

recognized upon the delivery of the expanded premises to the Company to make tenant improvements, which occurred in October 2019.

The Company leases additional office and laboratory facilities in Rockville, Maryland, as well as laboratory and other equipment, under operating leases with various expiration dates through 2022.

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, 2019	Nine Months Ended September 30, 2019
Operating lease cost	$ 690	$ 2,088
Variable lease cost	164	463
Total lease cost	$ 854	$ 2,551
Cash paid for amounts included in operating lease liabilities	$ 757	$ 2,152
Right-of-use assets acquired through operating lease liabilities	$ 557	$ 222

Right-of-use assets acquired through operating lease liabilities for the nine months ended September 30, 2019 includes a reduction of $0.4 million related to the May 2019 amendment to the 400 Madison Lease for the adjustment of the lease term. Short-term lease expense for the three and nine months ended September 30, 2019 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, 2019
Weighted-average remaining lease term (years)	2.7
Weighted-average discount rate	5.5%

The following table presents a reconciliation of the undiscounted future minimum lease payments remaining under leases that have not yet commenced and other operating leases to the amounts reported as operating lease liabilities on the consolidated balance sheet as of September 30, 2019 (in thousands):

	Leases Not Yet Commenced (a)	Other Operating Leases	Total Minimum Lease Payments
Undiscounted future minimum lease payments:			
2019 (remainder of year)	$ 73	$ 521	$ 594
2020	413	3,057	3,470
2021	2,306	2,662	4,968
2022	5,420	631	6,051
2023	6,369	479	6,848
Thereafter	83,731	—	83,731
Total undiscounted future minimum lease payments	$ 98,312	$ 7,350	$ 105,662
Amount representing imputed interest		(524)	
Total operating lease liabilities		6,826	
Current portion of operating lease liabilities		(2,506)	
Operating lease liabilities, non-current		$ 4,320	

(a) Includes undiscounted future minimum lease payments under the 9800 Medical Center Drive Lease and 400 Madison Lease which are not included in the lease liabilities reported on the consolidated balance sheet as of September 30, 2019. The actual

timing and amounts of these payments are subject to adjustment based on the commencement dates and actual square footage of the leased premises. Accordingly, these amounts were estimates as of September 30, 2019.

As of December 31, 2018, future minimum lease payments under Topic 840 for the 9800 Medical Center Drive Lease and other operating leases were as follows (in thousands):

	9800 Medical Center Drive Lease (a)	Other Operating Leases	Total Minimum Lease Payments
2019	$ —	$ 2,798	$ 2,798
2020	—	3,054	3,054
2021	1,329	2,391	3,720
2022	4,289	621	4,910
2023	5,156	479	5,635
Thereafter	76,420	—	76,420
Total minimum lease payments	$ 87,194	$ 9,343	$ 96,537

(a) Includes all future minimum lease payments under the 9800 Medical Center Drive Lease, including amounts recorded as financing lease obligations on the consolidated balance sheet. The actual timing and amounts of payments under the 9800 Medical Center Drive Lease are subject to adjustment based on the commencement date and actual square footage of the leased premises. Accordingly, these amounts were estimates as of December 31, 2018.

7. Commitments and Contingencies

Licenses and Collaborations

The Trustees of the University of Pennsylvania

In February 2009, the Company entered into a license agreement, which has been amended from time to time, with The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn) for exclusive, worldwide rights to certain patents owned by Penn underlying the Company's NAV Technology Platform, as well as exclusive rights to certain data, results and other information generated in connection with the clinical trial for RGX-501, the Company's product candidate for the treatment of homozygous familial hypercholesterolemia (HoFH). Pursuant to the license agreement, the Company and is obligated to pay Penn royalties on net sales of licensed products and sublicense fees. Additionally, the Company is obligated to reimburse Penn for certain costs incurred related to the maintenance of the licensed patents.

In April 2019, the Company amended its license from Penn to include exclusive license rights to certain know-how, including research data and other information, relating to the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease. In consideration for the additional licensed rights, and in addition to any consideration owed under the license prior to the amendment, the Company paid Penn an up-front fee and is obligated to pay milestone fees of up to $20.5 million upon the achievement of various development and sales-based milestones and additional royalties on net sales of licensed products for the treatment of CLN2 disease. Additionally, the amendment modifies the percentage of sublicense fees the Company is obligated to pay Penn on amounts the Company receives from third parties for the sublicensing of the licensed rights for the treatment of CLN2 disease.

Neurimmune AG

In July 2019, the Company entered into a collaboration and license agreement with Neurimmune AG (Neurimmune) pursuant to which the Company and Neurimmune will jointly develop and commercialize novel gene therapies using AAV vectors from the NAV Technology Platform to deliver human antibodies for chronic neurodegenerative diseases. The Company and Neurimmune will share all research and development costs for the first two years of the agreement, after which each party will have the option, on a target-by-target basis, to: (i) continue as a 50% partner in the collaboration; (ii) receive a phase-based worldwide royalty in lieu of continued development investment; or (iii) negotiate with the other party to lead the development and commercialization of the respective program. Unless the parties agree otherwise, upon the commercialization of any product candidates, if any, it is anticipated that profits and losses will be shared equally on a worldwide basis.

The Company evaluated the collaboration and license agreement with Neurimmune and determined that it is a collaborative arrangement within the scope of Topic 808, and that no unit of account under the arrangement should be accounted for as a transaction with a customer within the scope of Topic 606. In accordance with the Company's accounting policies for collaborative arrangements, if Neurimmune's development costs incurred under the collaboration exceed those incurred by the Company during a reporting period,

the Company will recognize research and development expense and record a liability for the amount due to Neurimmune at the end of the period. Alternatively, if the Company's development costs incurred under the collaboration exceed those incurred by the Neurimmune during a reporting period, the Company will recognize a reduction of research and development expenses and record an amount due from Neurimmune at the end of the period. During the three and nine months ended September 30, 2019, the Company recognized net research and development expenses of less than $0.1 million under the collaboration and license agreement with Neurimmune.

Clearside Biomedical, Inc.

In August 2019, the Company entered into an option and license agreement with Clearside Biomedical, Inc. (Clearside) pursuant to which the Company was granted an option to exclusively license the worldwide rights to certain patents related to Clearside's proprietary, in-office SCS Microinjector™ for the delivery of RGX-314 to the suprachoroidal space to treat wet age-related macular degeneration (wet AMD), diabetic neuropathy (DR) and other diseases. The Company exercised its license option in October 2019, resulting in a payment of $1.6 million to Clearside upon exercise. Additionally, the Company is obligated to pay milestone fees of up to $136.0 million upon the achievement of various development and sales-based milestones, as well as royalties on net sales of licensed products using the SCS Microinjector. Clearside is responsible for supplying the SCS Microinjector to the Company to support all preclinical, clinical and commercial needs.

European Patent Office Proceeding

In June 2017, a third party filed an opposition with the European Patent Office (EPO) challenging the validity of a European patent owned by Penn for the AAV8 vector, which the Company has exclusively licensed (EU AAV8 Patent). The EPO conducted oral proceedings in October 2018 and upheld the validity of the EU AAV8 Patent subject to certain amendments made during the proceeding. Each party to the proceeding has appealed the EPO's ruling. As of September 30, 2019 and December 31, 2018, the Company had not recorded any liabilities related to this matter nor does the Company believe this matter will have a material adverse impact on its business.

8. License and Royalty Revenue

As of September 30, 2019, 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 only included in the transaction price of each license and recognized as license revenue to the extent they 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, 2019, 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 $20.1 million upon the commencement of various stages of clinical trials, $31.0 million upon the submission of regulatory approval filings, $96.0 million upon the approval of commercial products by regulatory agencies and $207.0 million upon the achievement of specified sales targets for licensed products. To the extent the milestone payments are realized by the Company, the Company will be obligated to pay sublicense fees to licensors based on a specified percentage of the fees earned by the Company. The achievement of milestones by licensees is highly dependent on the successful development and commercialization of licensed products and it is at least reasonably possible that some or all of the milestone fees will not be realized by the Company.

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

	Balance at Beginning of Period	Net Additions (Deductions)	Balance at End of Period
Three Months Ended September 30, 2019			
Receivables and contract assets:			
Accounts receivable, current and non-current	$ 33,634	$ 9,886	$ 43,520
Contract assets	$ —	$ —	$ —
Contract liabilities:			
Deferred revenue, current and non-current	$ 3,333	$ —	$ 3,333

	Balance at Beginning of Period	Net Additions (Deductions)	Balance at End of Period
Nine Months Ended September 30, 2019			
Receivables and contract assets:			
Accounts receivable, current and non-current	$ 31,599	$ 11,921	$ 43,520
Contract assets	$ 750	$ (750)	$ —
Contract liabilities:			
Deferred revenue, current and non-current	$ 3,933	$ (600)	$ 3,333

	Balance at Beginning of Period	Net Additions (Deductions)	Balance at End of Period
Three Months Ended September 30, 2018			
Receivables and contract assets:			
Accounts receivable, current and non-current	$ 5,224	$ 2,002	$ 7,226
Contract assets	$ —	$ 2,000	$ 2,000
Contract liabilities:			
Deferred revenue, current and non-current	$ 600	$ —	$ 600

	Balance at Beginning of Period	Net Additions (Deductions)	Balance at End of Period
Nine Months Ended September 30, 2018			
Receivables and contract assets:			
Accounts receivable, current and non-current	$ 5,850	$ 1,376	$ 7,226
Contract assets	$ 350	$ 1,650	$ 2,000
Contract liabilities:			
Deferred revenue, current and non-current	$ —	$ 600	$ 600

The net increases in accounts receivable during the three and nine months ended September 30, 2019 were primarily attributable to royalties on net sales of Zolgensma, which commenced in the second quarter of 2019. During the three and nine months ended September 2019, the Company recognized royalty revenue of $9.2 million and $10.1 million, respectively, related to net sales of Zolgensma. As of September 30, 2019, the Company had recorded accounts receivable of $9.2 million related to Zolgensma royalties.

As of September 30, 2019, the Company had recorded deferred revenue, current and non-current, of $3.3 million which represents consideration received from licensees for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations consist of options granted to licensees that provide material rights to the licensee to acquire additional licenses from the Company. These performance obligations will be satisfied, and underlying revenue will be recognized, upon the exercise or expiration of the options. During the three and nine months ended September 30, 2019, the Company recognized zero million and $0.6 million, respectively, of license revenue that was included in deferred revenue at the beginning of the period as a result of options exercised by licensees during the period. The Company did not recognize any license revenue during the three and nine months ended September 30, 2018 that was included in deferred revenue at the beginning of the period.

During the three and nine months ended September 30, 2019, the Company recognized license revenue of $0.9 million and $4.6 million, respectively, from licenses delivered to licensees in prior periods as a result of changes in the transaction prices of its license agreements. Changes in the transaction prices during the three and nine months ended September 30, 2019 were primarily attributable to development milestones achieved or deemed probable of achievement during the period that were previously not considered probable of achievement. During the three and nine months ended September 30, 2018, the Company recognized license revenue of $4.1 million and $4.2 million, respectively, from licenses delivered to licensees in prior periods as a result of changes in the transaction prices of its license agreements. Changes in the transaction prices during the three and nine months ended September 30, 2018 were primarily attributable to development milestones achieved or deemed probable of achievement during the period that were previously not considered probable of achievement. Changes in the transaction price exclude revenue recognized from sublicense fees and royalties on sales of licensed products, which are excluded from the transaction price and recognized as revenue in the period that the underlying sales or sublicenses occur.

As of September 30, 2019, the Company had recorded total current and non-current accounts receivable of $43.5 million, of which $1.2 million had been billed to customers and $42.4 million was billable to customers in future periods. As of December 31, 2018, the Company had recorded total current and non-current accounts receivable of $31.6 million, of which $0.4 million had been billed to customers and $31.2 million was billable to customers in future periods.

Accounts receivable, current and non-current, as of September 30, 2019 and December 31, 2018 included $28.0 million and $26.0 million, respectively, related to the November 2018 license agreement with Abeona Therapeutics Inc. for the development and commercialization of treatments for various diseases. The Company believes that it is not exposed to significant credit risk related to accounts receivable due to the credit quality and history of collections from its significant customers. The Company is unaware of any concentrations of credit risk related to accounts receivable from significant customers with deteriorated credit quality. As of September 30, 2019 and December 31, 2018, the Company had not recognized any impairment losses on its receivables or contract assets from contracts with customers and no allowance for doubtful accounts was recorded.

AveXis March 2014 License and January 2018 Amendment

In March 2014, the Company entered into an exclusive license agreement (the March 2014 License) with AveXis. Under the license, the Company granted AveXis an exclusive, worldwide commercial license, with rights to sublicense, to the NAV AAV9 vector for the treatment of SMA in humans by *in vivo* gene therapy. In consideration for the license, AveXis paid the Company an up-front fee of $2.0 million, and is required to pay annual fees, development milestone payments of up to $12.3 million, mid-single to low double-digit royalties on net sales of licensed products, subject to reduction in specified circumstances, and a lower mid-double digit percentage of any sublicense fees AveXis receives from sublicensees for the licensed intellectual property rights.

In January 2018, the Company and AveXis amended the March 2014 License (the January 2018 Amendment). Under the January 2018 Amendment, the licensed intellectual property was expanded to include, in addition to the NAV AAV9 vector previously licensed, sublicenses to other third-party patents exclusively licensed by the Company as well as any other recombinant AAV vector in the Company's intellectual property portfolio during a period of 14 years from the effective date of the January 2018 Amendment, for the treatment of SMA in humans by *in vivo* gene therapy. The Company may also, in its sole discretion, provide specified collaborative services to AveXis as specified in the January 2018 Amendment.

The January 2018 Amendment also modified the assignment provision of the March 2014 License. Under the amended assignment provision, AveXis was permitted to transfer the March 2014 License, as amended, without the Company's consent in connection with a change of control of AveXis, subject to certain conditions. Under the original March 2014 License, any assignment by AveXis without the Company's prior written consent had been prohibited.

In consideration for the additional rights granted under the January 2018 Amendment, and in addition to any consideration owed under the original March 2014 License, AveXis paid to the Company a fee of $80.0 million upon entry into the January 2018 Amendment. In addition, AveXis was obligated to pay the Company (i) $30.0 million on the first anniversary of the effective date of the January 2018 Amendment, (ii) $30.0 million on the second anniversary of the effective date of the January 2018 Amendment and (iii) potential sales-based milestone payments of up to $120.0 million. In the event of a change of control of AveXis, to the extent that any fee described in (i) or (ii) above, or the first $40.0 million of sales-based milestone payments described in (iii) above, had not yet been paid to the Company, AveXis was required to pay any such unpaid fee to the Company upon the change of control. For any product developed for the treatment of SMA using the NAV AAV9 vector, AveXis will continue to be obligated to pay to the Company mid-single to low double-digit royalties on net sales as required by the March 2014 License, and for any product developed for the treatment of SMA using a licensed vector other than NAV AAV9, the Company will receive a low double-digit royalty on net sales.

In May 2018, AveXis was acquired by Novartis, which qualified as a change of control of AveXis under the January 2018 Amendment. Pursuant to the January 2018 Amendment, AveXis paid the Company $100.0 million in accelerated license payments as a result of the change of control.

In May 2019, the U.S. Food and Drug Administration (the FDA) approved Zolgensma for marketing in the United States, which is a licensed product under the March 2014 License, as amended, with AveXis. Upon its commercial launch in the second quarter of 2019, the Company began recognizing royalty revenue on net sales of Zolgensma.

Accounting Analysis

The January 2018 Amendment was accounted for under Topic 606 as a modification of the license agreement resulting in a new and separate contract from the original March 2014 License for revenue recognition purposes. The Company determined that a substantive termination penalty is associated with AveXis' termination rights under the amended license agreement, and therefore the contract term for revenue recognition purposes is equal to the stated term of the license. The only material performance obligation of the Company under the January 2018 Amendment is for the delivery of the modified license, which occurred upon the execution of the amendment in January 2018.

As of September 30, 2019, the transaction price of the original March 2014 License was $11.0 million. The transaction price includes (i) the up-front payment in March 2014 of $2.0 million, (ii) the present value of aggregate annual fees payable to the Company over the term of the license and (iii) payments for development milestones achieved to date or which are deemed probable of achievement. The discounted portion of the annual fees represents the financing benefit provided to AveXis and is recognized as interest income from licensing over the term of the license. Variable consideration under the original March 2014 License, which has been excluded from the transaction price, includes $3.5 million in payments for remaining development milestones that had not yet been achieved and were not considered probable of achievement, as well as any potential sublicense fees or royalties on sales of licensed products, which will be recognized in the period of the underlying sales or sublicenses. The transaction price of the original March 2014 License increased by $3.5 million during the nine months ended September 30, 2019 as a result of development milestones achieved during the period which were previously excluded from the transaction price.

Upon its execution, the transaction price of the January 2018 Amendment was $132.1 million, which was fully recognized as license revenue upon the delivery of the modified license in January 2018. In May 2018, as a result of the acquisition of AveXis by Novartis, the transaction price was increased by $40.0 million to account for the acceleration of the sale-based milestone which was previously excluded from the transaction price. The $40.0 million increase in the transaction price was recognized as license revenue upon the completion of the change of control in May 2018 since the amended license had been fully delivered to AveXis. Additionally, due to the acceleration of the two $30.0 million payments originally due in January 2019 and January 2020, the Company recognized $6.1 million of interest income from licensing upon the completion of the change of control of AveXis, which represents the remaining present value discount on such payments as of the date of the change of control. As of September 30, 2019, the transaction price of the January 2018 Amendment was $172.1 million, which includes: (i) the $80.0 million payment in January 2018, (ii) the present value, as of the date of the January 2018 Amendment, of the two $30.0 million payments originally due in January 2019 and January 2020 and (iii) the $40.0 million sales-based milestone which was accelerated upon the change of control in May 2018. Variable consideration under the January 2018 Amendment, which has been excluded from the transaction price, includes

the remaining sales-based milestone payment of $80.0 million, as well as any potential sublicense fees or royalties on sales of licensed products, which will be recognized in the period of the underlying sales or sublicenses. There were no increases in the transaction price of the January 2018 Amendment during the nine months ended September 30, 2019.

The Company recognized the following amounts under the March 2014 License with AveXis, as amended, which include amounts from both the original March 2014 License and the January 2018 Amendment (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
License revenue	$ —	$ 4,000	$ 3,500	$ 176,066
Zolgensma royalty revenue	9,182	—	10,106	—
Total license and royalty revenue	$ 9,182	$ 4,000	$ 13,606	$ 176,066
Interest income from licensing	$ 7	$ 8	$ 22	$ 7,958

As of September 30, 2019, the Company had recorded $9.4 million of accounts receivable from AveXis under the March 2014 License, as amended, of which $9.2 million were included in current assets and $0.2 million were included in non-current assets. As of December 31, 2018, the Company had recorded $0.2 million of accounts receivable from AveXis under the March 2014 License, as amended, of which less than $0.1 million were included in current assets and $0.2 million were included in non-current assets.

9. Stock-based Compensation

In January 2019, an additional 1,444,808 shares became available for issuance under the 2015 Equity Incentive Plan (the 2015 Plan). As of September 30, 2019, the total number of shares of common stock authorized for issuance under the 2015 Plan and the 2014 Stock Plan (the 2014 Plan) was 10,933,221, of which 2,189,562 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,	
	2019	2018	2019	2018
Stock options	$ 6,965	$ 4,309	$ 19,261	$ 11,248
Restricted stock units	69	69	206	206
Employee stock purchase plan	128	105	512	301
	$ 7,162	$ 4,483	$ 19,979	$ 11,755

As of September 30, 2019, the Company had $67.7 million of unrecognized stock-based compensation expense related to stock options, restricted stock units and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 2.8 years.

The Company 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,	
	2019	2018	2019	2018
Research and development	$ 3,657	$ 1,940	$ 9,530	$ 5,364
General and administrative	3,505	2,543	10,449	6,391
	$ 7,162	$ 4,483	$ 19,979	$ 11,755

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, 2018	4,855	$ 19.31	7.6	$ 118,185
Granted	1,596	$ 47.82		
Exercised	(684)	$ 8.07		
Cancelled or forfeited	(237)	$ 33.74		
Outstanding at September 30, 2019	5,530	$ 28.31	7.7	$ 69,769
Exercisable at September 30, 2019	2,827	$ 15.48	6.6	$ 60,340
Vested and expected to vest at September 30, 2019	5,530	$ 28.31	7.7	$ 69,769

(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, 2019 was $31.84. During the nine months ended September 30, 2019, the total number of stock options exercised was 684,218, resulting in total proceeds of $5.5 million. The total intrinsic value of options exercised during the nine months ended September 30, 2019 was $27.8 million.

Restricted Stock Units

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

	Shares	Weighted-average Grant Date Fair Value
Unvested balance at December 31, 2018	40	$ 20.90
Granted	—	$ —
Vested	—	$ —
Forfeited	—	$ —
Unvested balance at September 30, 2019	40	$ 20.90

Employee Stock Purchase Plan

As of September 30, 2019, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 254,000, of which 133,586 remained available for future issuance. During the nine months ended September 30, 2019, 35,994 shares of common stock were issued under the 2015 ESPP.

10. Income Taxes

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, including a three-year cumulative loss position as of September 30, 2019 and December 31, 2018, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for its net deferred tax assets as of September 30, 2019 and December 31, 2018.

During the three and nine months ended September 30, 2019, the Company recognized income tax benefit of $0.2 million and $0.4 million, respectively, and income tax benefit (expense) in other comprehensive income (loss) of less than $0.1 million and $(0.6) million, respectively, related to net unrealized gains on available-for-sale debt securities. As of September 30, 2019, the Company had accrued $0.3 million related to this tax benefit, which is expected to be generated from losses in continuing operations in 2019 and is included in accrued expenses and other current liabilities on the consolidated balance sheet.

11. Related Party Transactions

FOXKISER LLP

Since 2016, the Company has been party to professional services agreements with FOXKISER LLP (FOXKISER), an affiliate of certain stockholders of the Company and an affiliate of a member of the Company's Board of Directors, pursuant to which the Company pays a fixed monthly fee in consideration for certain strategic services provided by FOXKISER. Effective January 2019, the Company entered into a new professional services agreement with FOXKISER with similar terms and conditions as the previous agreements. The agreement was amended effective June 2019 to expand the services provided and increase the monthly fee, and the amended agreement expires in December 2020. Expenses incurred under the agreements with FOXKISER for the three and nine months ended September 30, 2019 were $1.2 million and $2.9 million, respectively. Expenses incurred under the agreements with FOXKISER for the three and nine months ended September 30, 2018 were $0.5 million and $1.6 million, respectively. Expenses incurred under the agreements with FOXKISER are recorded as research and development expenses in the consolidated statements of operations and comprehensive income (loss).

12. Net Income (Loss) Per Share

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Basic net income (loss) per share:				
Net income (loss) applicable to common stockholders	$ (34,584)	$ (19,202)	$ (68,269)	$ 95,631
Shares used in computation:				
Weighted-average common shares outstanding	36,813	33,988	36,618	32,576
Basic net income (loss) per share	$ (0.94)	$ (0.56)	$ (1.86)	$ 2.94
Diluted net income (loss) per share:				
Net income (loss) applicable to common stockholders	$ (34,584)	$ (19,202)	$ (68,269)	$ 95,631
Shares used in computation:				
Weighted-average common shares outstanding	36,813	33,988	36,618	32,576
Stock options	—	—	—	3,268
Restricted stock units	—	—	—	31
Weighted-average diluted common shares	36,813	33,988	36,618	35,875
Diluted net income (loss) per share	$ (0.94)	$ (0.56)	$ (1.86)	$ 2.67

For periods in which the Company incurred net losses applicable to common stockholders, common stock equivalents are excluded from the calculation of diluted net loss per share as their effect would be anti-dilutive, and accordingly, basic and diluted net loss per share are the same for such periods. Outstanding stock options with exercise prices greater than the average market price of common stock are excluded from the calculation of diluted net income (loss) per share as their effect would be anti-dilutive. 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,	
	2019	2018	2019	2018
Stock options issued and outstanding	5,530	5,171	5,530	1,128
Unvested restricted stock units outstanding	40	40	40	—
Employee stock purchase plan	16	4	16	4
	5,586	5,215	5,586	1,132

13. Supplemental Disclosures

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

	September 30, 2019	December 31, 2018
Accrued personnel costs	$ 8,869	$ 9,484
Accrued external research and development expenses	4,390	4,274
Accrued sublicense fees and royalties	3,245	1,617
Accrued external general and administrative expenses	1,137	773
Accrued income taxes payable	508	726
Accrued purchases of property and equipment	349	221
Other accrued expenses and current liabilities	733	69
	$ 19,231	$ 17,164

Other liabilities of $1.8 million and $2.5 million reported as of September 30, 2019 and December 31, 2018, respectively, consist of accrued sublicense fees payable to licensors in periods beyond 12 months from the reporting date.

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, 2018, which we filed with the SEC on February 27, 2019. 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, 2018 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 an internal pipeline of product candidates, including ophthalmologic, central nervous system (CNS) and liver-directed therapies. Our therapeutic programs are comprised of both adeno-associated virus (AAV)-mediated antibodies for chronic diseases and gene therapy candidates for rare monogenic diseases.

AAV-mediated antibody programs for the potential treatment of chronic diseases

- **RGX-314:** We are developing RGX-314 for the treatment of wet age-related macular degeneration (wet AMD), a leading cause of blindness in the United States, Europe and Japan. On May 30, 2019, we announced completion of dosing in the Phase I/IIa clinical trial for RGX-314 for the treatment of wet AMD. Eight leading retinal surgery centers across the United States participated in the trial, designed to evaluate the safety and tolerability of RGX-314 in 42 dosed subjects across five escalating dose cohorts. Each subject received a single dose of RGX-314 administered by subretinal delivery.

  We have been informed by the U.S. Food and Drug Administration (the FDA) that they are assessing aspects of the surgical delivery system for RGX-314, specifically the use of certain third-party commercially available devices that are used to deliver RGX-314 in our Phase I/IIa trial for the treatment of wet AMD. While discussions with the FDA are ongoing, the investigational new drug (IND) application for the trial has been placed on partial clinical hold, which is not related to the gene therapy product itself. As a result, we expect to initiate a Phase IIb trial in subjects with wet AMD and file an IND application with the FDA for a Phase II trial to evaluate RGX-314 for the treatment of diabetic retinopathy in the first quarter of 2020.

- **AAV-Mediated Antibody Expression for the Treatment of Tauopathies:** Effective in July 2019, we entered into an exclusive license, development and commercialization agreement with Neurimmune AG (Neurimmune) to jointly develop and commercialize novel gene therapies using NAV Vectors to deliver human antibodies for chronic neurodegenerative diseases, with an initial focus on diseases associated with the accumulation and deposition of the microtubule-associated protein tau (tauopathies). The companies have initiated their first exclusive collaboration program for the treatment of tauopathies.

- **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.

Gene therapy programs for the potential treatment of rare monogenic diseases

- **RGX-501:** We are developing RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH), a severe genetic disease characterized by premature and aggressive plaque buildup, life threatening coronary artery disease and aortic valve disease predominantly due to abnormalities in the function or expression of the low-density lipoprotein receptor. We have completed dosing of patients in the second cohort of the Phase I/II clinical trial for RGX-501 with corticosteroid prophylaxis.

- **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. Subject enrollment has been completed in the first of two dose cohorts in the Phase I/II clinical trial for RGX-121.
- **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, screening and additional site activations are ongoing in the Phase I clinical trial for RGX-111. In October 2019, RGX-111 was administered to a subject with MPS I through an investigator-initiated study.
- **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 plan to file an IND application, or foreign equivalent, for RGX-181 for the treatment of CLN2 to the FDA, or a foreign regulatory authority, in the second half of 2020 to enable initiation of a first-in-human clinical trial.

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

RGX-314 Update

In our Phase I/IIa trial for RGX-314, as of October 9, 2019, all 42 subjects with wet AMD have received a single administration of RGX-314 across five dose cohorts. To qualify for inclusion in the trial, participants were required to have a history of frequent anti-vascular endothelial growth factor (VEGF) treatments (including at least four anti-VEGF injections in the eight months preceding trial enrollment) and a documented history of response to anti-VEGF therapy. The trial design included doses of 3×10^9 (Cohort 1), 1×10^{10} (Cohort 2), 6×10^{10} (Cohort 3), 1.6×10^{11} (Cohort 4) and 2.5×10^{11} (Cohort 5) genome copies (GC)/eye. Subjects will be assessed every four weeks to the six-month primary endpoint, with long-term follow-up continuing for two years. Below is a summary of the preliminary results of our Phase I/IIa trial as of October 9, 2019:

- RGX-314 continues to be well-tolerated across all five dose cohorts, with no drug related serious adverse events (SAEs) reported.
- Dose-dependent increases in RGX-314 protein expression levels, as measured from aqueous samples by electrochemiluminescence immunoassay (ECL) at approximately one month after administration of RGX-314, have been observed across all doses.
- Up to six months after administration of RGX-314, subjects in Cohort 5 demonstrated a reduction of over 80% from the mean annualized anti-VEGF injection rate during the 12 months prior to administration of RGX-314, and 75% of subjects in Cohort 5 remained free of anti-VEGF injections, with mean improvement in vision and retinal thickness.
- Durable effects on vision and retinal thickness have been demonstrated over 1.5 years in Cohort 3, and 50% of subjects remain free of anti-VEGF injections more than 1.5 years after RGX-314 administration.

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively license our proprietary adeno-associated virus (AAV) gene therapy delivery platform (NAV Technology Platform) to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of September 30, 2019, our NAV Technology Platform was being applied in the one FDA approved product, and the clinical development of 15 partnered product candidates, with over 20 partnered programs in total. Licensing the NAV Technology Platform allows us to maintain our internal product development focus on our core disease indications and therapeutic areas while still expanding the NAV gene therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform, and creating potential additional revenue.

Financial Overview

Revenues

Our revenues to date 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 fail to obtain their 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 use in children less than two years old with spinal muscular atrophy (SMA). Zolgensma is a licensed product under our March 2014 license agreement, as amended, 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 Phase IIb clinical trial to evaluate the safety and efficacy of our RGX-314 program for the treatment of wet AMD, and a planned Phase II clinical trial to evaluate the safety and efficacy of our RGX-314 program for the treatment of diabetic retinopathy;
- a Phase I/II clinical trial to evaluate the safety and efficacy of our RGX-501 program for the treatment of HoFH;
- a Phase I/II clinical trial to evaluate the safety and efficacy of our RGX-121 program for the treatment of MPS II;
- a Phase I clinical trial to evaluate the safety and efficacy of our RGX-111 program for the treatment of MPS I;
- preclinical research and development for our RGX-314 program with the SCS Microinjector™ licensed from Clearside Biomedical, Inc. for the treatment of wet AMD, diabetic retinopathy and other conditions for which anti-VEGF treatment is currently the standard of care;
- preclinical research and development and a planned clinical trial for our RGX-181 program for the treatment of CLN2;
- preclinical research and development for potential product candidates to treat neurodegenerative diseases, including tauopathies, under our collaboration with Neurimmune;
- preclinical research and development for potential product candidates to treat HAE;
- preclinical research and development for additional product candidates addressing other diseases in the ophthalmologic, CNS and liver-directed therapeutic 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, 2019 and 2018 (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Direct Expenses				
RGX-314	$ 9,170	$ 1,451	$ 15,156	$ 4,599
RGX-501	1,325	1,065	3,390	10,103
RGX-121	775	829	2,848	2,620
RGX-111	645	209	2,253	2,361
RGX-181	1,448	2,103	6,751	2,103
Total direct expenses	13,363	5,657	30,398	21,786
Unallocated Expenses				
Unallocated external expenses	5,631	2,908	14,486	8,561
Personnel-related	13,345	7,929	36,159	23,709
Facilities and depreciation expense	2,422	1,386	6,918	3,947
Other unallocated	931	628	2,417	1,541
Total unallocated expenses	22,329	12,851	59,980	37,758
Total research and development	$ 35,692	$ 18,508	$ 90,378	$ 59,544

Expenses incurred in the development of RGX-181 were included in unallocated external expenses through the second quarter of 2018. Unallocated external expenses 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 preclinical research and development for prospective product candidates and new technologies. We typically utilize our employee and infrastructure resources across our development programs. We do not allocate personnel and other internal costs, such as facilities and other overhead costs, to specific product candidates or development programs.

General and Administrative Expense

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

Other Income

Interest Income from Licensing

In accordance with our revenue recognition policy, interest income from licensing consists of imputed interest recognized from significant financing components identified in our license agreements with NAV Technology Licensees.

Investment Income

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

Critical Accounting Policies and Significant Judgments and Estimates

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

Our significant accounting policies and recently announced accounting pronouncements, including the expected impact of such pronouncements, 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, 2018. Other than the critical accounting policies discussed below, there have been no significant changes in our critical accounting policies since December 31, 2018.

Revenue Recognition

We recognize revenue in accordance with Accounting Standards Codification (ASC) 606, *Revenue from Contracts with Customers* (Topic 606). Topic 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of Topic 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

We apply the five-step model to contracts that are within the scope of Topic 606 only when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, for contracts within the scope of Topic 606, we assess the goods or services promised within each contract and determine those that are

performance obligations and whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

We evaluate our contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, we evaluate the credit profile of the customer and prevailing market interest rates and select an interest rate in which we believe would be charged to the customer in a separate financing arrangement over a similar financing term.

License and Royalty Revenue

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 our 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 payable to us 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.

Our license agreements are accounted for as contracts with customers within the scope of Topic 606. At the inception of each license agreement, we determine the contract term for purposes of applying the requirements of Topic 606. Licenses are generally terminable at the option of the licensee with advance notice to us. For each license, we evaluate these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options. The determination of whether a substantive termination penalty is associated with the termination rights requires significant judgment. In making this determination, we consider, among other things, the nature of the intellectual property rights that would be returned to us upon termination, including the exclusivity of the licensed rights and the stage of development of the licensed products, the payment terms, including the amount and timing of non-refundable or guaranteed payments, and the business purpose of the termination rights granted to the licensee. Generally, the most significant judgment in determining whether a substantive termination penalty exists is the related to amount of any up-front or guaranteed non-refundable payments relative to the amount of annual payments that may be avoided by the licensee upon termination of the license. We consider all of the facts and circumstances relevant to each license when making this determination.

Performance obligations under our license agreements may include (i) the delivery of intellectual property licenses and (ii) options granted to licensees to acquire additional licenses to the extent the options represent material rights to the licensee. At the inception of each license agreement which contains options for the licensee to acquire additional licenses, or contract renewal options, we evaluate the options to determine whether they provide material rights to the licensee. In making this determination, we consider whether the options are priced at a discount to the standalone selling price for the underlying licenses. If an option is priced at a discount to the standalone selling price for the underlying license, the option is considered to be a material right to the licensee and is accounted for as a separate performance obligation under the current license agreement.

We evaluate the transaction price of our license agreements at the inception of each agreement and at each reporting date. The transaction price includes the fixed consideration payable to us during the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the license agreements includes up-front and annual fees payable during the contract term. Variable consideration under the license agreements includes development and sales-based milestone payments, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by a licensee is excluded from the transaction price and not accounted for as part of the license agreement until the option is exercised.

The transaction price for each license agreement is allocated to the underlying performance obligations and recognized as revenue when the performance obligations are satisfied. Consideration allocated to performance obligations for the delivery of an intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. Consideration allocated to

performance obligations for license options is recognized as revenue in full upon the earlier of the option exercise or expiration. The exercise of a license option by a licensee is accounted for as a new license for revenue recognition purposes.

Up-front and annual licenses fees payable to us over the contract term of each license are included in the transaction price, and the portion of this consideration that is allocated to the performance obligation for the delivery of the intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. If annual license fees are payable to us in periods beyond 12 months from the delivery of the license, a significant financing component is deemed to exist which provides a financing benefit to the licensee. If a significant financing component is identified, we adjust the transaction price for the license to include only the present value of the annual license fees payable to us over the contract term. The discounted portion of the license fees is recognized as interest income from licensing over the financing period of the license.

Development milestone payments are payable to us upon the achievement of specified development milestones by licensees. At the inception of each license agreement that contains development milestone payments, we evaluate whether the milestones are considered probable of achievement and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price and recognized as revenue upon the delivery of the license. Milestone payments contingent on the achievement of development milestones that are not within our control or the control the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, we re-evaluate the probability of achievement of outstanding development milestones and, if necessary, adjust the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. Any such adjustments are recorded on a cumulative catch-up basis and recognized as revenue in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists of royalties on net sales of Zolgensma, which is marketed by AveXis, a wholly owned subsidiary of Novartis. Zolgensma is a licensed product under our March 2014 license agreement, as amended, with AveXis for the development and commercialization of treatments for SMA. We recognize royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by AveXis, which in certain cases may require us to estimate royalty revenue for periods of net sales which have not yet been reported to us. Sales-based milestone payments related to net sales of Zolgensma are recognized as royalty revenue in the period in which the milestone is achieved.

We receive payments from licensees based on the billing schedules established in each license agreement. Amounts recognized as revenue which have not yet been received from licensees, including unbilled royalties, are recorded as accounts receivable when our rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from licensees are recorded as contract assets when our rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to us and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of license revenue in the period of the termination. Amounts received by us prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

Recent Accounting Pronouncements

See Note 2 “Recent Accounting Pronouncements” in the notes to the accompanying unaudited consolidated financial statements for a full description of recently announced accounting pronouncements and the expected impact to our financial statements.

In February 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in ASC 840, *Leases* (Topic 840). Effective January 1, 2019, we adopted Topic 842 using the modified retrospective transition method. Under this method, we applied Topic 842 to all leases in effect as of, or entered into after, January 1, 2019 and recorded the cumulative impact of the adoption as an adjustment to our accumulated deficit on January 1, 2019. Our consolidated financial statements for periods ending after January 1, 2019 are presented in accordance with the requirements of Topic 842, while comparative prior period amounts have not been adjusted and continue to be reported in accordance with Topic 840.

Upon the adoption of Topic 842, we recorded operating lease right-of-use assets of $7.4 million and operating lease liabilities of $8.4 million for our leases which were in effect and had commenced prior to January 1, 2019 and had original lease terms of more than 12 months. We also derecognized current and non-current deferred rent liabilities of $1.4 million and prepaid expenses, other current assets and other assets of $0.4 million upon the adoption of Topic 842. Additionally, upon the adoption of Topic 842, we derecognized $5.9 million of property and equipment and $5.9 million of financing lease obligations related to construction-in-progress at 9800 Medical Center Drive, as we do not control the building during the construction period under the requirements of Topic 842. The cumulative impact of the adoption of Topic 842 resulted in an increase in accumulated deficit of less than $0.1 million on January 1, 2019. The adoption of Topic 842 did not have a material impact on our results of operations for the three and nine months ended September 30, 2019, nor do we believe it will have a material impact on future results of operations based on our current leasing arrangements.

Results of Operations

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2019	2018	Change	2019	2018	Change
	(in thousands)					
Revenues						
License and royalty revenue	$ 14,700	$ 5,306	$ 9,394	$ 23,465	$ 177,728	$ (154,263)
Total revenues	14,700	5,306	9,394	23,465	177,728	(154,263)
Operating Expenses						
Cost of revenues	2,494	517	1,977	4,450	6,797	(2,347)
Research and development	35,692	18,508	17,184	90,378	59,544	30,834
General and administrative	12,402	9,008	3,394	37,365	25,706	11,659
Other operating expenses (income)	8	(2)	10	(54)	31	(85)
Total operating expenses	50,596	28,031	22,565	132,139	92,078	40,061
Income (loss) from operations	(35,896)	(22,725)	(13,171)	(108,674)	85,650	(194,324)
Other Income						
Interest income from licensing	716	109	607	2,091	8,362	(6,271)
Investment income	431	2,122	(1,691)	37,950	4,177	33,773
Total other income	1,147	2,231	(1,084)	40,041	12,539	27,502
Income (loss) before income taxes	(34,749)	(20,494)	(14,255)	(68,633)	98,189	(166,822)
Income Tax Benefit (Expense)	165	1,292	(1,127)	364	(2,558)	2,922
Net income (loss)	$ (34,584)	$ (19,202)	$ (15,382)	$ (68,269)	$ 95,631	$ (163,900)

Comparison of the Three Months Ended September 30, 2019 and 2018

License and Royalty Revenue. License and royalty revenue increased by $9.4 million, from $5.3 million for the three months ended September 30, 2018 to $14.7 million for the three months ended September 30, 2019. The increase was primarily attributable to $9.2 million of royalty revenue recognized during the three months ended September 30, 2019 related to net sales of Zolgensma. Commercial sales of Zolgensma commenced in the second quarter of 2019. We are also eligible to receive a milestone payment of $80.0 million from AveXis upon the achievement of $1.0 billion in cumulative net sales of Zolgensma. The increase in license and royalty revenue during the three months ended September 30, 2019 resulted in a corresponding increase cost of revenues incurred during the period related to royalties that we are obligated to pay to our licensors.

Research and Development Expense. Research and development expenses increased by $17.2 million, from $18.5 million for the three months ended September 30, 2018 to $35.7 million for the three months ended September 30, 2019. The increase was primarily attributable to the following:

- an increase of $5.2 million for personnel costs as a result of increased headcount of research and development personnel, including a $1.7 million increase in stock-based compensation expense;
- an increase of $5.2 million for external costs associated with clinical trial activities for our lead product candidates;
- an increase of $3.2 million for external costs associated with manufacturing-related services; and
- an increase of $1.7 million for laboratory costs and facilities used by research and development personnel, including a $0.9 million increase in depreciation expense allocated to research and development functions.

General and Administrative Expense. General and administrative expenses increased by $3.4 million, from $9.0 million for the three months ended September 30, 2018 to $12.4 million for the three months ended September 30, 2019. The increase was primarily attributable to the following:

- an increase of $2.0 million for personnel costs as a result of increased headcount of general and administrative personnel, including a $1.0 million increase in stock-based compensation expense; and
- an increase of $0.4 million for professional services, including commercial, legal, accounting and other advisory services.

Investment Income. Investment income decreased by $1.7 million, from $2.1 million for the three months ended September 30, 2018 to $0.4 million for the three months ended September 30, 2019. The increase was primarily attributable to an unrealized loss of $2.2 million recognized during the three months ended September 30, 2019 related to our marketable equity securities of Prevail Therapeutics Inc. (Prevail). We acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its initial public offering (IPO) in June 2019. Pursuant to a lock-up agreement executed in connection with Prevail's IPO, we are restricted from selling our common stock of Prevail prior to December 2019. As of September 30, 2019, no amounts have been realized by us related to our marketable equity securities of Prevail as there have been no sales of the securities. Significant fluctuations in the fair value of the securities may occur from period to period.

Comparison of the Nine Months Ended September 30, 2019 and 2018

License and Royalty Revenue. License and royalty revenue decreased by $154.3 million, from $177.7 million for the nine months ended September 30, 2018 to $23.5 million for the nine months ended September 30, 2019. The decrease was primarily attributable to $176.1 million of non-recurring license revenue recognized during the nine months ended September 30, 2018 under our amended March 2014 license agreement with AveXis. The decrease was partially offset by $10.1 million of royalty revenue recognized during the nine months ended September 30, 2019 related to net sales of Zolgensma. Commercial sales of Zolgensma commenced in the second quarter of 2019. We are also eligible to receive a milestone payment of $80.0 million from AveXis upon the achievement of $1.0 billion in cumulative net sales of Zolgensma. The decrease in license and royalty revenue during the nine months ended September 30, 2019 resulted in a corresponding decrease in cost of revenues incurred during the period related to sublicense fees that we are obligated to pay to our licensors.

Research and Development Expense. Research and development expenses increased by $30.8 million, from $59.5 million for the nine months ended September 30, 2018 to $90.4 million for the nine months ended September 30, 2019. The increase was primarily attributable to the following:

- an increase of $12.2 million for personnel costs as a result of increased headcount of research and development personnel, including a $4.2 million increase in stock-based compensation expense;
- an increase of $6.7 million for external costs associated with clinical trial activities for our lead product candidates;
- an increase of $4.5 million for laboratory costs and facilities used by research and development personnel, including a $2.5 million increase in depreciation expense allocated to research and development functions; and
- an increase of $3.9 million for external costs associated with manufacturing-related services.

General and Administrative Expense. General and administrative expenses increased by $11.7 million, from $25.7 million for the nine months ended September 30, 2018 to $37.4 million for the nine months ended September 30, 2019. The increase was primarily attributable to the following:

- an increase of $6.5 million for personnel costs as a result of increased headcount of general and administrative personnel, including a $4.1 million increase in stock-based compensation expense; and
- an increase of $2.5 million for professional services, including commercial, legal, accounting and other advisory services.

Interest Income from Licensing. Interest income from licensing decreased by $6.3 million, from $8.4 million for the nine months ended September 30, 2018 to $2.1 million for the nine months ended September 30, 2019. The decrease was primarily attributable to $8.0 million of interest income recognized during the nine months ended September 30, 2018 under our amended March 2014 license agreement with AveXis.

Investment Income. Investment income increased by $33.8 million, from $4.2 million for the nine months ended September 30, 2018 to $38.0 million for the nine months ended September 30, 2019. The increase 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. We acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017.

Prevail completed its IPO in June 2019. Prior to Prevail's IPO, the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of $0.4 million. Upon Prevail's IPO in June 2019, the securities were reclassified to marketable securities and are measured at fair value. Pursuant to a lock-up agreement executed in connection with Prevail's IPO, we are restricted from selling our common stock of Prevail prior to December 2019. As of September 30, 2019, no amounts have been realized by us related to our marketable equity securities of Prevail as there have been no sales of the securities. Significant fluctuations in the fair value of the securities may occur from period to period.

Liquidity and Capital Resources

As of September 30, 2019, we had cash, cash equivalents and marketable securities of $417.1 million, which were primarily derived from the sale of 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, 2019, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months from the date of this report, based on our current business plan.

Commercial sales of Zolgensma commenced in the second quarter of 2019, upon which we began recognizing royalty revenue on net sales of the licensed product. We are also eligible to receive, in addition to other development milestone payments, a milestone payment of $80.0 million from AveXis upon the achievement of $1.0 billion in cumulative net sales of Zolgensma.

During the nine months ended September 30, 2019, we recognized an unrealized gain of $29.4 million related to our marketable equity securities of Prevail. As of September 30, 2019, our marketable equity securities of Prevail had a fair value of $29.8 million. We are restricted from selling our common stock of Prevail prior to December 2019, and significant fluctuations in the fair value of the securities may occur from period to period.

We have incurred cumulative losses since our inception and had an accumulated deficit of $151.4 million as of September 30, 2019. Our transition to recurring profitability is dependent upon the successful development, approval and commercialization of our product candidates and achieving a level of revenues adequate to support our cost structure. We do not expect to achieve such revenues, and expect to continue to incur losses, for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase for the foreseeable future as we continue the development of, and seek regulatory approval for, our product candidates. Subject to obtaining regulatory approval for our product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. Additionally, we expect our capital expenditures will increase significantly in the future for costs associated with building out additional office, laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

Cash Flows

	Nine Months Ended September 30,	
	2019	2018
	(in thousands)	
Net cash provided by (used in) operating activities	$ (82,199)	$ 108,788
Net cash provided by (used in) investing activities	79,405	(212,176)
Net cash provided by financing activities	6,827	199,155
Net increase in cash and cash equivalents and restricted cash	$ 4,033	$ 95,767

Operating Activities

Our net cash used in operating activities for the nine months ended September 30, 2019 increased by $191.0 million from the nine months ended September 30, 2018. The change was primarily attributable to $180.0 million in license payments we received during the nine months ended September 30, 2018 related to the amendment of our March 2014 license agreement with AveXis, as well as an increase in operating expenses in the nine months ended September 30, 2019. The increase in operating expenses 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.

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 were primarily attributable to an increase in accounts receivable of $10.7 million, an increase in other assets of $2.2 million and an increase in prepaid expenses of $1.4 million, and were partially offset by an increase in accounts payable of $5.3 million and a decrease in other current assets of $1.3 million. The increase in accounts receivable was largely driven by royalties on net sales of Zolgensma during the third quarter of 2019 which were recorded as accounts receivable as of September 30, 2019. The increases in prepaid expenses and other assets were largely driven by an increase in the amounts we were billed by service providers as of September 30, 2019 which are applicable to future periods of performance, including periods beyond 12 months from September 30, 2019.

For the nine months ended September 30, 2018, our net cash provided by operating activities of $108.8 million consisted of net income of $95.6 million, $7.0 million in adjustments for non-cash items and changes in working capital of $6.2 million. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of $11.8 million, depreciation and amortization expense of $2.7 million and net amortization of premiums on marketable debt securities of $0.9 million and were partially offset by imputed interest earned from our license agreements of $8.4 million. The changes in working capital were primarily attributable to a decrease in accounts receivable of $7.0 million and an increase in accrued expenses and other current liabilities of $3.4 million, and were partially offset by an increase in prepaid expenses and other current assets of $4.0 million.

Investing Activities

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.

For the nine months ended September 30, 2018, net cash used in investing activities consisted of $319.9 million to purchase marketable securities and $8.4 million to purchase property and equipment, offset by $116.1 million in sales and maturities of marketable securities.

Financing Activities

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.

For the nine months ended September 30, 2018, net cash provided by financing activities consisted of $189.3 million in aggregate net proceeds from a follow-on public offering of common stock, net of underwriting discounts and commissions and additional offering expenses we paid during the period, and $9.9 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

Future Funding Requirements

To date, we have primarily generated revenue through license agreements with strategic partners for research, development and commercialization of product candidates using our NAV Technology Platform. We do not expect to generate recurring revenue sufficient to offset our cost structure unless and until we obtain regulatory approval for and commercialize our product candidates. We expect our expenses to increase in connection with our ongoing development activities, particularly as we continue to expand the research, development and clinical trials of, and seek regulatory approval for, our product candidates. In addition, 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. We anticipate that we will need substantial additional funding in connection with our continuing operations.

We expect that our cash, cash equivalents and marketable securities as of September 30, 2019 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. We intend to devote the majority of our current capital to clinical development and seeking regulatory approval of our product candidates. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the amount of increased capital outlays and operating expenditures

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.

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, if any;
- 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

Our principal commitments include obligations under vendor contracts to provide research services and other purchase commitments with our vendors. In the normal course of business, we enter into services agreements with contract research organizations, contract manufacturing organizations and other third parties. Generally, these agreements provide for termination upon notice, with specified amounts due upon termination based on the timing of termination and the terms of the agreement. The actual amounts and timing of payments under these agreements are uncertain and contingent upon the initiation and completion of the services to be provided. These amounts are not fixed and determinable and therefore are not included in the table below.

Our commitments also include obligations to our licensors under our in-license agreements, which may include sublicense fees, milestones fees, royalties and reimbursement of patent maintenance costs. Sublicense fees are due to the licensors when we sublicense underlying intellectual property to third parties; the fees are based on a percentage of the license fees we receive from the

sublicensees. Based on license fees we have received from sublicensees or recorded as accounts receivable as of September 30, 2019, we have accrued $3.1 million of sublicense fees payable to our licensors, of which $1.3 million is expected to be paid within 12 months and $1.8 million is expected to be paid in periods beyond 12 months. The actual amount of sublicense fees payable in future periods could differ materially if new licenses are granted to sublicensees, existing licenses are terminated by sublicensees or if certain other contingent consideration, such as milestone payments, is received from sublicensees in the future. Accordingly, the amount of sublicense fees payable in future periods is not fixed and determinable and therefore is not included in the table below. Milestone fees are payable by us upon our future achievement of certain development and regulatory milestones. Royalties are payable by us based on a percentage of net sales of licensed products. Maintenance costs are reimbursements to the licensors for maintaining licensed patents. These amounts are not fixed and determinable and therefore are not included in the table below.

We have entered into a number of long-term leases for office and laboratory space in Rockville, Maryland and New York, New York, as well as a number of laboratory and other equipment leases. The table below includes the future minimum lease payments under our lease agreements.

The following table summarizes our contractual obligations as of September 30, 2019, excluding the items discussed above related to vendor contracts, purchase commitments and license commitments:

	Total	Remainder of 2019	2020 and 2021	2022 and 2023	2024 and Thereafter
			(in thousands)		
Future minimum lease payments	$ 105,662	$ 594	$ 8,438	$ 12,899	$ 83,731
Total contractual obligations	$ 105,662	$ 594	$ 8,438	$ 12,899	$ 83,731

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, “Qualitative and Quantitative Disclosures About Market Risk,” included in our most recent Annual Report on Form 10-K for the year ended December 31, 2018. There have been no material changes to our exposure to market risk during the nine months ended September 30, 2019.

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, 2019. 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, 2019, 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, 2019 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls

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

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

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

Item 1A. Risk Factors.

Our material risk factors are disclosed in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2018 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2019. There have been no material changes from the risk factors previously disclosed in such filings, other than the risk factors set forth below:

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

Our business depends substantially on the success of our lead product candidates. If we are unable to obtain regulatory approval for, or successfully commercialize, our lead product candidates, our business will be materially harmed.

Our lead product candidates are in the early stages of development and will require substantial clinical development and testing, manufacturing bridging studies and process validation and regulatory approval prior to commercialization. Successful continued development and ultimate regulatory approval of our lead product candidates is critical for our future business success and our ability to generate product revenue. We have invested, and will continue to invest, a significant portion of our financial resources in the development of our lead product candidates. We will need to raise sufficient funds for, and successfully complete, our clinical trials of our lead product candidates in appropriate subjects. The future regulatory and commercial success of these product candidates is subject to a number of risks, including the following:

- we may not have sufficient financial and other resources or patient availability to complete the necessary clinical trials for our lead product candidates;
- we may not be able to provide evidence of quality, efficacy and safety for our lead product candidates;
- we do not know the degree to which our lead product candidates will be accepted by patients, the medical community and third-party payors as a therapy for the respective diseases to which they relate, even if approved;
- the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA, EMA or comparable foreign regulatory bodies for marketing approval, and modifications to the design of our clinical trials could delay their enrollment, commencement or completion;
- subjects in our clinical trials may die or suffer other adverse effects for reasons that may or may not be related to our lead product candidates;
- subjects in clinical trials undertaken by licensees under a license we grant of certain intellectual property related to our NAV Technology Platform (our NAV Technology Licensees), or undertaken by others using AAV, may die or suffer other adverse effects for reasons that may or may not be related to our NAV Technology Platform or AAV;
- certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes;
- we may not successfully establish commercial manufacturing capabilities;
- if approved for treatment of the expected conditions, our lead product candidates will likely compete with other treatments then available, including the off-label use of products already approved for marketing and other therapies currently available or which may be developed;
- our products and products developed by our NAV Technology Licensees, if any, may not maintain a continued acceptable safety profile following regulatory approval;
- we may not maintain compliance with post-approval regulation and other requirements; and
- we may not be able to obtain, maintain or enforce our rights under our licensed patents and other intellectual property rights.

We have been informed by the FDA that they are assessing aspects of the surgical delivery system for RGX-314, specifically the use of certain third-party commercially available devices that are used to deliver RGX-314 in our Phase I/IIa trial for the treatment of wet age-related macular degeneration (wet AMD). While discussions with the FDA are ongoing, the investigational new drug (IND) application for the trial has been placed on partial clinical hold, which is not related to the gene therapy product itself. The FDA

may decide not to lift the partial clinical hold promptly, if at all. If the FDA does not lift the partial clinical hold or maintains the hold for longer than we expect, then our business may be materially harmed.

Of the large number of biologics and drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a Biologics License Application (BLA) to the FDA or marketing authorization application (MAA) to the EMA and even fewer are approved for commercialization. Furthermore, even if we do receive regulatory approval to market our lead product candidates, any such approval may be subject to limitations on the indicated uses for which we may market the product. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development programs, we cannot assure you that our lead product candidates will be successfully developed or commercialized. If we or any of our future development partners are unable to develop, or obtain regulatory approval for, or, if approved, successfully commercialize, our lead product candidates, we may not be able to generate sufficient revenue to continue our business.

We may encounter substantial delays in our planned clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates. Clinical testing is expensive, time-consuming and uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely commencement and completion of preclinical and clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board or independent Ethics Committee approval at each clinical trial site;
- delays in recruiting suitable subjects to participate in our clinical trials;
- imposition of a clinical hold by regulatory authorities, as the FDA has done with our IND application for RGX-314 for the treatment of wet AMD, including as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA good clinical practice (GCP), or applicable regulatory guidelines in the European Union and other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform;
- delays in having subjects complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or subjects dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete research studies, preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our planned clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;

- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing or other requirements;
- have regulatory authorities withdraw, vary or suspend their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Risks Related to the Commercialization of Our Product Candidates

If we do not achieve our projected development goals in the time frames we announce and expect, the commercialization of our products may be delayed and, as a result, our stock price may decline.

From time to time, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include, but are not limited to, the commencement or completion of scientific studies and clinical trials, the submission of regulatory filings, the announcement of results from scientific studies or clinical trials and the announcement of additional product candidates. From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones are and will be based on numerous assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in some cases for reasons beyond our control. If we do not meet these milestones as publicly announced, or at all, the commercialization of our products may be delayed or never achieved and, as a result, our stock price may decline.

We have been informed by the FDA that they are assessing aspects of the surgical delivery system for RGX-314, specifically the use of certain third-party commercially available devices that are used to deliver RGX-314 in our Phase I/IIa trial for the treatment of wet AMD. While discussions with the FDA are ongoing, the IND application for the trial has been placed on partial clinical hold, which is not related to the gene therapy product itself. The FDA may decide not to lift the partial clinical hold promptly, if at all. If the FDA does not lift the partial clinical hold or maintains the hold for longer than we expect, then we may not achieve our projected development goals for RGX-314 in the time frames we have announced, or at all, the commercialization of RGX-314 may be delayed or never achieved and, as a result, our stock price may decline.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

On November 4, 2019, we entered into the second amendment (the Lease Amendment) to our lease agreement with ARE Maryland No. 24, LLC relating to a building (the Building) being constructed at 9800 Medical Center Drive in Rockville, Maryland (the 9800 Medical Center Drive Lease).

The Lease Amendment expands the premises under the 9800 Medical Center Drive Lease (the Premises) by approximately 38,000 rentable square feet (rsf), including approximately 6,000 rsf on the first floor of the Building and approximately 32,000 rsf on the second floor the Building, resulting in all of the rentable square footage of the Building being included in the Premises. The additional future minimum lease payments resulting from the expansion of the Premises pursuant to the Lease Amendment are expected to total $22.6 million, subject to adjustment upon confirmation of the rentable area of the Premises,

The foregoing descriptions of certain terms of the Lease Amendment do not purport to be complete and are qualified in their entirety by reference to the full text of the Lease Amendment, which is included as Exhibit 10.1 to this Quarterly Report on Form 10-Q.

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	Second Amendment to Lease dated November 4, 2019 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, 2019 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, 2019 formatted in Inline XBRL (included in Exhibit 101)				

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

SIGNATURES

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

REGENXBIO Inc.

Dated: November 5, 2019

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

Dated: November 5, 2019

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

SECOND AMENDMENT TO LEASE AGREEMENT

THIS SECOND AMENDMENT TO LEASE AGREEMENT ("**this Second Amendment**") is dated as of November 4, 2019 ("**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 and a First Amendment to Lease Agreement dated April 23 2019 ("**First Amendment**"; together with the Original Lease, the "**Lease**"), wherein Landlord leased to Tenant certain premises containing approximately 139,281 rentable square feet ("**Original 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 (i) expand the Original Premises by approximately 6,016 rentable square feet on the first floor of the Building as shown on **Exhibit A** attached hereto labeled "1st Floor BOMA Plan" ("**First Floor Expansion Premises**"), (ii) expand the Original Premises by approximately 31,535 rentable square feet on the second floor of the Building as shown on **Exhibit A** attached hereto labeled "2nd Floor BOMA Plan" ("**Second Floor Expansion Premises**"), (iii) adjust certain definitions contained in the Basic Lease Provisions to reflect the addition of the First Floor Expansion Premises and the Second Floor Expansion Premises, (iv) modify the amount of the TI Allowance based on the addition of the First Floor Expansion Premises and the Second Floor Expansion Premises, and (v) adjust certain provisions of the Lease governing maintenance and repair, signage, and the Emergency Generators, all on the terms and conditions set forth in this Second Amendment.

AGREEMENT

Now, therefore, the parties hereto agree that the Lease is amended as follows:

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

2. Expansion of Premises. Effective as of the Effective Date, (i) the Original Premises shall be expanded to include the First Floor Expansion Premises and the Second Floor Expansion Premises resulting in Tenant now leasing all of the rentable square footage of the Building in its entirety, (ii) **Exhibit A** to this Second Amendment, which depicts the First Floor Expansion Premises, the Second Floor Expansion Premises, and the Original Premises, hereby replaces **Exhibit A** to the Lease, and (iii) the following amendments are hereby made to the definitions contained in the Basic Lease Provisions:

2.1 The section of the Basic Lease Provisions of the Lease entitled, "**Premises**", is hereby amended by (i) deleting the first (1st) paragraph thereof in its entirety and replacing it with the following:

That portion of the Project, containing approximately 176,832 rentable square feet, as determined by Landlord, as shown as the hatched area on **Exhibit A**. The Premises consist of the following, all of which are depicted on **Exhibit A**: (i) approximately 24,703 rentable square feet located on the first floor of the Building ("**1st Floor Premises**"), (ii) approximately 31,535 rentable square feet on the second floor of the Building ("**2nd Floor Premises**), (iii) approximately 40,198 rentable square feet located on the third floor of the Building ("**3rd Floor Premises**"), (iv) approximately 40,198 rentable square feet located on the 4th floor of the Building ("**4th Floor Premises**"), and (v) approximately 40,198 rentable square feet located on the 5th floor of the Building ("**5th Floor Premises**"). The 1st Floor Premises, the 2nd Floor Premises, the 3rd Floor Premises, and the 5th Floor Premises are collectively referred to as the "**Initial Premises**," and the Initial Premises and 4th Floor Premises are collectively referred to as the "**Premises**."

2.2 The section of the Basic Lease Provisions of the Lease entitled, "**Base Rent**", shall be deleted in its entirety and replaced with the following:

Base Rent: Initially, $435,253.13 per month (i.e., $37.50 per rentable square foot per annum x 139,281 rentable square feet) for the Premises, subject to adjustment upon confirmation of the rentable area of the Premises as provided above.

2.3 The section of the Basic Lease Provisions of the Lease entitled, "**Rentable Area of Premises**", shall be deleted in its entirety and replaced with the following:

Rentable Area of Premises: 176,832 rentable square feet, subject to adjustment upon confirmation of the rentable area of the Premises as provided above.

2.4 The section of the Basic Lease Provisions of the Lease entitled, "**Rentable Area of Project**", shall be deleted in its entirety and replaced with the following:

Rentable Area of Project: 459,268 rentable square feet, subject to adjustment upon confirmation of the rentable area of the Building as provided above. As of the Commencement Date, set forth below is the rentable area of the buildings located in the Project (excluding Building E, the Parking Garage):

Building A:	43,380 rentable square feet
Building B:	58,326 rentable square feet
Building C:	124,351 rentable square feet
Building D:	56,379 rentable square feet
Building F:	176,832 rentable square feet
Total:	459,268 rentable square feet

Landlord covenants and agrees that Landlord shall not re-measure the rentable area of the Project during the Term except to reflect actual changes in the physical size of the Project, and then only in accordance with the measurement standards that have been used historically to measure such rentable area of the Project. Tenant's Project Share and Building's Share of Project shall be promptly re-adjusted based on any changes in the Rentable Area of Project after the Commencement Date.

2.5 The section of the Basic Lease Provisions of the Lease entitled, "**Tenant's Share**", shall be deleted in its entirety and replaced with the following:

Tenant's Share: 100%, subject to adjustment upon confirmation of the rentable areas of the Building and the Premises as provided above.

2.6 The section of the Basic Lease Provisions of the Lease entitled, "**Tenant's Project Share**", shall be deleted in its entirety and replaced with the following:

Tenant's Project Share: 38.50%, subject to adjustment upon confirmation of the rentable areas of the Building and the Premises as provided above.

2.7 The section of the Basic Lease Provisions of the Lease, entitled "**Rentable Area of Building**", shall be deleted in its entirety and replaced with the following:

Rentable Area of Building: 176,832 rentable square feet, subject to adjustment upon confirmation of the rentable area of the Building as provided above.

2.8 The section of the Basic Lease Provisions of the Lease, entitled "**Building's Share of Project**", shall be deleted in its entirety and replaced with the following:

Building's Share of Project: 38.50% (i.e., 176,832/459,268), subject to adjustment upon confirmation of the rentable area of the Building as provided above.

2.9 Effective as of the Effective Date, the section of the Basic Lease Provisions of the Lease, entitled "**Landlord's Notice Address**", shall be deleted in its entirety and replaced with the following:

26 North Euclid Avenue
Pasadena, CA 91101
Attention: Corporate Secretary

2.10 The section of the Basic Lease Provisions of the Lease, entitled "**Security Deposit**", shall be deleted in its entirety and replaced with the following:

Security Deposit: $1,105,200 (i.e., $870,506.25, which represents 2 months of Base Rent for the Premises (exclusive of the Expansion Premises (as defined in the First Amendment)) at the initial rate set forth above, plus $234,693.75, which represents 2 months of Base Rent for the First Floor Expansion Premises and the Second Floor Expansion Premises at the initial rate set forth above), subject to adjustment upon confirmation of the rentable area of the Premises as provided above. By no later than the Effective Date of that certain Second Amendment to Lease Agreement between Landlord and Tenant, Tenant shall deliver to Landlord an amendment to the existing Letter of Credit increasing the amount of the existing Letter of Credit to $1,105,200.

2.11 **Amendment to Exhibits**. The section of the Basic Lease Provisions of the Lease containing the table of Exhibits is hereby amended by adding the following new Exhibits after EXHIBIT I:

EXHIBIT J – ROOF SPACE
EXHIBIT K- EQUIPMENT YARD

3. Amendment to Section 1 (Lease of Premises). Section 1 of the Lease is hereby amended by deleting the last sentence thereof and replacing it with the following new sentence:

Subject to a Taking (as defined in Section 19) and Force Majeure (as defined in Section 34), Tenant shall have, 24 hours per day, 7 days per week, 365/366 days per year during the Term, (a) exclusive use of and access to the Premises, (b) exclusive use of and access to the roof (subject to the provisions of Section 13), penthouse, and equipment yard (as shown on **Exhibit K** attached hereto), which areas do not form a part of the Premises, and (c) the right to use the Common Areas subject to a Taking, Force Majeure, and the provisions of Section 13.

4. Amendment to Section 2(b) (Definitions of Various Dates). Section 2(b) of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 2(b):

(b) Definitions of Various Dates. For purposes of this Lease:

(i) the "**Commencement Date**" shall mean the date of this Lease;

(ii) the "**Lease Commencement Date**" means 10 business days after Landlord Delivers the Premises for the performance of the Tenant Improvements;

(iii) the "**Rent Commencement Date**" for the Initial Premises

(exclusive of the First Floor Expansion Premises and the Second Floor Expansion Premises) means the first anniversary of the Lease Commencement Date;

(iv) the "**4th Floor Rent Commencement Date**" for the 4th Floor Premises means the first anniversary of the Rent Commencement Date; and

(v) the "**First Floor/Second Floor Expansion Premises Rent Commencement Date**" for the First Floor Expansion Premises and the Second Floor Expansion Premises means the date that is 10 months after the 4th Floor Rent Commencement Date.

Landlord shall provide Tenant with notice of the anticipated the Lease Commencement Date not more than 60 days and not less than 30 days before such date. Upon written request of Landlord, Tenant shall execute and deliver one or more written acknowledgments of the Commencement Date, the Lease Commencement Date, the Rent Commencement Date, the 4th Floor Rent Commencement Date, the First Floor/Second Floor Expansion Premises Rent Commencement Date, and the expiration date of the Base Term when such are established in the form of the "Acknowledgement of Commencement Date" attached to this Lease as **Exhibit D**; provided, however, that Landlord's failure to request, provide, or execute, or Tenant's failure to execute and deliver, such acknowledgment(s) shall not affect Landlord's or Tenant's rights and obligations hereunder. The "**Term**" of this Lease shall be the Base Term, as defined above in the Basic Lease Provisions and any Extension Terms that Tenant may elect pursuant to Section 42 (Right to Extend Term).

5. New Section 2(h) (Occupancy of First Floor Expansion Premises and Second Floor Expansion Premises). Section 2 of the Lease is hereby amended by adding the following new Section 2(h) immediately after Section 2(g):

(g) Occupancy of First Floor Expansion Premises and Second Floor Expansion Premises. Notwithstanding anything to the contrary contained herein, Tenant shall have the right to occupy and use the First Floor Expansion Premises and the Second Floor Expansion Premises at any time and from time to time from and after the Lease Commencement Date, for the Permitted Use, without affecting the First Floor/Second Floor Expansion Premises Rent Commencement Date or triggering the payment of Base Rent with respect to First Floor Expansion Premises and the Second Floor Expansion Premises before such date.

6. Amendment to Section 3(a) (Base Rent). Section 3(a) of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 3(a):

(a) Base Rent. The first month's Base Rent for the Premises and the Security Deposit shall be due and payable on delivery of a Tenant-executed copy of this Lease to Landlord; such first month's Base Rent shall be applied to the first payment(s) of Base Rent during the Term after the Initial Premises Base Rent Abatement. Beginning on the Rent Commencement Date, the 4th Floor Rent Commencement Date, and the First Floor/Second Floor Expansion Premises Rent Commencement Date, respectively, and on or before the first day of each calendar month thereafter during the Term hereof (but subject to the Base Rent Abatement described in Section 4(a)) Tenant shall pay to Landlord in advance, without demand, abatement, deduction or set-off, except as otherwise provided herein, monthly installments of Base Rent for the Initial Premises, the 4th Floor Premises, the First Floor Expansion Premises, and the Second Floor Expansion Premises, respectively, in lawful money of the United States of America, at the office of Landlord for payment of Rent set forth above, or to such other entity or person or at such other place as Landlord may from time to time notify Tenant in writing not less than 30 days in advance. Payments of Base Rent for any fractional calendar month shall be prorated on a per diem basis. The obligation of Tenant to pay Base Rent and other sums to Landlord and the obligations of Landlord under this Lease are independent obligations. Tenant shall have no right at any time to abate, reduce, or set-off any Rent (as defined in Section 5) due hereunder except for any abatement or set-off as may be expressly provided in this Lease.

7. Amendment to Section 4(a) (Base Rent Abatement). Section 4(a) of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 4(a):

(a) Base Rent Abatement. Notwithstanding anything to the contrary contained in this Lease, but provided Tenant is not in Default hereunder, Landlord hereby grants Tenant an abatement of the Base Rent:

(i) payable for the Initial Premises (exclusive of the First Floor Expansion Premises and the Second Floor Expansion Premises) during the period beginning on the Lease Commencement Date and ending the day before the Rent Commencement Date (i.e., 12 months after the Lease Commencement Date) ("**Initial Premises Base Rent Abatement**");

(ii) payable for the 4th Floor Premises during the period beginning on the Lease Commencement Date and ending the day before the 4th Floor Rent Commencement Date (i.e., 12 months after the Rent Commencement Date; 24 months after the Lease Commencement Date) ("**4th Floor Base Rent Abatement**"); and

(iii) payable for the First Floor Expansion Premises and the Second Floor Expansion Premises during the period beginning on the Lease Commencement Date and ending the day before the First

Floor/Second Floor Expansion Premises Rent Commencement Date (i.e., 22 months after the Rent Commencement Date; 34 months after the Lease Commencement Date) ("**First Floor/Second Floor Expansion Premises Base Rent Abatement**"; together with the Initial Premises Base Rent Abatement and the 4th Floor Base Rent Abatement, the "**Base Rent Abatement**").

For the avoidance of doubt, if the Lease Commencement Date occurs on the first day of a calendar month, the Base Rent Abatement will be measured from that date. If the Lease Commencement Date occurs on a day other than the first day of a calendar month, the Base Rent Abatement will be measured from the first day of the following calendar month. Except as provided in the preceding sentences, Tenant shall pay the full amount of Base Rent due in accordance with the provisions of this Lease. The administration rent set forth in Section 5 below 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. Notwithstanding anything to the contrary in this Section 4(a), the adjustment in the Base Rent as set forth in this Section 4 shall be based on the full and unabated amount of Base Rent payable for the first 12 month period from and after the Lease Commencement Date.

8. Amendment to Section 5 (Operating Expense Payments). Section 5 of the Lease is hereby amended by deleting the second paragraph in that Section in its entirety and replacing it with the following new second paragraph:

The term "**Operating Expenses**" means, except as otherwise provided herein, all costs and expenses of any kind or description whatsoever incurred or accrued each calendar year by Landlord with respect to the Building (including the Building's Share of all costs and expenses of any kind or description incurred or accrued by Landlord with respect to the Project which are not specific to the Building or any other building located in the Project) determined in accordance with generally acceptable accounting principles consistently applied from year to year as such principles are generally applied in the real estate industry ("**GAAP**") (including, without duplication, Taxes (as defined in Section 9), a Capital Repair/Replacement (as defined in Section 14(a)) made to accomplish a reduction in the Operating Expenses, to comply with any changes in applicable Legal Requirements enacted after the Lease Commencement Date or to ensure continued compliance with Legal Requirements in effect on the Lease Commencement Date, or to replace worn out or obsolete building systems or equipment (which Capital Repair/Replacement shall be amortized over its useful life on a straight-line basis without interest), the costs of Landlord's third party property manager or, if there is no third party property manager, administration rent in the amount of 3% of Base Rent, and the cost to repair or replace exterior glass, caulking, or brick, the cost of any tuck pointing), excluding only:

9. Amendment to Sections 5(a), 5(b), and 5(e) (Operating Expense Payments). Sections 5(a) and 5(b) of the Lease are hereby amended by deleting Sections 5(a), 5(b), and 5(e) in their entirety and replacing them with the following new Sections 5(a), 5(b), and 5(e):

(a) costs of repair or replacement of the roof, foundation, slab, and structural walls of the Building, the original construction costs of the Project, the costs of any subsequent renovation of the Project (except for any Capital Repair/Replacement and normal repair and maintenance, the costs of which are includable in Operating Expenses as provided above), and costs of correcting defects in such original construction or renovation;

(b) capital expenditures for expansion or renovation of the Project (except for any Capital Repair/Replacement, the cost of which is includable in Operating Expenses as provided above);

* * * * * * * * * * * * * * * * * * *

(e) amortization and depreciation of the Project (except for amortization of any Capital Repair/Replacement, which is includable in Operating Expenses as provided above);

10. Amendment to Section 7(a) (Modifications to Common Areas). Section 7(a) of the Lease is hereby amended by deleting the first sentence and replacing it with the following new sentence:

Landlord shall, as an Operating Expense (to the extent such Legal Requirement is first generally applicable to the Building after the Lease Commencement Date and subject to the provisions of Section 5 requiring amortization of costs of certain Capital Repair/Replacement) or at Tenant's expense (to the extent such Legal Requirement is applicable solely by reason of Tenant's, as compared to other biotechnology or laboratory tenants of the Project, particular use of the Premises) make any alterations, repairs, replacements, or modifications to the Common Areas and to the exterior, structure, and roof of the Building that are required by Legal Requirements, including the ADA

11. Amendment to Section 7(c) (Loading Docks). Section 7(c) of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 7(c):

(c) **Loading Docks**. The Building will contain 2 loading docks as identified on **Exhibit H** attached hereto. Tenant shall have an exclusive license to use both loading docks and associated receiving areas serving the Building in accordance with Legal Requirements and the terms and conditions of this paragraph (c) ("**Loading Docks**"). Tenant shall be permitted at its sole cost and expense, to install, maintain, repair, replace,

and remove signage, as reasonably approved by Landlord, at and/or over the Loading Docks denoting that the Loading Docks are for Tenant's exclusive use, and to install fencing, caging, or other comparable barrier in the associated receiving areas (together with the Loading Docks, the "**Loading Dock Areas**") as reasonably approved by Landlord, to segregate the Loading Dock, and to use the Loading Dock Areas for staging and storage purposes as permitted by this Lease. During any period of replacement, repair, or maintenance of the Loading Docks when they are not operational, Landlord shall have no obligation to provide Tenant with alternative, supplemental, temporary, or back-up loading docks, provided that Landlord shall perform such replacement, repair, or maintenance in a manner that minimizes, to the extent reasonably practicable, the duration and extent of any material interference with Tenant's ability to use the Loading Docks, including limiting staging replacement, repair, and maintenance activities on one Loading Dock at a time. Except as expressly set forth in Section 30(i) below, Landlord makes no warranties of any kind, express or implied, with respect to the Loading Docks, and Landlord disclaims any such warranties. Without limiting the foregoing, Tenant expressly acknowledges and agrees that Landlord does not guaranty or warrant that the Loading Docks will be operational at all times, will be of sufficient capacity to accommodate Tenant's use thereof, will be free of Hazardous Materials except as expressly set forth in Section 30(i) below, or will function or perform adequately, and Landlord shall not be liable for any damages resulting from the failure of the Loading Docks. Although the Loading Docks do not form a part of the Premises, the applicable provisions of this Lease (A) governing Tenant's compliance with Legal Requirements, (B) imposing obligations on Tenant for matters occurring in, on, within, or about the Premises or arising out of the use or occupancy of the Premises (including, but not limited to, those obligations relating to insurance, indemnification, Hazardous Materials Clearance (as defined in Section 18), and Environmental Requirements (as defined in Section 30(i)), or (C) limiting Landlord's liability, shall apply with equal force to Tenant's use of the Loading Docks.

(1) If Tenant defaults in its obligations under this Section 7(c) and fails to cure such default within 3 business days after written notice from Landlord, Landlord shall have the right, in addition to any other rights and remedies available to Landlord for a Default by Tenant, to suspend immediately Tenant's license to use the Loading Docks. If Tenant cures such default, Tenant's license to use the Loading Docks shall be immediately restored.

(2) Tenant shall have the right to enforce its right to use the Loading Dock Areas by posting warning notices on unauthorized vehicles blocking Tenant's use of the Loading Docks and/or causing any such vehicle to be towed by a reputable towing company engaged by Tenant (with any towing and storage charges being payable by the owners of any such towed

vehicle). Tenant shall indemnify, defend, hold and save Landlord harmless from and against any and all such Claims arising out of Tenant's enforcement of its right to use the Loading Dock Areas.

(3) The expiration or earlier termination of this Lease shall automatically terminate the license hereby granted to Tenant to so use the Loading Docks. The terms and provisions of this paragraph shall survive the expiration or termination of this Lease.

12. Amendment to Section 10 (Parking). Section 10 of the Lease is hereby amended by deleting the phrase "Tenant shall have the exclusive right to 10 reserved parking spaces" in the fourth sentence and replacing it with the following phrase: "Tenant shall have the exclusive right to 13 reserved parking spaces. . . ."

13. Amendment to Section 11(b) (Emergency Generators). Section 11(b) of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 11(b):

(b) Emergency Generators. Landlord's sole obligation for either providing emergency generators or providing emergency back-up power (collectively, "**Emergency Generators**") to Tenant shall be: (i) to provide emergency generators with not less than the stated capacity of the Emergency Generators to be located in the Building as of the Lease Commencement Date (which capacity shall not be less than 750 Kw), and (ii) to make connection of such Emergency Generators to the Premises available to Tenant during the Term. Landlord shall have no obligation to provide Tenant with operational Emergency Generators or back-up power or to supervise, oversee, and confirm that any third party maintaining the Emergency Generators is maintaining the Emergency Generators as per the manufacturer's standard guidelines or otherwise. During any period of replacement, repair, or maintenance of the Emergency Generators when the Emergency Generators are not operational, including any delays thereto due to the inability to obtain parts or replacement equipment, Landlord shall have no obligation to provide Tenant with an alternative back-up generator or generators or alternative sources of back-up power. Tenant expressly acknowledges and agrees that Landlord does not guaranty that such Emergency Generators will be operational at all times or that emergency power will be available to the Premises when needed.

14. Amendment to Section 12 (Alterations). Section 12 of the Lease is hereby amended by adding the following new Section 12(d) immediately after Section 12(c):

(d) February 2019 Material Changes. On the expiration or earlier termination of this Lease, Tenant shall have no obligation to remove the February 2019 Material Changes (as defined in the letter agreement between Landlord and Tenant dated as of April 12, 2019) from the Premises.

15. Amendment to Section 13 (Landlord's Repairs). Section 13 of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 13:

> **13. Landlord's Repairs**. Landlord shall, in accordance with the Maintenance Standard (as defined below), maintain the following: (a) as an Operating Expense (subject to the provisions of Section 5), exterior glass, caulking, tuck pointing, brick repair, Building façade, repair or replacement of roof membrane, exterior portions of the Building, landscaping, and parking areas (both surface and the Parking Garage), including snow and ice removal from the parking areas and sidewalks, and (b) at Landlord's expense and not as an Operating Expense, the repair or replacement of the roof, foundation, slab, and structural walls of the Building, correction of defects in the original construction of the Building, any repair or replacement to the extent covered under any warranties of Landlord's contractors or vendors, and of any damage to the Premises caused by the gross negligence or willful misconduct of Landlord or its employees. Losses and damages caused by Tenant, or by any of Tenant's agents, servants, employees, invitees, and contractors (collectively, "**Tenant Parties**"), shall be repaired by Landlord, to the extent not covered by insurance, at Tenant's sole cost and expense. Landlord reserves the right to stop Building Systems (as defined in Section 14(a)) services when necessary by reason of accident or emergency. Except as otherwise expressly provided in this Lease, Landlord shall have no responsibility or liability to supply Building Systems services during any such period of interruption; provided, however, that Landlord shall (A) except in case of emergency, give Tenant at least 3 business days' advance notice of any planned stoppage of Building Systems services for routine maintenance, repairs, alterations, or improvements, and (B) use all commercially reasonable efforts to minimize the extent and duration of such stoppage (including, where practicable, performing any such maintenance, repairs, alterations, or improvements at times and in manner that will minimize any stoppage of Building Systems services during normal business hours). Tenant waives its rights under any Legal Requirement to terminate this Lease or to make such repairs at Landlord's expense and agrees that the parties' respective rights with respect to such matters shall be solely as set forth herein. Repairs required as the result of fire, earthquake, flood, or other casualty, vandalism, war, or similar cause of damage or destruction shall be controlled by Section 18. Repairs required as a result of a Taking (as defined in Section 19) shall be controlled by Section 19. For purposes of this Lease, "**Maintenance Standard**" means the standards customarily maintained by owners of Class A office/laboratory buildings in the Gaithersburg/Rockville, Maryland market of comparable size and age, reasonable wear and tear excepted, and in compliance with all applicable Legal Requirements.

If, pursuant to Section 14(a), Tenant requests that Landlord perform a Capital Repair/Replacement, Landlord shall perform the Capital Repair/Replacement in accordance with the Maintenance Standard. The allocation of the cost to perform the Capital Repair/Replacement shall be governed by the provisions of Section 5.

16. Amendment to Section 14 (Tenant's Repairs). Section 14 of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 14:

14. Tenant's Repairs.

(a) General. Subject to Section 13 hereof and the provisions of this paragraph, Tenant, at its expense, shall repair, replace and maintain in accordance with the Maintenance Standard all portions of the Premises, including, without limitation, the interior of the Building, all HVAC systems, plumbing, fire sprinklers, elevators and all other building systems serving all or any part of the Building ("**Building Systems**"), entries, doors, ceilings, interior windows, demising walls, interior walls, the interior side of demising walls, the Emergency Generators described in Section 11(b), and the Dedicated Generator and Fuel Tank (if any). To the extent the repair or replacement of any Building System, Emergency Generator, Dedicated Generator and Fuel Tank, base Building HVAC system, or other interior portion of the Building constitutes a capital repair or replacement ("**Capital Repair/Replacement**"), Tenant shall promptly notify Landlord of the need for the Capital Repair/Replacement. On receipt of such notice, Landlord shall perform the Capital Repair/Replacement as more fully described in Section 13. Should Tenant fail to make any such repair or replacement that does not constitute a Capital Repair/Replacement or fail to maintain the Premises, Landlord shall give Tenant notice of such failure. If Tenant fails to commence cure of such failure within 10 business days of Landlord's notice, and thereafter diligently prosecute such cure to completion, Landlord may perform such work and shall be reimbursed by Tenant within 30 days after receipt of Landlord's written demand therefor (together with copies of the paid invoices evidencing the costs incurred by Landlord); provided, however, that if such failure by Tenant creates or could create an emergency, Landlord may immediately commence cure of such failure and shall notify Tenant promptly after such action has been undertaken, and thereafter be entitled to recover the costs of such cure from Tenant as provided in this paragraph. Subject to Sections 17 and 18, Tenant shall bear the full uninsured cost of any repair or replacement to any part of the Project that results from damage caused by Tenant or any Tenant Party and any repair that benefits only the Premises. For the avoidance of doubt, a repair shall be deemed to benefit only the Premises (i) if an act or omission by Tenant or any Tenant Party causes or triggers the need for such repair (but only to the extent the cost of which repair is not covered by insurance), or (ii) if Tenant requests a repair that Landlord

is not otherwise obligated to perform under the terms of this Lease. The cost of any such repair shall be excluded from the Operating Expenses if caused by any other tenant of the Building that is directly invoiced for such repair.

(b) Maintenance Contracts. Tenant, at its expense, shall at all times during the Term maintain with qualified contractors maintenance and repair contracts ("**Maintenance Contracts**") for (i) all HVAC systems serving all or any part of the Building, and (ii) the Emergency Generators. The Maintenance Contracts shall be in form and content reasonably satisfactory to Landlord. Landlord shall be a third party beneficiary of the Maintenance Contracts and, within 30 days after Landlord's request, Tenant shall deliver a copy of the Maintenance Contracts to Landlord.

17. Amendment to Section 38 (Signs; Exterior Appearance). Section 38 of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 38:

38. Signs; Exterior Appearance. Except as provided in this Section 38, Tenant shall not, without the prior written consent of Landlord, which consent shall not be unreasonably withheld, conditioned, or delayed: (i) attach any awnings, exterior lights, decorations, balloons, flags, pennants, banners, painting or other projection to any outside wall of the Project, (ii) use any curtains, blinds, shades or screens other than Landlord's standard window coverings as specified in the Basis of Design Report (as defined in **Exhibit C-1** attached hereto), (iii) coat or otherwise sunscreen the interior or exterior of any windows, (iv) place any bottles, parcels, or other articles on the window sills, (v) place any equipment, furniture or other items of personal property on any exterior balcony, or (vi) paint, affix or exhibit on any part of the Building or the Project any signs, notices, window or door lettering, placards, decorations, or advertising media of any type that can be viewed from the exterior of the Building. Subject to the limitation in subparagraph (vi) of the immediately preceding sentence, Tenant may install signs, notices, window or door lettering, placards, decorations, or advertising media of any type in the main lobby and all other interior locations of the Building without Landlord's consent. The Building directory tablet shall be provided exclusively for the display of the name and location of Tenant.

(a) Identification Signage. Tenant shall have the right, at its sole option, cost, and expense and in compliance with all applicable Legal Requirements, to install and affix to the exterior of the Building not more than 2 mounted, illuminated signs as desired by Tenant and permitted by applicable Legal Requirements (and related electrical connections and equipment) bearing the then-current name and the corporate logo of Tenant or any assignee of this Lease or sublessee of all or any portion of the Premises pursuant to a Permitted Transfer ("**Identification Signage**").

Such right shall be personal to REGENXBIO Inc. and any assignee of this Lease or sublessee of all or any portion of the Premises pursuant to a Permitted Transfer. Landlord shall have the right to approve the placement on the wall, size, and design of the Identification Signage, which approval shall not be unreasonably withheld, delayed, or conditioned. Landlord hereby approves the location of the Identification Signage set forth on **Exhibit G** attached hereto. Tenant shall, at its sole cost and expense, maintain the Identification Signage in good order and repair consistent with the Maintenance Standard and have the right to replace, renovate, and/or update the Identification Signage from time to time, subject to Landlord's approval, which approval shall not be unreasonably withheld, delayed, or conditioned. On the expiration or earlier termination of the Term, Tenant shall, at its sole cost and expense, (i) remove the Identification Signage in a good and workmanlike manner and in compliance with all applicable Legal Requirements, and (ii) repair any damage to the façade or appearance of the Building caused by installation, replacement, renovation, updating and/or removal of the Identification Signage. Except as provided in this sentence, Tenant's rights under this paragraph are exclusive and the percentage of the total identification signage occupied by Tenant's name on the exterior of the Building shall be 100%; provided, however, that Landlord reserves the right to place its name and identifying logo on the exterior of the Building in a location that does not reasonably detract from Tenant's signage rights under this paragraph.

(b) Monument Signage. Landlord shall, in compliance with all applicable Legal Requirements, install (before the Rent Commencement Date) and thereafter throughout the Term, maintain in good condition and repair, as part of the Operating Expenses, Tenant's name on the monument sign serving the Building. Landlord shall have the right to approve the placement on such monument sign, size, and design of such signage, which approval shall not be unreasonably withheld, delayed, or conditioned. Except as provided in this sentence, Tenant's rights under this paragraph to have signage on such monument sign are exclusive, it being understood and agreed that Landlord has not heretofore granted or may not hereafter grant rights to signage on such monument sign to any other person or entity, and the area occupied by Tenant's name on such monument sign shall not exceed Tenant's proportionate share of the monument (which share shall be equal to Tenant's Share (100%) as to any monument sign serving the Building and Tenant's Project Share as to any monument sign serving the Project) provided, however, that Landlord reserves the right to place its name and identifying logo toward the bottom of any such monument sign.

(c) Plaque Signage. Plaque signage will be located adjacent to the main entrance to the Building. Landlord shall install, before the Rent Commencement Date, and thereafter throughout the Term, maintain in good condition and repair, as part of the Operating Expenses, Tenant's

name on the plaque. Tenant shall have the right to approve the placement on the wall, size, and design of such plaque signage, which approval shall not be unreasonably withheld, delayed, or conditioned. Tenant's rights under this paragraph are exclusive and the percentage of the total plaque area occupied by Tenant's name on such plaque shall be 100%.

18. Section 39 (Right of First Negotiation). Section 39 of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following: "**Intentionally Deleted**."

19. Amendment to Section 40 (Right to Expand in the Building or Project). Section 40 of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 40:

40. Right to Expand in the Project.

(a) Expansion in the Project. Tenant shall have the right, but not the obligation, to expand the Premises ("**Project Expansion Right**") to include any Project Expansion Space (as defined below) in the Project upon the terms and conditions in this Section. For purposes of this Section, "**Project Expansion Space**" means any space in the Project containing at least 50,000 rentable square feet of contiguous space in Building B that is not occupied by a tenant or that is occupied by an existing tenant whose lease is expiring within 6 months or less and such tenant does not wish to renew (whether or not such tenant has a right to renew) its occupancy of such space. Landlord shall, when the availability of the Project Expansion Space becomes known (but not earlier than 180 days before such availability), deliver to Tenant written notice ("**Project Expansion Notice**") of the Project Expansion Space. The Project Expansion Notice shall set forth the terms and conditions on which Landlord is prepared to lease the Project Expansion Space. The rental terms for the Project Expansion Space shall be the fair market rent, including market concessions (collectively, "**Project Expansion Space FMR/Concessions**") as mutually determined by Landlord and Tenant, and the Project Expansion Notice shall set forth Landlord's proposed Project Expansion Space FMR/Concessions. Tenant shall respond to the Project Expansion Notice within 10 business days after receipt thereof, which response shall state that Tenant (1) declines to lease the Project Expansion Space, (2) agrees to lease the Project Expansion Space on the terms set forth in the Project Expansion Notice (including the Project Expansion Space FMR/Concessions), in which event Landlord and Tenant shall within a period of 15 days thereafter execute and deliver an amendment to this Lease or a lease agreement for the Project Expansion Space, or (3) desires to lease the Project Expansion Space but in good faith disagrees with the proposed Project Expansion Space FMR/Concessions, in which event Landlord and Tenant shall, for a period of up to 15 days, negotiate in good faith for Tenant's lease of the Project Expansion Space on mutually acceptable Project Expansion Space FMR/Concessions. If Landlord and Tenant are unable to agree on the Project

Expansion Space FMR/Concessions within such 15 day period after negotiating in good faith, the parties shall proceed to arbitration as set forth below.

i. **Project Expansion Space FMR/Concession Proposal**. Within 10 days after the expiration of such 15 day period, each party shall deliver to the other a proposal containing the Project Expansion Space FMR/Concessions that the submitting party believes to be correct ("**Project Expansion Space FMR/Concessions Proposal**"). If either party fails to timely submit a Project Expansion Space FMR/Concessions Proposal, and such failure is not remedied within 3 business days after written notice thereof to such party, the other party's submitted Project Expansion Space FMR/Concessions Proposal shall be deemed the Project Expansion Space FMR/Concessions. If both parties submit Project Expansion Space FMR/Concessions Proposals, then Landlord and Tenant shall meet within 7 days after delivery of the last Project Expansion Space FMR/Concessions Proposal and make a good faith attempt to mutually appoint a single Arbitrator to determine the Project Expansion Space FMR/Concessions. If Landlord and Tenant are unable to agree upon a single Arbitrator, then each shall, by written notice delivered to the other within 10 days after the meeting, select an Arbitrator. If either party fails to timely give notice of its selection for an Arbitrator, and such failure is not remedied within 3 business days after written notice thereof to such party, the other party's submitted Project Expansion Space FMR/Concessions Proposal shall be deemed the Project Expansion Space FMR/Concessions. The 2 Arbitrators so appointed shall, within 5 business days after their appointment, appoint a third Arbitrator. If the 2 Arbitrators so selected cannot agree on the selection of the third Arbitrator within the time above specified, then either party, on behalf of both parties, may request such appointment of such third Arbitrator by application to any state court of general jurisdiction in the jurisdiction in which the Premises are located, upon 10 days prior written notice to the other party of such intent.

ii. **Decision**. The decision of the Arbitrator(s) shall be made within 30 days after the appointment of a single Arbitrator or the third Arbitrator, as applicable. The decision of the Arbitrator(s) shall be limited solely as to which of the Project Expansion Space FMR/Concessions Proposals submitted by the parties is closest to the actual Project Expansion Space FMR/Concessions in such Arbitrator(s)' good faith professional judgment. The decision of the single Arbitrator, or the majority or unanimity of the 3 Arbitrator panel, as applicable, shall be final and binding upon the parties. Each party shall pay the fees and expenses of the Arbitrator appointed by or on behalf of such party (if applicable) and the fees and expenses of the agreed-upon single Arbitrator or the third Arbitrator, as applicable, shall be borne equally by both parties. Landlord and Tenant shall then execute and deliver a mutually acceptable amendment or a lease agreement recognizing the Project Expansion Space FMR/Concessions, along with

the other terms set forth in the Project Expansion Notice, for the Project Expansion Space.

Provided that no right to expand is exercised by any tenant with superior rights (as set forth in **Exhibit I**) and more than 3 years remain on the Term, Tenant shall be entitled to lease the Project Expansion Space upon the terms and conditions set forth in this Section 40(a).

b. **Amended Lease**. If Tenant fails to deliver notice responding to a Project Expansion Notice as provided herein within 10 business days following such tender, Tenant shall be deemed to have waived its right to lease the Project Expansion Space described in such Project Expansion Notice. Landlord and Tenant shall execute and deliver a lease amendment or lease agreement for the Project Expansion Space within the 30 day period from the date Tenant gives notice accepting Landlord's offer to lease the Project Expansion Space. Both Landlord and Tenant shall exercise diligence to ensure that such lease amendment or lease agreement is timely executed and delivered.

c. **Exceptions**. Notwithstanding the above, the Project Expansion Right shall not be in effect and may not be exercised by Tenant: (i) during any period of time that Tenant is in Default under any provision of this Lease; or (ii) if Tenant has been in Default under any provision of this Lease 3 or more times, regardless of whether the Defaults are cured, during the 12 month period prior to the date on which Tenant seeks to exercise the Project Expansion Right.

d. **Termination**. The Project Expansion Right shall terminate and be of no further force or effect even after Tenant's due and timely exercise of the Project Expansion Right, if, after such exercise, but prior to the commencement date of the lease of the Project Expansion Space, (i) Tenant is in Default under any provision of this Lease; or (ii) Tenant has Defaulted 3 or more times during the period from the date of the exercise of the Project Expansion Right to the date of the commencement of the lease of the Project Expansion Space, regardless of whether such Defaults are cured.

e. **Subordinate**. Tenant's rights in connection with the Project Expansion Right are and shall be subject to and subordinate to any expansion or extension rights granted in the Project as set forth in **Exhibit I**.

f. **Right Personal**. The Project Expansion Right is personal to REGENXBIO Inc. and is not assignable without Landlord's consent, which may be granted or withheld in Landlord's sole discretion separate and apart from any consent by Landlord to an assignment of Tenant's interest in this Lease, except that it shall automatically be assigned without Landlord's

consent in connection with any assignment of this Lease that is a Permitted Transfer.

g. **No Extensions**. The period of time within which any Project Expansion Right may be exercised shall not be extended or enlarged by reason of Tenant's inability to exercise the Project Expansion Right.

20. Amendment to Section 41 (Hold Space Option). Section 41 of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following: "**Intentionally Deleted**."

21. Amendment to Section 46 (Roof Equipment). Section 46 of the Lease is hereby amended by deleting that Section in its entirety and replacing it with the following new Section 46:

46. Roof Equipment. As long as Tenant is not in Default, Tenant shall have the right at its sole cost and expense, subject to compliance with all Legal Requirements, to install on the roof of the Building (in all space on the roof other than such space as is required by Landlord for (i) perimeter setbacks mandated by any Governmental Authority, (ii) Building HVAC systems, and (iii) solar panels installed by Landlord but thereafter maintained, repaired, and replaced by Tenant, such permitted space and such restricted space as further depicted on **Exhibit J** attached hereto; any notations of limitation on use depicted on **Exhibit J** shall, in all cases, be determined by applicable Legal Requirements), and throughout the Term, operate, maintain, repair, replace, and remove, (1) one or more satellite dishes, communication antennae, or other communications equipment (all of which having a diameter and height reasonably acceptable to Landlord) for the transmission or reception of communication of signals as Tenant may from time to time desire, and (2) equipment for any supplemental HVAC systems installed by or on behalf of Tenant and providing service to the Premises, as may be approved by Landlord as part of the Tenant Improvements or any subsequent Alterations (collectively, the "**Roof Equipment**"), on the following terms and conditions:

(a) Requirements. Tenant shall submit to Landlord (i) the plans and specifications for the installation of the Roof Equipment (which may be part of the plans and specifications for the Tenant Improvements or any subsequent Alterations), (ii) copies of all required governmental and quasi-governmental permits, licenses, and authorizations that Tenant will and must obtain at its own expense, with the cooperation of Landlord, if necessary for the installation and operation of the Roof Equipment, and (iii) an insurance policy or certificate of insurance evidencing insurance coverage as required by this Lease and any other insurance as reasonably required by Landlord for the installation and operation of the Roof Equipment, which such other insurance shall be consistent with that typically required by landlords of comparable buildings in the vicinity of the Project. Landlord shall not unreasonably withhold or delay its approval for

the installation and operation of the Roof Equipment; provided, however, that Landlord may reasonably withhold its approval if the installation or operation of the Roof Equipment (A) is reasonably likely to damage the structural integrity of the Building, (B) is reasonably likely to void, terminate, or invalidate any applicable roof warranty, (C) is reasonably likely to interfere with any service provided by Landlord, (D) is not reasonably screened from viewing from the ground level adjacent to the Building.

(1) Removal—Pre-Rent Commencement Date. Tenant shall not be required to remove all or any part of the Roof Equipment on the expiration or earlier termination of this Lease if the Roof Equipment has been installed on the roof of the Building (or intended to be installed per the final approved plans) on or before the Rent Commencement Date in accordance with the terms and conditions of this Lease.

(2) Removal—Post-Rent Commencement Date. With respect to any Roof Equipment that Tenant desires to install on the roof of the Building from and after the Rent Commencement Date, Tenant shall not be required to remove all or any part of such Roof Equipment on the expiration or earlier termination of this Lease unless, at the time of Landlord's approval, Landlord requires that Tenant remove all or any part of the Roof Equipment on the expiration or earlier termination of this Lease because such Roof Equipment is, in Landlord's reasonable judgment, (A) uniquely specific to Tenant's use and operation of the Building and (B) unlikely to be of use or utility to another tenant of the Building.

(b) No Damage to Roof. If installation of the Roof Equipment requires Tenant to make any roof cuts or perform any other roofing work, such cuts shall only be made in the manner reasonably designated in writing by Landlord; and any such installation work (including any roof cuts or other roofing work) shall be performed by Tenant, at Tenant's sole cost and expense by a roofing contractor designated by Landlord and reasonably acceptable to Tenant. If Tenant or its agents shall otherwise cause any damage to the roof during the installation, operation, and removal of the Roof Equipment such damage shall be repaired promptly at Tenant's expense and the roof shall be restored to the same condition it was in before the damage, reasonable wear and tear excepted. Landlord shall not charge Tenant Additional Rent for the installation and use of the Roof Equipment. If, however, Landlord's insurance premium or Tax assessment increases solely as a result of the Roof Equipment, Tenant shall pay such increase as Additional Rent within 30 days after receipt of a reasonably detailed invoice from Landlord. Tenant shall not be entitled to any abatement or reduction in the amount of Rent payable under this Lease if for any reason Tenant is unable to use the Roof Equipment. In no event whatsoever shall the installation, operation, maintenance, or removal of the

Roof Equipment by Tenant or its agents void, terminate, or invalidate any applicable roof warranty.

(c) **Protection**. The installation, operation, maintenance, and removal of the Roof Equipment shall be at Tenant's sole risk. Tenant shall indemnify, defend, and hold Landlord harmless from and against any and all claims, costs, damages, liabilities and expenses (including, but not limited to, reasonable attorneys' fees) of every kind and description that may arise out of or be connected in any way with Tenant's installation, operation, maintenance, or removal of the Roof Equipment, except to the extent caused by the willful misconduct or gross negligence of Landlord or its employees.

(d) **Removal**. Subject to the provisions of Section 46(a) above, at the expiration or earlier termination of this Lease or the permanent discontinuance of the use of the Roof Equipment by Tenant, Tenant shall, at its sole cost and expense, remove the Roof Equipment from the roof of the Building, in which event Tenant shall leave the portion of the roof where the Roof Equipment was located in the same condition it was in before the installation thereof, reasonable wear and tear excepted. If Tenant does not so remove the Roof Equipment, Tenant hereby authorizes Landlord to remove and dispose of the Roof Equipment and charge Tenant as Additional Rent for all reasonable out-of-pocket costs and expenses incurred by Landlord in such removal and disposal. Tenant agrees that Landlord shall not be liable for any Roof Equipment disposed of or removed by Landlord.

(e) **No Interference**. The Roof Equipment shall not interfere (other than in a de minimus manner) with the proper functioning of any telecommunications equipment or devices that have been installed or will be installed by Landlord. Tenant agrees that Landlord will be permitted to install such telecommunication equipment that is of a type and frequency that will not interfere (other than in a de minimus manner) with the Roof Equipment.

(f) **Intentionally Omitted**

(g) **Access**. The right of Tenant to install, operate, maintain, repair, replace, and remove the Roof Equipment shall be personal solely to REGENXBIO Inc. and its permitted assignees and subtenants, and (i) no other person or entity shall have any right to use or operate the Roof Equipment, and (ii) Tenant shall not assign, convey, or otherwise transfer to any person or entity any right, title, or interest in all or any portion of the Roof Equipment or the use and operation thereof, except that the right of Tenant to install, operate, maintain, repair, replace, and remove the Roof Equipment shall be automatically assigned without Landlord's consent in connection with any assignment of this Lease that is a Permitted Transfer.

(h) Appearance. The Roof Equipment shall be reasonably screened from viewing from the ground level adjacent to the Building.

22. Amendment to Exhibit C-1 (Landlord Work Letter)—New Section 2.j. Exhibit C-1 of the Lease is hereby amended by adding the following new Section 2.j immediately after Section 2.i:

j. **Miscellaneous Changes**. (1) Landlord shall retain (or restore to the Base Building Work) the "Won" horizontal accordion fire partition at the central stair location, (2) the Base Building Work shall provide a service elevator with front and rear access on the first floor of the Building (it being acknowledged that front and rear access for the service elevator exists on the fifth floor of the Building), (3) Tenant shall have the right to control the selection of the Building's automation system as long as such selection does not conflict with, and will function properly with, any previously procured, ordered, or installed Building automation system, and (4) Tenant shall have the right to control the selection of a security system for the Building.

23. Amendment to Exhibit C-1 (Landlord Work Letter)—Amendment to Section 3.c. (Warranties). Exhibit C-1 of the Lease is hereby amended by adding the following language at the end of Section 3.c.:

If Tenant is not an express third party beneficiary of the General Contractor's warranty or any such equipment manufacturer's warranty, Landlord shall assign to Tenant, on a non-exclusive basis and only to the extent Tenant has responsibility under the Lease for the maintenance, repair, or replacement of the component or item that is the subject of such warranty, such General Contractor's warranty and any such equipment manufacturer's warranty. If any such warranty is not assignable, Tenant shall request in writing that Landlord pursue a warranty claim if and when Tenant has a reasonable and good faith basis for asserting such a claim. If so requested, Landlord shall promptly pursue such warranty claim at no cost or expense to Tenant. For the avoidance of doubt, any warranty affecting the roof shall not be assigned to Tenant.

24. Adjustment to TI Allowance. Exhibit C-2 of the Lease is hereby amended by deleting Section 5(b) thereof in its entirety and replacing it with the following new Section 5(b):

(b) TI Allowance. Landlord shall provide to Tenant a tenant improvement allowance ("**TI Allowance**") of **$110** per rentable square foot of the Premises, or **$19,451,520** in the aggregate (based on the Premises containing 176,832 rentable square feet and subject to adjustment upon remeasurement of the Premises as provided in the Lease). The TI Allowance shall be disbursed in accordance with this Work Letter. Tenant shall have no right to any portion of the TI Allowance that is not requested before the last day of the month that is 24 months after the Lease

Commencement Date.

25. Amendment to Exhibit D (Acknowledgement of Commencement Date). Exhibit D of the Lease is hereby deleted in its entirety and replaced with the form of Exhibit D attached hereto.

26. Amendment to Exhibit H (Loading Docks and Dedicated Generator Area). Exhibit H of the Lease is hereby deleted in its entirety and replaced with the form of Exhibit H attached hereto.

27. New Exhibit J (Roof Space). Exhibit J of the Lease is hereby added in the form of Exhibit J attached hereto.

28. New Exhibit K (Equipment Yard). Exhibit K of the Lease is hereby added in the form of Exhibit K attached hereto.

29. Miscellaneous.

a. **Entire Agreement**. The Lease, as amended by this Second 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 Second Amendment, may be amended only by an agreement in writing, signed by the parties hereto.

b. **Binding Effect**. This Second 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 Second 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 Second Amendment.

d. **Counterparts**. This Second 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) 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 Second 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 Second 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 Second Amendment. In the event of any conflict between the provisions of this Second Amendment and the provisions of the Lease, the provisions of this Second Amendment shall prevail. Regardless of whether specifically amended by this Second 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 Second Amendment.

[**SIGNATURES APPEAR ON NEXT PAGE**]

IN WITNESS WHEREOF, the parties hereto have executed this Second 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/ Jennifer Banks (SEAL)
Name: Jennifer Banks
Title: Co-Chief Operating Officer
& General Counsel

EXHIBIT A
DESCRIPTION OF PREMISES

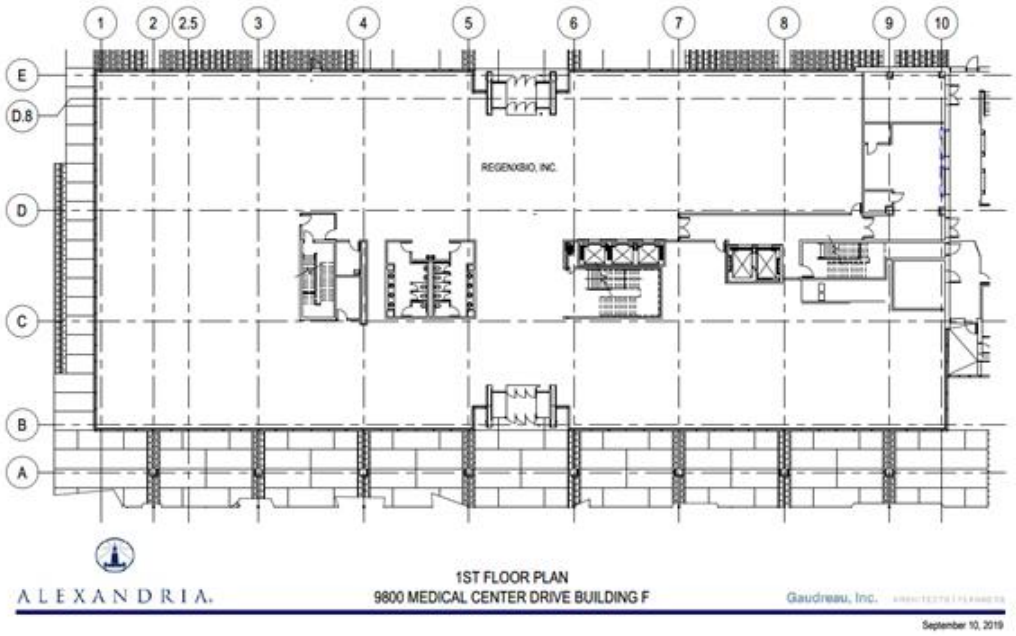


EXHIBIT A
DESCRIPTION OF PREMISES—continued

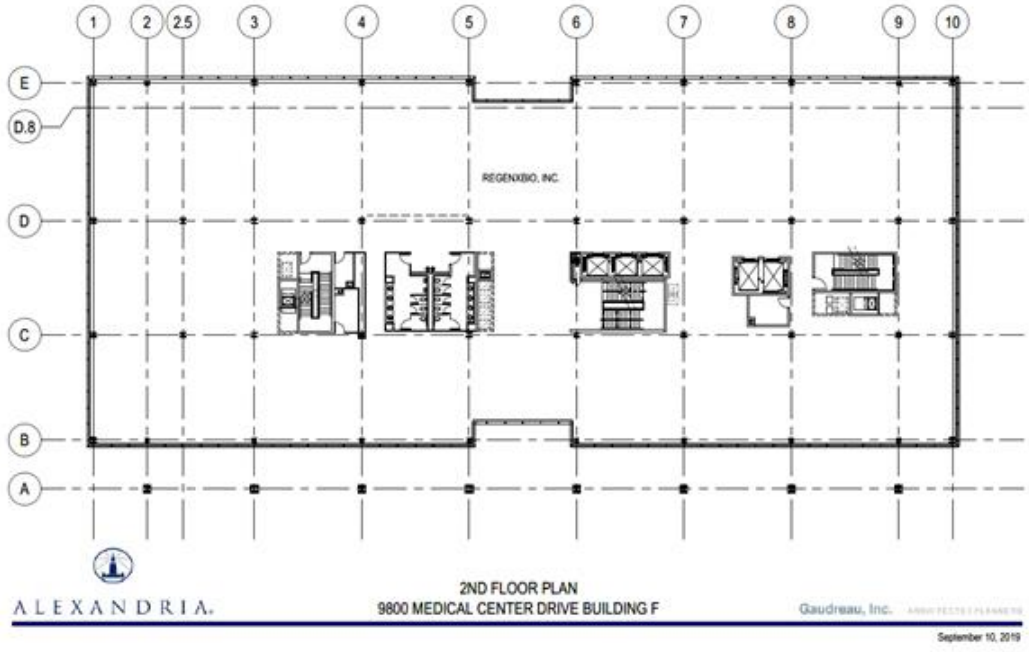
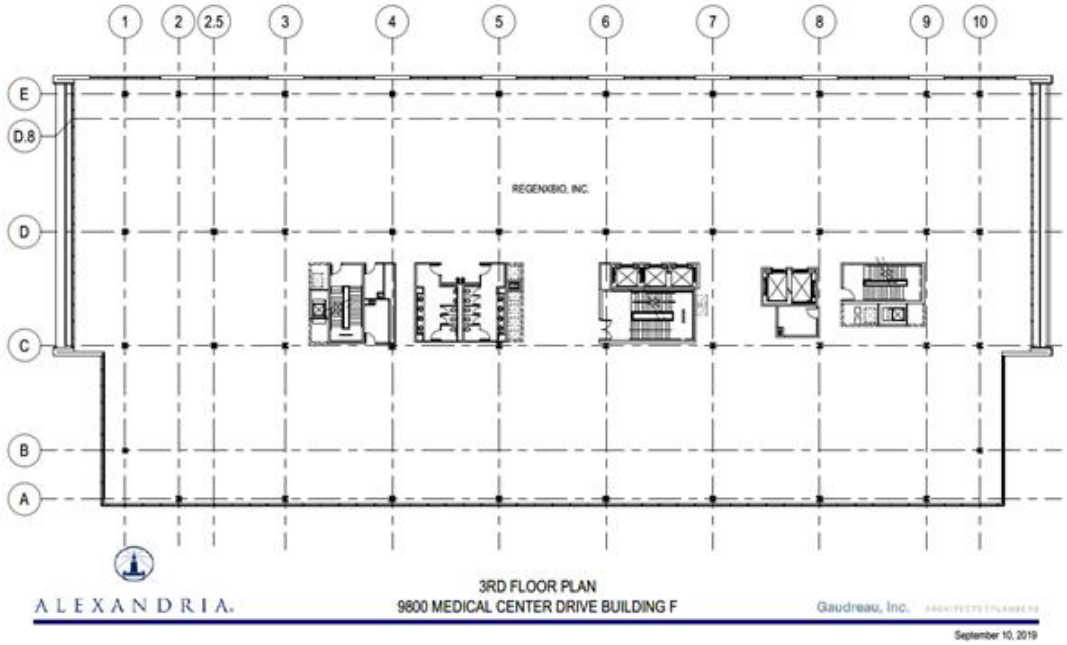


EXHIBIT A
DESCRIPTION OF PREMISES—continued



ALEXANDRIA.

EXHIBIT A
DESCRIPTION OF PREMISES—continued

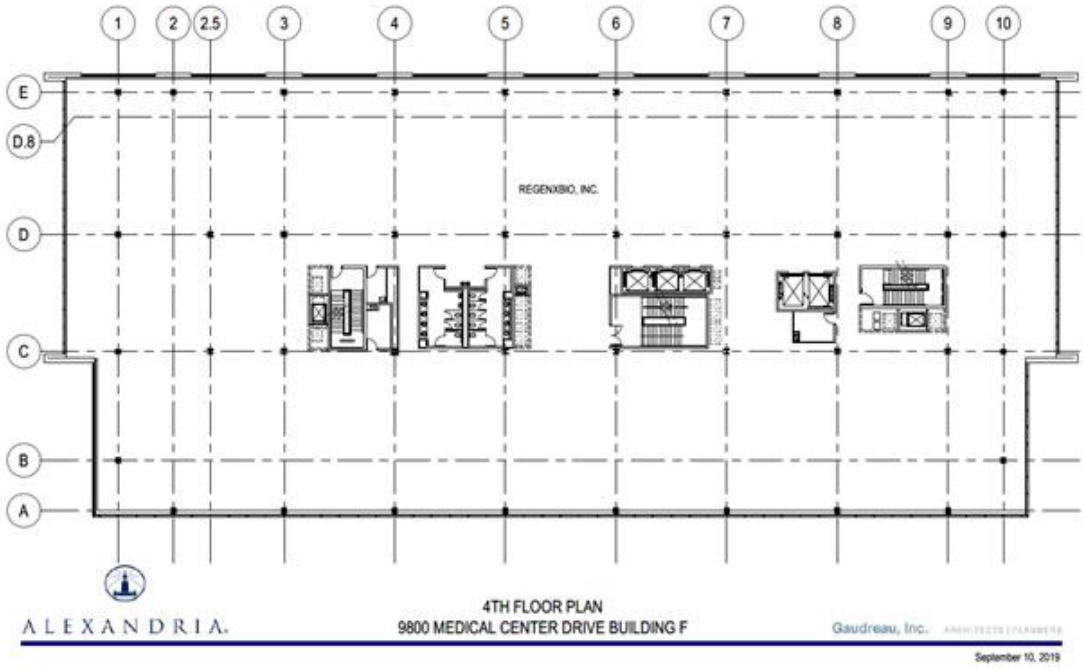


EXHIBIT A
DESCRIPTION OF PREMISES—continued

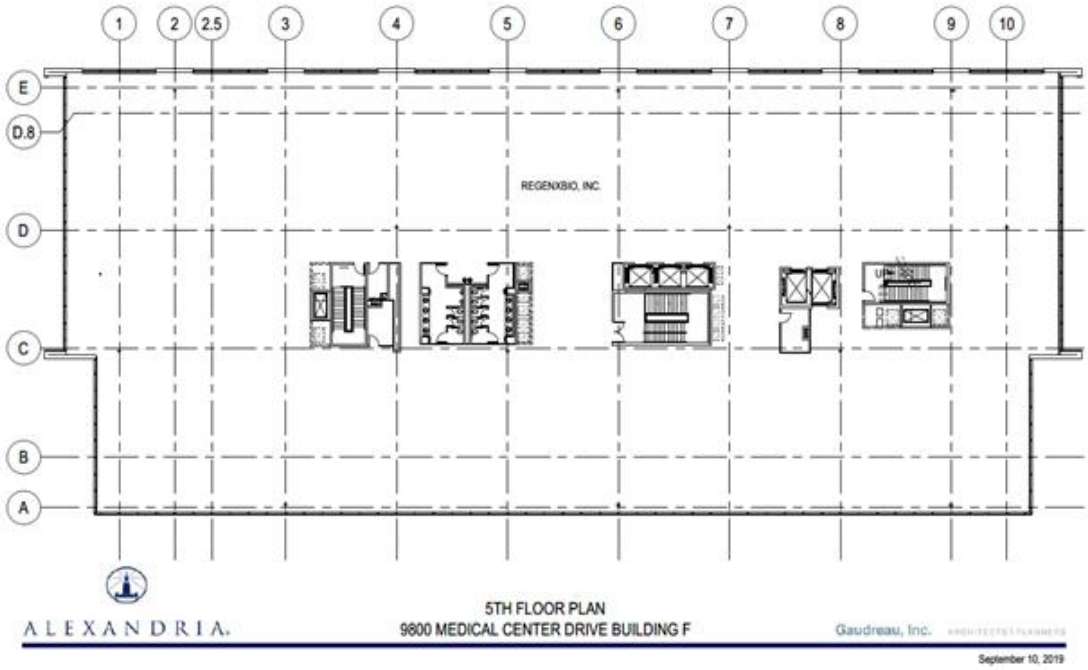


EXHIBIT D TO LEASE
ACKNOWLEDGMENT OF COMMENCEMENT DATE

This **ACKNOWLEDGMENT OF COMMENCEMENT DATE** is made as of this _____ day of _____, 201__, between **ARE-MARYLAND NO. 24, LLC**, a Delaware limited liability company ("**Landlord**"), and **REGENXBIO INC.**, a Delaware corporation ("**Tenant**"), and is attached to and made a part of the Lease Agreement dated as of November 1, 2018 (as amended, the "**Lease**"), by and between Landlord and Tenant. Any initially capitalized terms used but not defined herein shall have the meanings given them in the Lease.

Landlord and Tenant hereby acknowledge and agree that the Commencement Date of the Base Term of the Lease is November 1, 2018, the Lease Commencement Date is _____, the Rent Commencement Date (subject to the Base Rent Abatement) is _____, the 4th Floor Rent Commencement Date is _____, the First Floor/Second Floor Expansion Premises Rent Commencement Date is _____, and the expiration date of the Base Term of the Lease shall be midnight on _____. In case of a conflict between the terms of the Lease and the terms of this Acknowledgement of Commencement Date, this Acknowledgement of Commencement Date shall control for all purposes.

IN WITNESS WHEREOF, Landlord and Tenant have executed this ACKNOWLEDGMENT OF COMMENCEMENT DATE under seal to be effective on the date first above written.

TENANT:

REGENXBIO INC.,
a Delaware corporation

By:_____(SEAL)
Name:_____
Title:_____

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: _____(SEAL)
Name:_____
Title:_____

EXHIBIT H TO LEASE
LOADING DOCKS AND DEDICATED GENERATOR AREA

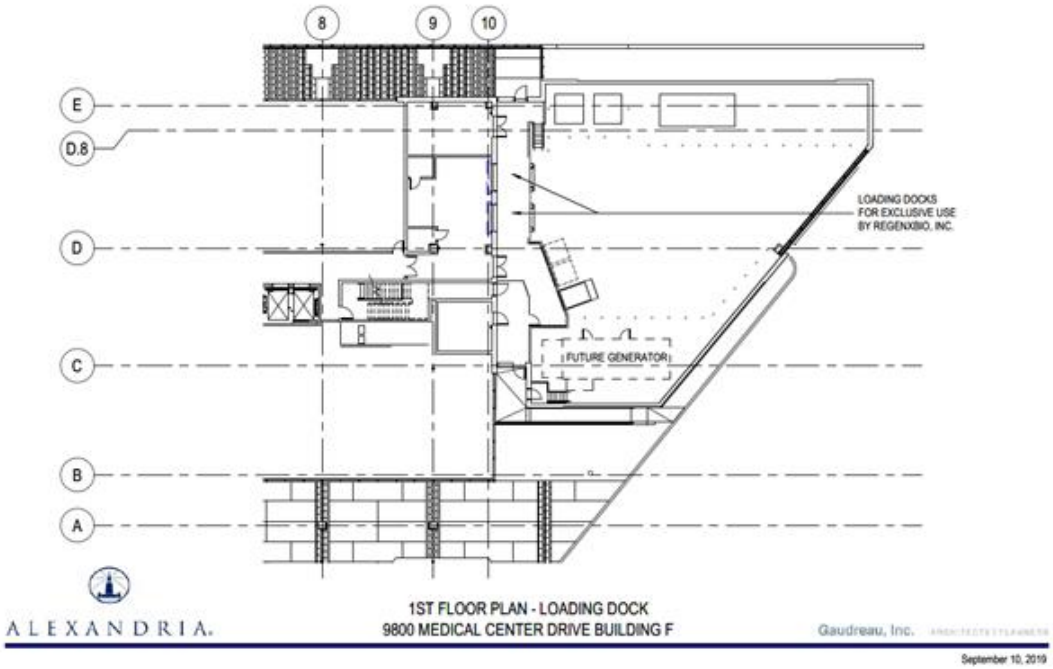


EXHIBIT J TO LEASE
ROOF SPACE

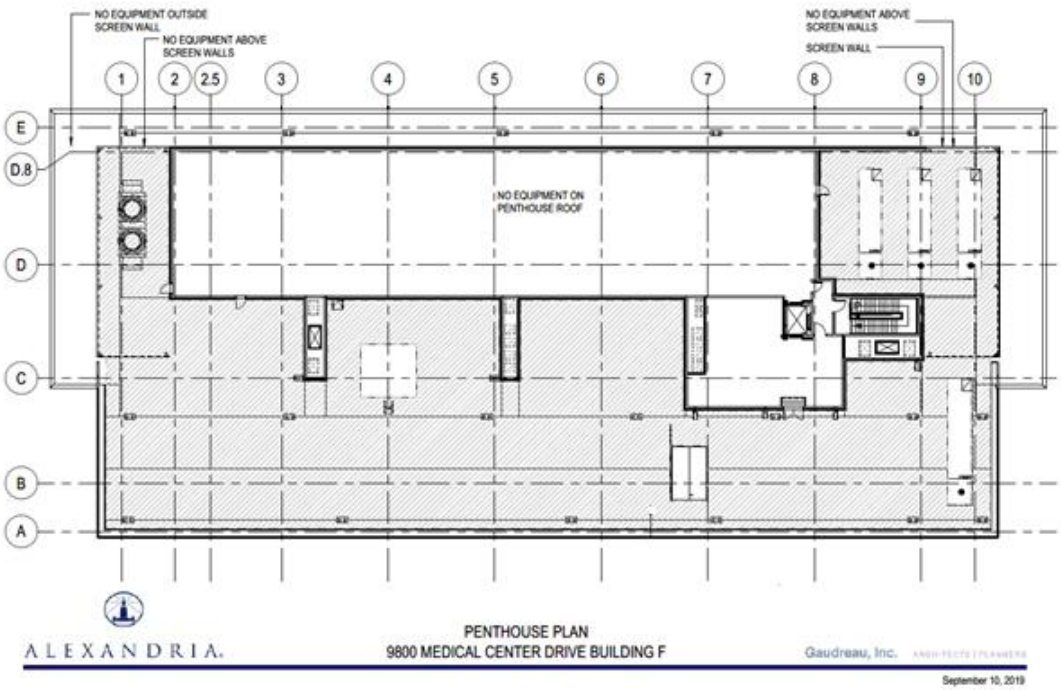


EXHIBIT K TO LEASE
EQUIPMENT YARD

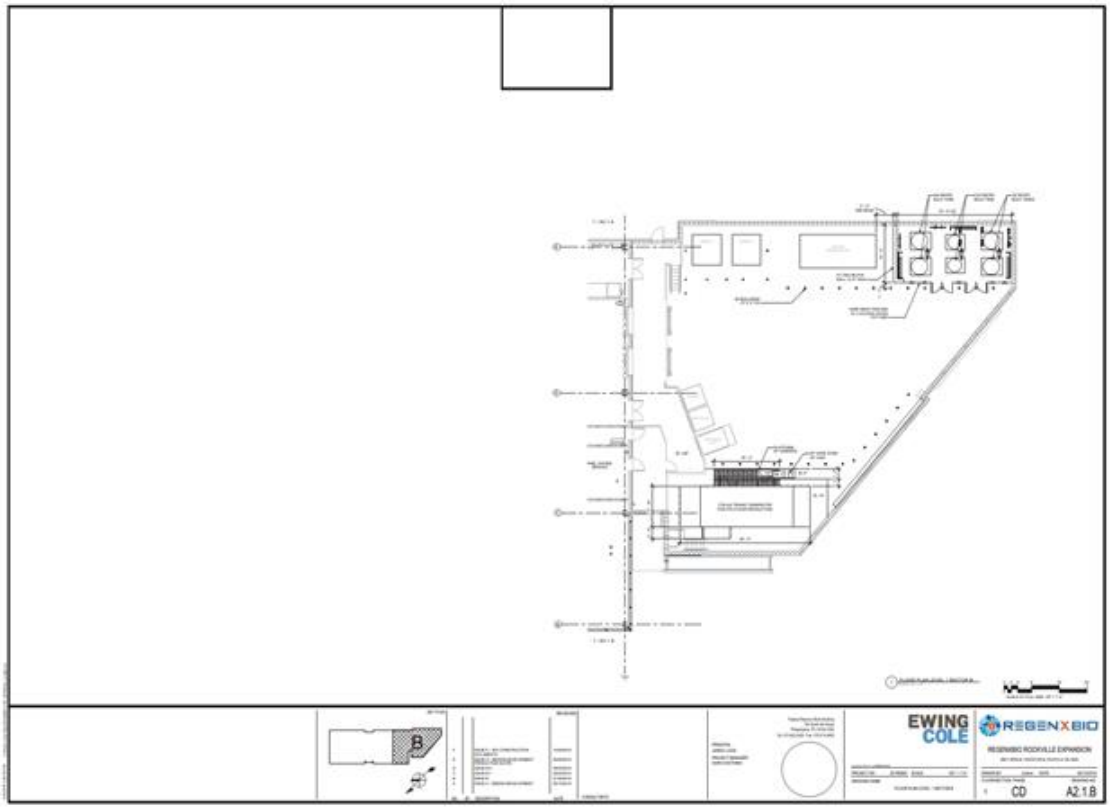


EXHIBIT 31.1

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 5, 2019

/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 5, 2019

/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, 2019 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 5, 2019

/s/ Kenneth T. Mills

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

Date: November 5, 2019

/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.