

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
Washington, D.C. 20549
FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 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
(State or other jurisdiction of
incorporation or organization)

9600 Blackwell Road, Suite 210
Rockville, MD
(Address of principal executive offices)

47-1851754
(I.R.S. Employer
Identification No.)

20850
(Zip Code)

(240) 552-8181

(Registrant's telephone number, including area code)

Not Applicable

(Former name, former address and former fiscal year, if changed since last report)

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	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

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

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

As of May 3, 2019, there were 36,636,825 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 MARCH 31, 2019

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INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “assume,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would” or by variations of such words or by similar expressions. We have based these forward-looking statements on our current expectations and assumptions and analyses made by us in light of our experience and our perception of historical trends, current conditions and expected future developments, as well as other factors we believe are appropriate under the circumstances. However, whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, uncertainties, assumptions and other important factors, including, but not limited to:

- the timing of enrollment, commencement and completion and the success of our clinical trials;
- the timing of commencement and completion and the success of preclinical studies conducted by us and our development partners;
- the timely development and launch of new products;
- the 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 and the rules of the SEC, we do not undertake any obligation, and specifically decline any obligation, to update or revise 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)

	March 31, 2019	December 31, 2018
Assets		
Current assets		
Cash and cash equivalents	\$ 55,852	\$ 75,561
Marketable securities	229,373	244,200
Accounts receivable	8,372	8,587
Prepaid expenses	6,292	5,734
Other current assets	3,995	3,831
Total current assets	303,884	337,913
Marketable securities	159,083	150,819
Accounts receivable	22,758	23,012
Property and equipment, net	23,140	28,702
Operating lease right-of-use assets	6,858	—
Restricted cash	1,053	1,053
Other assets	2,255	2,315
Total assets	<u>\$ 519,031</u>	<u>\$ 543,814</u>
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 4,204	\$ 4,412
Accrued expenses and other current liabilities	14,189	17,164
Deferred revenue	600	600
Operating lease liabilities	2,397	—
Total current liabilities	21,390	22,176
Deferred revenue	3,333	3,333
Operating lease liabilities	5,483	—
Deferred rent	—	1,098
Financing lease obligations	—	5,854
Other liabilities	1,772	2,505
Total liabilities	31,978	34,966
Commitments and contingencies (Note 6)		
Stockholders' equity		
Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at March 31, 2019 and December 31, 2018	—	—
Common stock; \$0.0001 par value; 100,000 shares authorized at March 31, 2019 and December 31, 2018; 36,611 and 36,120 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	602,425	592,580
Accumulated other comprehensive loss	(59)	(720)
Accumulated deficit	(115,317)	(83,016)
Total stockholders' equity	487,053	508,848
Total liabilities and stockholders' equity	<u>\$ 519,031</u>	<u>\$ 543,814</u>

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 March 31.	
	2019	2018
Revenues		
License revenue	\$ 884	\$ 132,391
Total revenues	884	132,391
Operating Expenses		
Costs of revenues		
Licensing costs	29	2,408
Research and development	25,203	19,550
General and administrative	11,558	8,380
Other operating expenses	—	28
Total operating expenses	36,790	30,366
Income (loss) from operations	(35,906)	102,025
Other Income		
Interest income from licensing	613	1,355
Investment income	2,995	859
Total other income	3,608	2,214
Income (loss) before income taxes	(32,298)	104,239
Income Tax Benefit		
Net income (loss)	\$ (32,228)	\$ 104,239
Other Comprehensive Income (Loss)		
Unrealized gain (loss) on available-for-sale securities, net of reclassifications and income tax expense	621	(188)
Total other comprehensive income (loss)	621	(188)
Comprehensive income (loss)	\$ (31,607)	\$ 104,051
Net income (loss) applicable to common stockholders	\$ (32,228)	\$ 104,239
Net income (loss) per share:		
Basic	\$ (0.89)	\$ 3.30
Diluted	\$ (0.89)	\$ 3.04
Weighted-average common shares outstanding:		
Basic	36,366	31,632
Diluted	36,366	34,275

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

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

	Three Months Ended March 31, 2019					
	Common Stock		Additional Paid-in Capital	Accumulated Other Comprehensive 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	481	—	3,762	—	—	3,762
Issuance of common stock under employee stock purchase plan	10	—	365	—	—	365
Stock-based compensation expense	—	—	5,718	—	—	5,718
Unrealized gain on available-for-sale securities, net of reclassifications and income tax expense	—	—	—	621	—	621
Net loss	—	—	—	—	(32,228)	(32,228)
Balances at March 31, 2019	<u>36,611</u>	<u>\$ 4</u>	<u>\$ 602,425</u>	<u>\$ (59)</u>	<u>\$ (115,317)</u>	<u>\$ 487,053</u>

	Three Months Ended March 31, 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
Exercise of stock options	586	—	3,824	—	—	3,824
Issuance of common stock under employee stock purchase plan	20	—	342	—	—	342
Stock-based compensation expense	—	—	3,291	—	—	3,291
Unrealized loss on available-for-sale securities, net of reclassifications and income tax expense	—	—	—	(188)	—	(188)
Net income	—	—	—	—	104,239	104,239
Balances at March 31, 2018	<u>31,900</u>	<u>\$ 3</u>	<u>\$ 378,954</u>	<u>\$ (903)</u>	<u>\$ (78,714)</u>	<u>\$ 299,340</u>

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

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

	Three Months Ended March 31,	
	2019	2018
Cash flows from operating activities		
Net income (loss)	\$ (32,228)	\$ 104,239
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities		
Stock-based compensation expense	5,718	3,291
Net amortization of premiums and accretion of discounts on marketable debt securities	(368)	378
Depreciation and amortization	1,614	834
Imputed interest income from licensing	(613)	(1,355)
Other non-cash adjustments	327	10
Changes in operating assets and liabilities		
Accounts receivable	591	(51,414)
Prepaid expenses	(790)	667
Other current assets	(295)	(446)
Operating lease right-of-use assets	573	—
Other assets	26	(21)
Accounts payable	316	477
Accrued expenses and other current liabilities	(2,932)	490
Operating lease liabilities	(592)	—
Deferred rent	—	27
Other liabilities	(644)	957
Net cash provided by (used in) operating activities	(29,297)	58,134
Cash flows from investing activities		
Purchases of marketable securities	(79,249)	(54,267)
Maturities of marketable securities	87,165	19,525
Purchases of property and equipment	(2,455)	(2,344)
Net cash provided by (used in) investing activities	5,461	(37,086)
Cash flows from financing activities		
Proceeds from exercise of stock options	3,762	3,824
Proceeds from issuance of common stock under employee stock purchase plan	365	342
Net cash provided by financing activities	4,127	4,166
Net increase (decrease) in cash and cash equivalents and restricted cash	(19,709)	25,214
Cash and cash equivalents and restricted cash		
Beginning of period	76,614	46,881
End of period	\$ 56,905	\$ 72,095

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 product candidates for a variety of diseases with unmet needs. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

Liquidity and Risks

As of March 31, 2019, the Company had generated an accumulated deficit of \$115.3 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 March 31, 2019, the Company had cash, cash equivalents and marketable securities of \$444.3 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 include all normal and recurring adjustments necessary for the fair statement of the Company's financial position as of March 31, 2019, and the results of its operations and its cash flows for the interim periods ended March 31, 2019 and 2018.

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: revenue, stock-based compensation expense, accrued research and development expenses and other accrued liabilities, income taxes and the fair value of financial instruments.

Restricted Cash

Restricted cash includes money market mutual funds used to collateralize irrevocable letters of credit as required by the Company's lease agreements. The following table provides a reconciliation of cash and cash equivalents and restricted cash as reported on the consolidated balance sheets to the total of these amounts as reported at the end of the period in the consolidated statements of cash flows (in thousands):

	March 31, 2019	March 31, 2018
Cash and cash equivalents	\$ 55,852	\$ 71,870
Restricted cash	1,053	225
Total cash and cash equivalents and restricted cash	<u>\$ 56,905</u>	<u>\$ 72,095</u>

Marketable Securities

Marketable securities consist of debt securities and are classified as available-for-sale and carried at fair value. Marketable 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. Unrealized gains and losses, 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 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. 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.

A decline in the fair value below cost of available-for-sale 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 investments 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 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 applies a dual approach to all leases in which it is a lessee and classifies 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 March 31, 2019 have been classified as operating leases. Operating lease expense is recognized on a straight-line basis over the term of the lease. Variable lease expense is 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.

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. 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 months ended March 31, 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 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 current 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 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 2018-13, *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 the Company's 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 the Company's financial position or results of operations.

3. Marketable Securities

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

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
March 31, 2019				
U.S. government and federal agency securities	\$ 105,533	\$ 202	\$ (3)	\$ 105,732
Certificates of deposit	9,237	42	—	9,279
Corporate bonds	272,988	502	(45)	273,445
	<u>\$ 387,758</u>	<u>\$ 746</u>	<u>\$ (48)</u>	<u>\$ 388,456</u>
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
	<u>\$ 395,304</u>	<u>\$ 129</u>	<u>\$ (414)</u>	<u>\$ 395,019</u>

As of March 31, 2019 and December 31, 2018, no available-for-sale securities had remaining maturities greater than three years.

The amortized cost of available-for-sale securities is adjusted for amortization of premiums and accretion of discounts to maturity, or to the earliest call date for callable debt securities purchased at a premium. As of March 31, 2019 and December 31, 2018, the balance in the Company's accumulated other comprehensive loss consisted solely of net unrealized gains and losses on available-for-sale securities, net of income tax effects and reclassification adjustments for realized gains and losses.

During the three months ended March 31, 2019, the Company recognized net unrealized gains on available-for-sale securities of \$1.0 million and income tax expense of \$0.4 million in other comprehensive income for the period. The Company recognized net realized gains of less than \$0.1 million on the sale or maturity of available-for-sale securities during the three months ended March 31, 2019, which were reclassified out of accumulated other comprehensive loss during the period and are included in investment income in the consolidated statements of operations and comprehensive income (loss). During the three months ended March 31, 2018, the Company recognized net unrealized losses on available-for-sale securities of \$0.2 million and income tax expense of zero in other comprehensive loss for the period. The Company did not recognize any realized gains or losses on the sale or maturity of available-for-sale securities during the three months ended March 31, 2018. Realized gains and losses from the sale or maturity of marketable securities are determined based on the specific identification method.

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

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
March 31, 2019						
U.S. government and federal agency securities	\$ 13,923	\$ (3)	\$ —	\$ —	\$ 13,923	\$ (3)
Corporate bonds	110,103	(44)	1,999	(1)	112,102	(45)
	<u>\$ 124,026</u>	<u>\$ (47)</u>	<u>\$ 1,999</u>	<u>\$ (1)</u>	<u>\$ 126,025</u>	<u>\$ (48)</u>
	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)
	<u>\$ 298,407</u>	<u>\$ (391)</u>	<u>\$ 12,424</u>	<u>\$ (23)</u>	<u>\$ 310,831</u>	<u>\$ (414)</u>

As of March 31, 2019, marketable securities held by the Company which were in an unrealized loss position consisted of 40 investment grade security positions. The Company has the intent and ability to hold such securities until recovery and has determined that none of its investments were other-than-temporarily impaired as of March 31, 2019 or December 31, 2018.

4. Fair Value of Financial Instruments

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

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
March 31, 2019				
Cash equivalents:				
Money market mutual funds	\$ —	\$ 55,834	\$ —	\$ 55,834
Total cash equivalents	—	55,834	—	55,834
Marketable securities:				
U.S. government and federal agency securities	—	105,732	—	105,732
Certificates of deposit	—	9,279	—	9,279
Corporate bonds	—	273,445	—	273,445
Total marketable securities	—	388,456	—	388,456
Total cash equivalents and marketable securities	<u>\$ —</u>	<u>\$ 444,290</u>	<u>\$ —</u>	<u>\$ 444,290</u>

	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 three months ended March 31, 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 March 31, 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.

The Company's non-marketable equity securities are measured at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. As of March 31, 2019 and December 31, 2018, non-marketable equity securities had carrying values of \$0.4 million and were included in other assets on the consolidated balance sheets. Since the acquisition of the securities, the Company has not identified any observable price changes or changes in circumstances that would have had an adverse effect on the fair value of the securities as of March 31, 2019 and December 31, 2018. No remeasurements or impairment losses were recorded on non-marketable equity securities during the three months ended March 31, 2019 and 2018.

5. Property and Equipment, Net

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

	March 31, 2019	December 31, 2018
Lab equipment	\$ 15,563	\$ 14,417
Computer equipment and software	2,104	2,002
Furniture and fixtures	1,935	1,915
Leasehold improvements	12,371	11,751
Construction-in-progress	—	5,854
Total property and equipment	31,973	35,939
Accumulated depreciation and amortization	(8,833)	(7,237)
Property and equipment, net	\$ 23,140	\$ 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 agreement for the facility at 9800 Medical Center Drive.

6. Commitments and Contingencies

Lease Commitments

9800 Medical Center Drive Lease

In November 2018, the Company entered into a lease for approximately 132,000 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). Construction of the new building, which will be conducted by the landlord, is expected to be completed in 2020 and the lease will expire approximately 16 years from the delivery of the leased premises to the Company, subject to certain extension and termination options. Under the original terms of the 9800 Medical Center Drive Lease, which was amended in April 2019 as discussed further below, the Company was entitled to receive a \$14.6 million tenant improvement allowance from the landlord to construct additional improvements to the leased premises. 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 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 the 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 March 31, 2019, the Company had incurred \$0.3 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. In April 2019, the Company agreed to pay \$4.0 million to the landlord to fund certain costs of construction related to material changes in the design and construction of the building requested by the Company.

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 as of March 31, 2019 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 facility to the Company.

In April 2019, the Company amended the 9800 Medical Center Drive Lease to expand the leased premises to include an additional 5,975 square feet of the building over the term of the lease. As a result of the amendment, the total amount of future rent under the 9800 Medical Center Drive Lease was increased by \$4.0 million and the Company's tenant improvement allowance under the lease was increased to \$15.3 million.

Other Lease Commitments

In March 2015, the Company entered into a non-cancelable 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 a 7.5-year, non-cancelable 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. 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. In November 2017, the Blackwell Road Lease was amended to include additional office space for the remainder of the lease term. Monthly payments under the lease began in September 2016 and escalate annually in accordance with the lease agreement. The Company received a \$0.8 million tenant improvement allowance from the landlord which has been recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of rent expense over the term of the lease.

The Company leases additional office and laboratory facilities in Rockville, Maryland and New York, New York, as well as laboratory and other equipment, under non-cancelable operating leases with various expiration dates through 2022 and which may contain annual escalations of rental payments. As required by the Company's lease agreement for its office space in New York, New York, 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.

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 operating leases (in thousands):

	Three Months Ended March 31, 2019	
Operating lease cost	\$	700
Variable lease cost		161
Total lease cost	\$	861
Cash paid for amounts included in the measurement of operating lease liabilities	\$	711
Right-of-use assets acquired through operating lease liabilities	\$	36

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 March 31, 2019	
Weighted-average remaining lease term (years)		3.1
Weighted-average discount rate		5.6%

The following table presents a reconciliation of the undiscounted future minimum lease payments remaining under the 9800 Medical Center Drive Lease and other operating leases to the amounts reported as operating lease liabilities on the consolidated balance sheet as of March 31, 2019 (in thousands):

	9800 Medical Center Drive Lease (a)	Other Operating Leases	Total Undiscounted Fixed Payments
Undiscounted future minimum lease payments:			
2019 (remainder of year)	\$ —	\$ 1,995	\$ 1,995
2020	—	3,088	3,088
2021	1,332	2,412	3,744
2022	4,308	623	4,931
2023	5,188	479	5,667
Thereafter	76,892	—	76,892
Total undiscounted future minimum lease payments	<u>\$ 87,720</u>	<u>\$ 8,597</u>	<u>\$ 96,317</u>
Amount representing imputed interest		(717)	
Total operating lease liabilities		7,880	
Current portion of operating lease liabilities		(2,397)	
Operating lease liabilities, non-current		<u>\$ 5,483</u>	

(a) Undiscounted future minimum lease payments under the 9800 Medical Center Drive Lease are not included in the lease liabilities reported on the consolidated balance sheet as of March 31, 2019 as the lease has not yet commenced. The actual timing and amounts of payments under the 9800 Medical Center Drive Lease are subject to adjustment based on the completion date of construction and actual square footage of the facility constructed. Accordingly, these amounts were estimates as of March 31, 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	<u>\$ 87,194</u>	<u>\$ 9,343</u>	<u>\$ 96,537</u>

(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 completion date of construction and actual square footage of the facility constructed. Accordingly, these amounts were estimates as of December 31, 2018.

License from 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). In consideration for the license, the Company issued Penn an equity interest in 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 is obligated to pay Penn an upfront fee, 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.

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 March 31, 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.

7. License Revenue

As of March 31, 2019, the Company's NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by 11 NAV Technology Licensees. 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. To date the Company has not recognized any revenue from the achievement of sales-based milestones or royalties on sales of licensed products.

Development milestone payments are only included in the transaction price of each license and recognized as 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 revenue in the period of achievement. As of March 31, 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 \$29.6 million upon the commencement of various stages of clinical trials, \$47.5 million upon the submission of regulatory approval filings, \$117.5 million upon the approval of commercial products by regulatory agencies and \$232.0 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 March 31, 2019			
Receivables and contract assets:			
Accounts receivable, current and non-current	\$ 31,599	\$ (469)	\$ 31,130
Contract assets	\$ 750	\$ 250	\$ 1,000
Contract liabilities:			
Deferred revenue, current and non-current	\$ 3,933	\$ —	\$ 3,933

	Balance at Beginning of Period	Net Additions (Deductions)	Balance at End of Period
Three Months Ended March 31, 2018			
Receivables and contract assets:			
Accounts receivable, current and non-current	\$ 5,850	\$ 52,762	\$ 58,612
Contract assets	\$ 350	\$ (100)	\$ 250
Contract liabilities:			
Deferred revenue, current and non-current	\$ —	\$ —	\$ —

The net increase in the balance of accounts receivable during the three months ended March 31, 2018 was primarily attributable to the January 2018 amendment to the license agreement with AveXis, Inc. (AveXis) for the development and commercialization of treatments for spinal muscular atrophy (SMA). As of March 31, 2018, the Company had recorded accounts receivable, current and non-current, of \$53.5 million related to the amended license agreement with AveXis.

As of March 31, 2019, the Company had recorded deferred revenue, current and non-current, of \$3.9 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 months ended March 31, 2019 and 2018, the Company recognized license revenue of \$0.8 million and \$0.3 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 price were primarily attributable to development milestones achieved or deemed probable of achievement during the period that were previously not considered probable of achievement.

As of March 31, 2019, the Company had recorded total current and non-current accounts receivable of \$31.1 million, of which \$0.1 million had been billed to customers and \$31.0 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 March 31, 2019 and December 2018 included \$26.7 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 March 31, 2019 and December 31, 2018, the Company had not recognized any impairment losses on its receivables or contract assets from contracts with customers.

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 AG (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.

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 March 31, 2019, the transaction price of the original March 2014 License was \$7.5 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 \$7.0 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, if any.

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 March 31, 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, if any.

During the three months ended March 31, 2019 and 2018, the Company recognized license revenue of zero and \$132.1 million, respectively, and interest income from licensing of less than \$0.1 million and \$1.2 million, respectively, from the March 2014 License, as amended, which includes amounts from both the original March 2014 License and the January 2018 Amendment. As of March 31, 2019 and 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 on the consolidated balance sheets.

8. 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 March 31, 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 2,583,144 remained available for future grants under the 2015 Plan.

Stock-based Compensation Expense

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

	Three Months Ended March 31,	
	2019	2018
Stock options	\$ 5,452	\$ 3,122
Restricted stock units	68	68
Employee stock purchase plan	198	101
	<u>\$ 5,718</u>	<u>\$ 3,291</u>

As of March 31, 2019, the Company had \$68.9 million of unrecognized stock-based compensation expense related to stock options, restricted stock units and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 3.1 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 March 31,	
	2019	2018
Research and development	\$ 2,347	\$ 1,530
General and administrative	3,371	1,761
	<u>\$ 5,718</u>	<u>\$ 3,291</u>

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	1,102	\$ 48.22		
Exercised	(480)	\$ 7.81		
Cancelled or forfeited	(137)	\$ 30.72		
Outstanding at March 31, 2019	<u>5,340</u>	\$ 26.02	7.9	\$ 170,518
Exercisable at March 31, 2019	<u>2,491</u>	\$ 12.22	6.8	\$ 112,324
Vested and expected to vest at March 31, 2019	<u>5,340</u>	\$ 26.02	7.9	\$ 170,518

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

The weighted-average grant date fair value per share of options granted during the three months ended March 31, 2019 was \$32.20. During the three months ended March 31, 2019, the total number of stock options exercised was 480,320, resulting in total proceeds of \$3.8 million. The total intrinsic value of options exercised during the three months ended March 31, 2019 was \$20.0 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 March 31, 2019	<u>40</u>	<u>\$ 20.90</u>

Employee Stock Purchase Plan

As of March 31, 2019, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 254,000, of which 159,339 remained available for future issuance. During the three months ended March 31, 2019, 10,241 shares of common stock were issued under the 2015 ESPP.

9. Income Taxes

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, including a three-year cumulative loss position as of March 31, 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 March 31, 2019 and December 31, 2018.

During the three months ended March 31, 2019, the Company recognized income tax benefit of \$0.1 million and income tax expense in other comprehensive income of \$0.4 million related to net unrealized gains on available-for-sale securities during the period. As of March 31, 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 sheets.

10. Related Party Transactions

FO XKISER LLP

Since 2016, the Company has been party to professional services agreements with FO XKISER LLP (FO XKISER), 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 FO XKISER. Effective January 2019, the Company entered into a new professional services agreement with FO XKISER with similar terms and conditions as the previous agreements and which has a term of one year and is terminable by either party. Expenses incurred under the agreements with FO XKISER for the three months ended March 31, 2019 and 2018 were \$0.8 million and \$0.5 million, respectively, and were recorded as research and development expenses in the consolidated statements of operations and comprehensive income (loss).

11. Net Income (Loss) Per Share

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

	Three Months Ended March 31,	
	2019	2018
Basic net income (loss) per share:		
Net income (loss) applicable to common stockholders	\$ (32,228)	\$ 104,239
Shares used in computation:		
Weighted-average common shares outstanding	36,366	31,632
Basic net income (loss) per share	<u>\$ (0.89)</u>	<u>\$ 3.30</u>
Diluted net income (loss) per share:		
Net income (loss) applicable to common stockholders	\$ (32,228)	\$ 104,239
Shares used in computation:		
Weighted-average common shares outstanding	36,366	31,632
Stock options	—	2,620
Restricted stock units	—	22
Employee stock purchase plan	—	1
Weighted-average diluted common shares	<u>36,366</u>	<u>34,275</u>
Diluted net income (loss) per share	<u>\$ (0.89)</u>	<u>\$ 3.04</u>

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 March 31,	
	2019	2018
Stock options issued and outstanding	5,340	1,582
Unvested restricted stock units outstanding	40	—
Employee stock purchase plan	20	—
	<u>5,400</u>	<u>1,582</u>

12. Supplemental Disclosures

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

	March 31, 2019	December 31, 2018
Accrued external research and development expenses	\$ 5,474	\$ 4,274
Accrued personnel costs	5,357	9,484
Accrued income taxes payable	1,134	726
Accrued licensing costs	911	1,617
Accrued external general and administrative expenses	854	773
Accrued purchases of property and equipment	191	221
Other accrued expenses and current liabilities	268	69
	<u>\$ 14,189</u>	<u>\$ 17,164</u>

Other liabilities of \$1.8 million and \$2.5 million reported as of March 31, 2019 and December 31, 2018, respectively, consist of accrued licensing costs payable 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 across the therapeutic areas of retinal, metabolic and neurodegenerative 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. We began enrollment in the Phase I/IIa clinical trial for RGX-314 for the treatment of wet AMD in May 2017, and as of April 18, 2019, we have completed dosing of 33 total subjects in five cohorts, including six subjects in each of the first three cohorts, 12 subjects in the fourth cohort and 3 subjects in the fifth cohort. We expect to initiate a Phase IIb trial in late 2019. Additionally, we expect to file an investigational new drug (IND) application with the U.S. Food and Drug Administration (the FDA) for a Phase II trial to evaluate RGX-314 for the treatment of diabetic retinopathy in the second half of 2019.
- **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. We have begun dosing subjects in the Phase I/II clinical trial for RGX-121 and we expect to continue enrollment and site activation in 2019.
- **RGX-111:** We are developing RGX-111 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type I (MPS I), a severe genetic lysosomal storage disease caused by deficiency of α -L-iduronidase (IDUA), an enzyme required for breakdown of cellular waste products. Recruitment and additional site activations are ongoing in the Phase I clinical trial for RGX-111.
- **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. Enrollment in the Phase I/II clinical trial for RGX-501 began in March 2017. We have completed dosing of the first cohort of three subjects and have dosed three subjects in the second cohort, for a total of six subjects. We have amended the clinical trial protocol to include steroid prophylaxis and screening has re-initiated in the Phase I/II clinical trial.
- **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 submit an IND application, or foreign equivalent, for RGX-181 for the treatment of CLN2 to the FDA, or a foreign regulatory authority, by the end of 2019 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 Interim Data Update

In our Phase I/IIa trial for RGX-314, as of April 18, 2019, 33 subjects with wet AMD have received a single administration of RGX-314 across five dose cohorts (six subjects in each of the first three cohorts, 12 subjects in the fourth cohort and three subjects currently in the fifth cohort). 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 have been 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 April 18, 2019:

- RGX-314 continues to be well-tolerated across all cohorts, with no drug-related serious adverse events (SAEs) reported.
- Sustained and durable RGX-314 intraocular protein expression was detected at one year in all subjects in Cohort 3 as measured by electrochemiluminescence immunoassay (ECL) after administration of RGX-314.
- 50% of subjects (3/6) in Cohort 3 continue to remain injection-free through one year with durable protein and clinical effect observed on best corrected visual acuity (BCVA) and central retinal thickness (CRT). Mean BCVA improved by +10 letters and mean CRT decreased by 59 μm from baseline in these subjects at one year. Evidence of durable protein expression was also observed (see Table 1).
- All subjects in Cohort 3 show a mean BCVA improvement of +5 letters and mean CRT decrease by 39 μm from baseline at one year. Cohort 3 subjects received a low number of anti-VEGF injections following the administration of RGX-314, with a mean of 2.3 injections over one year.

Table 1: RGX-314 Protein Expression Levels (ng/ml) of Cohort 3 Subjects with No Additional Anti-VEGF Injections through One Year (N=3)

Visit	1 month	6 months	1 year
Mean Protein Level (ng/ml)	236.2	274.9	260.5

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively sublicense our proprietary adeno-associated virus (AAV) gene therapy delivery platform (NAV Technology Platform) to other leading biotechnology companies, which we refer to as NAV Technology Licensees. As of March 31, 2019, our NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by our NAV Technology Licensees. Sublicensing 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

To date, we have primarily generated revenues through the licensing of our NAV Technology Platform to NAV Technology Licensees. We have not generated any revenues from the sale of approved 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. As of March 31, 2019, our NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by 11 NAV Technology Licensees. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicenseable 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. To date we have not recognized any revenue from the achievement of sales-based milestones or royalties on sales of licensed products.

Future license revenue is highly dependent on the successful development and commercialization of licensed products by our licensees, which is uncertain, and revenue 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 license revenue is 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 license revenue we recognize in future periods.

Operating Expenses

Our operating expenses consist primarily of costs of revenue, research and development 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.

Costs of Revenue

Costs of revenue consist primarily of sublicense fees to licensors as a result of revenues generated from the licensing of our NAV Technology Platform. Sublicense fees are based on a percentage of license fees we receive from licensees as specified in the agreements with our licensors. We recognize sublicense fees in the period that the underlying license revenue is recognized. Future costs of revenue are uncertain due to the nature of our license agreements and significant fluctuations in costs of revenue 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 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-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;
- a Phase I/II clinical trial to evaluate the safety and efficacy of our RGX-501 program for the treatment of HoFH;
- preclinical research and development and a planned clinical trial for our RGX-181 program for the treatment of CLN2;
- preclinical research and development for additional product candidates addressing other diseases in the retinal, metabolic and neurodegenerative 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 months ended March 31, 2019 and 2018 (in thousands):

	Three Months Ended March 31,	
	2019	2018
Direct Expenses		
RGX-314	\$ 2,404	\$ 1,620
RGX-121	1,774	958
RGX-111	841	1,233
RGX-501	1,000	4,149
RGX-181	2,296	—
Total direct expenses	8,315	7,960
Unallocated Expenses		
Unallocated external expenses	3,396	2,616
Personnel-related	10,574	7,371
Facilities and depreciation expense	2,205	1,221
Other unallocated	713	382
Total unallocated expenses	16,888	11,590
Total research and development	\$ 25,203	\$ 19,550

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

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. 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 fixed income debt 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. There have been no significant changes in our critical accounting policies since December 31, 2018.

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 Accounting Standards Codification (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. 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 months ended March 31, 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 March 31,		Change
	2019	2018	
	(in thousands)		
Revenues			
License revenue	\$ 884	\$ 132,391	\$ (131,507)
Total revenues	884	132,391	(131,507)
Operating Expenses			
Costs of revenues			
Licensing costs	29	2,408	(2,379)
Research and development	25,203	19,550	5,653
General and administrative	11,558	8,380	3,178
Other operating expenses	—	28	(28)
Total operating expenses	36,790	30,366	6,424
Income (loss) from operations	(35,906)	102,025	(137,931)
Other Income			
Interest income from licensing	613	1,355	(742)
Investment income	2,995	859	2,136
Total other income	3,608	2,214	1,394
Income (loss) before income taxes	(32,298)	104,239	(136,537)
Income Tax Benefit	70	—	70
Net income (loss)	\$ (32,228)	\$ 104,239	\$ (136,467)

Comparison of the Three Months Ended March 31, 2019 and 2018

License Revenue. License revenue decreased by \$131.5 million, from \$132.4 million for the three months ended March 31, 2018 to \$0.9 million for the three months ended March 31, 2019. The decrease was primarily attributable to \$132.1 million of non-recurring revenue that we recognized during the three months ended March 31, 2018 under the January 2018 amendment to our license agreement with AveXis, Inc. (AveXis) for the development and commercialization of treatments for spinal muscular atrophy (SMA). The decrease in license revenue during the three months ended March 31, 2019 also resulted in a \$2.4 million decrease in licensing costs incurred during the period related to the sublicense fees that we are obligated to pay to our licensors.

Novartis AG (Novartis), which acquired AveXis in May 2018, has announced that it expects to launch Zolgensma® in 2019, pending approval by regulatory authorities. If approved, Zolgensma will be the first approved product under our amended license agreement with AveXis for the development and commercialization of treatments for SMA. Upon its approval and launch, we will begin recognizing royalty revenue on net sales of Zolgensma.

Research and Development Expense. Research and development expenses increased by \$5.7 million, from \$19.6 million for the three months ended March 31, 2018 to \$25.2 million for the three months ended March 31, 2019. The increase was primarily attributable to the following:

- an increase of \$3.2 million for personnel costs as a result of increased headcount of research and development personnel, including a \$0.8 million increase in stock-based compensation expense;
- an increase of \$1.5 million for laboratory costs and facilities used by research and development personnel, including a \$0.8 million increase in depreciation expense allocated to research and development functions; and
- an increase of \$0.4 million for external costs associated with clinical trial activities for our lead product candidates, process development and manufacturing-related services and preclinical research and development.

General and Administrative Expense. General and administrative expenses increased by \$3.2 million, from \$8.4 million for the three months ended March 31, 2018 to \$11.6 million for the three months ended March 31, 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 \$1.6 million increase in stock-based compensation expense; and
- an increase of \$1.0 million for professional services, including commercial, legal, accounting and other advisory services.

Liquidity and Capital Resources

As of March 31, 2019, we had cash, cash equivalents and marketable securities of \$444.3 million, which were primarily derived from the sale of common stock. Additionally, we have supplemented our cash flows with fees received from granting commercial licenses to our NAV Technology Platform to other biotechnology and pharmaceutical companies. We expect that our cash, cash equivalents and marketable securities as of March 31, 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.

In January 2018, we amended our March 2014 license agreement with AveXis for the development and commercialization of treatments for SMA. Pursuant to the amended license agreement, AveXis is obligated to pay us \$80.0 million upon the achievement of a sales-based milestone, in addition to other regulatory milestone payments and royalties on net sales of licensed products. In May 2018, AveXis was acquired by Novartis. Novartis has announced that it expects to launch Zolgensma in 2019, pending approval by regulatory authorities. If approved, Zolgensma will be the first approved product under the amended license agreement. Upon its approval and launch, we will begin recognizing royalty revenue on net sales of Zolgensma.

We have incurred cumulative losses since our inception and had an accumulated deficit of \$115.3 million as of March 31, 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

	Three Months Ended March 31,	
	2019	2018
	(in thousands)	
Net cash provided by (used in) operating activities	\$ (29,297)	\$ 58,134
Net cash provided by (used in) investing activities	5,461	(37,086)
Net cash provided by financing activities	4,127	4,166
Net increase (decrease) in cash and cash equivalents and restricted cash	<u>\$ (19,709)</u>	<u>\$ 25,214</u>

Operating Activities

Our net cash used in operating activities for the three months ended March 31, 2019 increased by \$87.4 million from the three months ended March 31, 2018. The change was primarily attributable to \$80.0 million in license payments we received during the three months ended March 31, 2018 related to the amendment of our March 2014 license agreement with AveXis, as well as an increase in operating expenses in the three months ended March 31, 2019. The increase in operating expenses was primarily attributable to increased employee headcount and research and development expenses as we continue the development and advancement of our lead product candidates.

For the three months ended March 31, 2019, our net cash used in operating activities of \$29.3 million consisted of a net loss of \$32.2 million and changes in working capital of \$3.7 million, offset by \$6.7 million in adjustments for non-cash items. The change in working capital was primarily attributable to a decrease in accrued expenses and other current liabilities of \$2.9 million, an increase in prepaid expenses and other current assets of \$1.1 million and a decrease in other liabilities of \$0.6 million. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of \$5.7 million and depreciation and amortization expense of \$1.6 million and were partially offset by imputed interest earned from our license agreements of \$0.6 million and net accretion of discounts on marketable debt securities of \$0.4 million. The decrease in accrued expenses and other current liabilities was largely driven by decreases in accrued personnel costs and accrued licensing costs as of March 31, 2019 as compared to December 31, 2018.

For the three months ended March 31, 2018, our net cash provided by operating activities of \$58.1 million consisted of net income of \$104.2 million and \$3.2 million in adjustments for non-cash items, offset by changes in working capital of \$49.3 million. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of \$3.3 million, depreciation and amortization expense of \$0.8 million and net amortization of premiums on marketable debt securities of \$0.4 million and were partially offset by imputed interest earned from our license agreements of \$1.4 million. The change in working capital was primarily attributable to an increase in accounts receivable of \$51.4 million, which was largely driven by accounts receivable recorded in connection with the amendment of the March 2014 license agreement with AveXis in January 2018.

Investing Activities

For three months ended March 31, 2019, net cash provided by investing activities consisted of \$87.2 million in sales and maturities of marketable securities, offset by \$79.2 million to purchase marketable securities and \$2.5 million to purchase property and equipment.

For the three months ended March 31, 2018, net cash used in investing activities consisted of \$54.3 million to purchase marketable securities and \$2.3 million to purchase property and equipment, offset by \$19.5 million in sales and maturities of marketable securities.

Financing Activities

For the three months ended March 31, 2019, net cash provided by financing activities consisted of \$4.1 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

For the three months ended March 31, 2018, net cash provided by financing activities consisted of \$4.2 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 proprietary technology. 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 March 31, 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 sale of our products, should any of our 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 that may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform sublicensing is dependent in part on the clinical and commercial success of our licensing partners. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

The issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

Contractual Obligations, Commitments and Contingencies

There have been no material changes to our contractual obligations, commitments and contingencies as of March 31, 2019 from the information provided in Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” included in our Annual Report on Form 10-K for the year ended December 31, 2018.

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 three months ended March 31, 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 March 31, 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 March 31, 2019, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

During the quarter ended March 31, 2019, we implemented changes to our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) to support the lease accounting and disclosure requirements of Topic 842, which we adopted on January 1, 2019. There were no other changes in our internal control over financial reporting that occurred during the quarter ended March 31, 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. There have been no material changes from the risk factors previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2018.

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

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
3.1	Restated Certificate of Incorporation	8-K	3.1	9/22/15	
3.2	Amended and Restated Bylaws	8-K	3.2	9/22/15	
10.1*	Employment Agreement effective April 17, 2019 between the Company and Steve Pakola, M.D.				X
10.2‡	Fourth Amendment to License Agreement effective April 4, 2019 between the Company and The Trustees of the University of Pennsylvania				X
10.3	Letter Agreement to Lease dated April 12, 2019 between the Company and ARE-Maryland No. 24, LLC				X
10.4	First Amendment to Lease dated April 23, 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 March 31, 2019 formatted in 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

* Management contract or compensatory plan or arrangement.

‡ Portions of this exhibit have been omitted.

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

SIGNATURES

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

REGENXBIO Inc.

Dated: May 7, 2019

/s/ Kenneth T. Mills

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

Dated: May 7, 2019

/s/ Vittal Vasista

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

[Letterhead of REGENXBIO Inc.]

This Employment Agreement (this “Agreement”) is entered into as of March 6, 2019, by and between Stephen Pakola (the “Employee”) and REGENXBIO Inc., a Delaware corporation (the “Company”) and made effective as of the Commencement Date as herein defined.

1. Position.

- (a) You agree that the Company’s offer to employ you is contingent upon the Company obtaining results of its investigation into your background that it determines in its sole discretion to be satisfactory. This Agreement shall not become effective until you report, ready, willing and able to work on April 15, 2019 (“Commencement Date”). During your employment with the Company pursuant to this Agreement, you will hold the title of Senior Vice President, Chief Medical Officer. As the Senior Vice President, Chief Medical Officer you shall report directly to the Chief Executive Officer. By signing this Agreement, you agree to perform the duties and fulfill the responsibilities normally inherent in the position of Senior Vice President, Chief Medical Officer and such other duties and responsibilities as may from time to time reasonably be assigned to you. You will be primarily located and working from our New York office, located at 400 Madison Avenue, New York, NY. For the first calendar year of your employment, you agree to spend four days per week at our Maryland, Corporate Headquarters office located in Rockville, MD, unless otherwise mutually agreed to in writing.
 - (b) You agree that, to the best of your ability and experience, you will at all times loyally and conscientiously perform all of the duties and obligations required of and from you pursuant to the express and implicit terms hereof, and to the reasonable satisfaction of the Company. During the term of your employment with the Company, you further agree that (i) you will devote substantially all of your business time and attention to the business of the Company, (ii) the Company will be entitled to all of the benefits and profits arising from or incident to all such business services, (iii) you will not render commercial or professional services of any nature to any person or organization outside of the Company without the prior written approval of the Company’s Board of Directors (the “Board”), and (iv) you will not directly or indirectly engage or participate in any business that is competitive in any manner with the business of the Company. Notwithstanding the above, you may continue, on your own time, at your own expense and so as to not interfere with your duties and responsibilities at the Company to (i) subject to the prior approval of the Company’s Chief Executive Officer, serve as a member of an advisory board or board of directors of other companies that are not competitive in any manner with the Company, (ii) accept speaking or presentation engagements in exchange for honoraria, and (iii) participate in civic, educational, charitable or fraternal organizations. This Agreement does not prevent you from owning no more than one percent (1%) of the outstanding equity securities of a corporation whose stock is listed on a national stock exchange and is a competitor or potential competitor of the Company.
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2. Compensation.

- (a) **Base Salary.** You will be paid a salary at a rate of \$16,538.47, which is equivalent to \$430,000 on an annualized basis, which will be paid bi-weekly in accordance with the Company's standard payroll procedures.
 - (b) **Incentive Bonus.** You shall be eligible for an annual incentive bonus with a target amount equal to 40% of your Base Salary (the "Annual Target Bonus"). Such bonus (if any) shall be awarded based on criteria established in advance by the Board or the Compensation Committee of the Board (the "Compensation Committee"). Any incentive bonus earned by you for any fiscal year shall only be paid to you if you remain employed by the Company through the payment date for the bonus. The Company shall determine when to pay to you any earned incentive bonus, but shall in no event pay such bonus more than 2½ months following the close of the fiscal year for which it is earned. Any bonus for the fiscal year in which your employment begins will be prorated, based on the number of days you are employed by the Company during that fiscal year. Employees starting employment on or after October 1 are not eligible for a bonus for that fiscal year. The determinations of the Board or the Compensation Committee with respect to such bonus shall be final and binding.
 - (c) **Annual Review.** Your compensation will be reviewed by the Board or Compensation Committee annually.
 - (d) **Stock Options.** Subject to the approval of the Board or its Compensation Committee, you will be granted an option to purchase 95,000 shares of the Company's Common Stock (the "Option"). The Option will be subject to the terms and conditions applicable to options granted under the Company's 2015 Equity Incentive Plan (the "Plan"), as described in the Plan and the related agreement governing the Option. You will vest in 25% of the shares after 12 months of continuous service, and the balance will vest in equal monthly installments over the next 36 months of continuous service, as described in the applicable agreement governing the Option.
3. **Benefits.** As an employee of the Company, you will also be eligible to receive certain employee benefits including paid time off and medical, dental, life, and long term disability insurance. You will also be eligible to participate in our 401(k) savings plan.
4. **At-Will Employment; Proprietary Information and Inventions Agreement.** Employment with the Company is for no specific period of time. Your employment with the Company is "at will," meaning that either you or the Company may terminate your employment at any time and for any reason, with or without cause. In addition, you should note that the Company may modify your job title, salary or benefits at its discretion. You agree and affirm that your continued employment with the Company is contingent upon your agreement to comply with the Proprietary Information and Inventions Agreement, a copy of which is attached hereto as Exhibit A.

5. **Indemnification.** The Company shall indemnify you to the fullest extent allowed by law, in accordance with the terms of the Company's Certificate of Incorporation and Bylaws. You shall become a party to the Company's standard Indemnification Agreement.
6. **Company Handbook.** As a Company employee, you will be expected to abide by the Company's rules of operation and standards of conduct, as amended from time to time in the Company's discretion. Specifically, you will be required to sign acknowledgments that you have read and that you understand such rules and standards, which are set forth in the Company Handbook and other written policies.
7. **Termination of Employment and Severance Benefits.**
 - (a) **Preconditions.** Any other provision of this Agreement notwithstanding, Subsections of this Section 7 providing for the payment of severance benefits shall not apply unless each of the following requirements is satisfied:
 - (i) You have executed a general release of all known and unknown claims that you may then have against the Company or persons affiliated with the Company in a form prescribed by the Company, without alterations. You shall execute and return the release on or before the date specified by the Company in the prescribed form. The release deadline shall in no event be later than sixty (60) days after your termination of employment (the "Release Deadline"). If the 60-day period described in the prior sentence spans two calendar years, then the payments will begin on the first payroll period, following expiration of the revocation period, in the second calendar year. If you fail to return the release on or before the Release Deadline, or if you revoke the release, then you shall not be entitled to the benefits described in this Section 7; and
 - (ii) You have returned all property of the Company in your possession.
 - (b) **Termination of Employment.** Except for the severance benefits provided below, the Company's obligations under this Agreement may be terminated upon the occurrence of any of the following events:
 - (i) The Company's determination in good faith that it is terminating you for Cause ("Termination for Cause");
 - (ii) The Company's determination that it is terminating you without Cause, which determination may be made by the Company at any time at the Company's sole discretion, for any or no reason ("Termination Without Cause");
 - (iii) Thirty (30) days following delivery by you of a written notice to the Company stating that you are electing to terminate your employment with the Company ("Voluntary Termination");
 - (iv) Following your death or Disability (as defined below); or
 - (v) Your determination in good faith that you are electing to terminate your employment with the Company for Good Reason.

(c) **Severance Benefits.** You shall be entitled to receive severance benefits upon termination of employment only as set forth in this Section 7(c):

- (i) **Voluntary Termination.** In the event of a Voluntary Termination you shall not be entitled to receive payment of any severance benefits. You will receive payment(s) for all salary and unpaid vacation accrued as of the date of your Voluntary Termination and your benefits will be continued under the Company's then existing benefit plans and policies to the extent permitted under such plans and policies and in accordance with such plans and policies in effect on the date of your Voluntary Termination and in accordance with applicable law.
- (ii) **Involuntary Termination/No Change in Control.** If your employment is terminated under Section 7(b)(ii) or (v) above (such termination, an "Involuntary Termination"), you, or your estate or representative, if applicable, will be entitled to receive payment of severance benefits on the date of your Involuntary Termination (the "Severance Benefits"). The Severance Benefits shall consist of salary continuation for nine (9) months of monthly Base Salary amounts; provided that if you become employed during this period, then the Company's obligation to pay Severance Benefits shall cease upon commencement of your new employment. If you elect to continue your health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act ("COBRA") following the Separation, then the Company shall pay your monthly premium under COBRA until the earliest of (A) the date that is nine (9) months following your Involuntary Termination (the "Continuation Period"), (B) the expiration of your continuation coverage under COBRA and (C) the date when you are offered substantially equivalent health insurance coverage in connection with new employment or self-employment. Notwithstanding anything to the contrary above, if deemed necessary or advisable by the Company in its sole discretion to avoid adverse tax consequences to the Company or any employee thereof, such COBRA premium payments will be treated as taxable compensation income to you, subject to all applicable withholdings.

- (iii) **Involuntary Termination/ Change in Control.** If your employment is terminated in an Involuntary Termination immediately prior to or in the eighteen months following a Change in Control, you, or your estate or representative, if applicable, will be entitled to receive payment of severance benefits on the date of your Involuntary Termination (the “Change in Control Severance Benefits”). The Change in Control Severance Benefits shall consist of salary continuation for twelve (12) months of monthly Base Salary plus a monthly amount equal to your Annual Target Bonus divided by twelve (12). If you elect to continue your health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act (“COBRA”) following the Separation, then the Company shall pay your monthly premium under COBRA until the earliest of (A) the date that is twelve (12) months following your Involuntary Termination (the “Continuation Period”), (B) the expiration of your continuation coverage under COBRA and (C) the date when you are offered substantially equivalent health insurance coverage in connection with new employment or self-employment. Notwithstanding anything to the contrary above, if deemed necessary or advisable by the Company in its sole discretion to avoid adverse tax consequences to the Company or any employee thereof, such COBRA premium payments will be treated as taxable compensation income to you, subject to all applicable withholdings. If immediately prior to or following a Change in Control (as defined in the Company’s 2015 Equity Incentive Plan), your employment with the Company (or the Company’s successor) is terminated in an Involuntary Termination during the remaining vesting period of the options then outstanding as of the date of closing of the Change in Control (the “Options”), then one hundred percent (100%) of the unvested shares subject to the Options shall automatically vest.
- (iv) **Termination for Cause.** In the event of your Termination for Cause, you will not be entitled to receive any severance payments. You will receive payment(s) for all salary and unpaid vacation accrued as of the date of your Termination for Cause.
- (v) **Termination by Reason of Death or Disability.** In the event that your employment with the Company terminates as a result of your death or Disability (as defined below), you or your estate or representative will receive all salary and unpaid vacation accrued as of the date of your death or Disability, all severance benefits payable under Section 7(b)(ii) above (only to the extent that you were entitled to such benefits before your death) and any other benefits payable under the Company’s then existing benefit plans and policies, to the extent permitted under such plans and policies and in accordance with such plans and policies in effect on the date of death or Disability and in accordance with applicable law. For purposes of this Agreement, “Disability” shall mean that you have been unable to perform your duties hereunder as the result of physical or mental incapacity lasting at least forty-five (45) consecutive calendar days or ninety (90) calendar days during any consecutive twelve-month period, after which time such incapacity is determined to be permanent by a physician chosen by the Company and its insurers and acceptable to you or to your legal representative (with such agreement on acceptability not to be unreasonably withheld).

- (d) **Cause.** For purposes of this Agreement, “Cause” shall mean:
- (i) the conviction of, or the entering a plea of guilty or no contest (or pleading or accepting deferred adjudication or receiving unadjudicated probation) to or for, any felony or any crime involving moral turpitude;
 - (ii) the commission of a material breach of any of the covenants, terms and provisions of this Agreement, the Proprietary Information and Inventions Agreement you will enter into as a condition of your employment, or any other agreement you enter into with the Company;
 - (iii) the commission of an act of fraud, embezzlement, misappropriation, willful misconduct or breach of fiduciary duty against the Company or other similar conduct materially harmful or potentially materially harmful to the Company’s best interest, as determined by the Board, in its reasonable sole discretion;
 - (iv) the failure to perform assigned duties or responsibilities as the Senior Vice President, Chief Medical Officer (other than a failure resulting from Disability (as defined below)); provided, however, that you shall be given written notice of, and shall have a ten (10) day period following such notice to cure a failure or refusal under this subclause (iv); or
 - (v) the violation of any federal or state law or regulation applicable to the Company’s business.
- (e) **Good Reason.** For purposes of this Agreement, “Good Reason” shall mean the occurrence of any of the following, without your written consent:
- (i) a significant reduction in your duties or responsibilities or your removal from the position contemplated by this Agreement;
 - (ii) a significant reduction (thirty percent (30%) or more) in your base salary as in effect immediately prior to such reduction;
 - (iii) a significant reduction in the type or level of employee benefits to which you are entitled that results in a significant reduction to your overall benefits package, as determined by the Board in its sole discretion; or
 - (iv) relocation of your principal workplace by more than 35 miles from the primary office where you performed services prior to the relocation.

Good Reason will not be deemed to occur unless you give the Company written notice of the condition within 90 days after the condition comes into existence and the Company fails to remedy the condition with 30 days after receiving said notice.

8. Tax Matters.

- (a) **Withholding.** All forms of compensation referred to in this Agreement are subject to reduction to reflect applicable withholding and payroll taxes and other deductions required by law.
- (b) **Tax Advice.** You are encouraged to obtain your own tax advice regarding your compensation from the Company. You agree that the Company does not have a duty to design its compensation policies in a manner that minimizes your tax liabilities, and you will not make any claim against the Company or the Board related to tax liabilities arising from your compensation.
- (c) **280G.** Notwithstanding anything contained in this Agreement to the contrary, if any of the payments or benefits received or to be received by you pursuant to this Agreement when taken together with payments and benefits provided to you under any other plans, contracts, or arrangements with the Company (all such payments and benefits, the “Total Payments”), would be subject to any excise tax (together with any interest or penalties, the “Excise Tax”) imposed under Section 4999 of the Internal Revenue Code (the “Code”), then such Total Payments will be reduced to the extent necessary so that no portion thereof will be subject to the Excise Tax; provided, however, that if you would receive in the aggregate greater value (as determined under Section 280G of the Code and the regulations thereunder) on an after tax basis if the Total Payments were not subject to such reduction, then no such reduction will be made. To effect the reduction described herein, if applicable, the Company will first reduce or eliminate the payments and benefits provided under this Agreement. All calculations required to be made under this Section will be made by the Company’s independent public accountants, subject to the right of your representative to review the same.
- (d) **409A.** The intent of the parties is that payments and benefits under this Agreement comply with or be exempt from Section 409A of the Code and the regulations and guidance promulgated thereunder (collectively, “Code Section 409A”), and this Agreement shall be interpreted and construed in a manner that establishes an exemption from (or compliance with) the requirements of Code Section 409A. Any terms of this Agreement that are undefined or ambiguous shall be interpreted in a manner that complies with Code Section 409A to the extent necessary to comply with Code Section 409A. For purposes of Code Section 409A, your right to receive any installment payments shall be treated as a right to receive a series of separate and distinct payments. Whenever a payment under this Agreement specifies a payment period with reference to a number of days, the actual date of payment within the specified period shall be within the sole discretion of the Company. In no event may you, directly or indirectly, designate the calendar year of any payment to be made under this Agreement, to the extent such payment is subject to Code Section 409A. The Company makes no representation or warranty and shall have no liability to you or any other person if any provisions of this Agreement are determined to constitute deferred compensation subject to Code Section 409A but do not satisfy an exemption from, or the conditions of, Code Section 409A.”

9. Miscellaneous Provisions.

- (a) **Choice of Law.** The validity, interpretation, construction and performance of this Agreement shall be governed by the laws of the State of Maryland, without giving effect to the principles of conflicts of law.
- (b) **Counterparts.** This Agreement may be executed in counterparts, each of which shall be deemed an original, but all of which together will constitute one and the same instrument.
- (c) **Severability.** In the event that any provision hereof becomes or is declared by a court of competent jurisdiction to be illegal, unenforceable, or void, this Agreement shall continue in full force and effect without such provision.
- (d) **Acknowledgment.** You acknowledge that you have had the opportunity to discuss this matter with and obtain advice from your private attorney, have had sufficient time to read, and have carefully read and fully understand, all the provisions of this Agreement, and are knowingly and voluntarily entering into this Agreement.
- (e) **Arbitration.** Any controversy or claim arising out of this Agreement and any and all claims relating to the Employee's employment with the Company shall be settled by final and binding arbitration. The arbitration shall take place in Montgomery County, Maryland, or, at the Employee's option, the County in which the Employee primarily worked when the arbitrable dispute or claim first arose. The arbitration shall be administered by the American Arbitration Association under its National Rules for the Resolution of Employment Disputes. Any award or finding shall be confidential. The Employee and the Company agree to provide one another with reasonable access to documents and witnesses in connection with the resolution of the dispute. The Company shall pay the costs of arbitration. However, each party shall be responsible for its own attorneys' fees, and the arbitrator may not award attorneys' fees unless a statute or contract at issue specifically authorizes such an award. This Section 9(e) shall not apply to claims for workers' compensation benefits or unemployment insurance benefits. This Section 9(e) also shall not apply to claims concerning the ownership, validity, infringement, misappropriation, disclosure, misuse or enforceability of any confidential information, patent right, copyright, mask work, trademark or any other trade secret or intellectual property held or sought by either the Employee or the Company (whether or not arising under the Proprietary Information and Inventions Agreement between the Employee and the Company) or with respect to any action the Company wishes to bring for injunctive relief.
- (f) **Entire Agreement.** This Agreement, together with the exhibits hereto, sets forth the terms and conditions of employment between the parties and fully supersedes and replaces any other agreement with respect to the terms and conditions of employment.

[The remainder of this page intentionally left blank]

IN WITNESS WHEREOF, each of the parties has executed this Agreement, in the case of the Company by its duly authorized officer, as of the day and year first above written.

REGENXBIO INC.

By: /s/ Kenneth Mills

Name: Kenneth Mills

Title: President & CEO

EMPLOYEE

/s/ Stephen Pakola

Date: March 6, 2019

EXHIBIT A

PROPRIETARY INFORMATION AND INVENTIONS AGREEMENT

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

UNIVERSITY *of* PENNSYLVANIA

Fourth Amendment to License Agreement

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

BACKGROUND

WHEREAS, there is a Sponsored Research Agreement between the Parties, which has an effective date of December 1, 2014, as subsequently amended by the First Amendment to Sponsored Research Agreement dated April 30, 2016, the Second Amendment to Sponsored Research Agreement dated December 21, 2016, the Third Amendment to Sponsored Research Agreement dated December 23, 2016, the Fourth Amendment to Sponsored Research Agreement dated June 15, 2017, the Fifth Amendment to Sponsored Research Agreement dated January 18, 2018, the Sixth Amendment to Sponsored Research Agreement dated June 20, 2018, and the Seventh Amendment to Sponsored Research Agreement dated December 18, 2018 (“2014 SRA”), and the 2014 SRA includes research relating to Batten disease as part of the Sponsored Research (as defined in the 2014 SRA);

WHEREAS, Penn has agreed to grant Company a license under the Institution Intellectual Property (as defined in the 2014 SRA) in the 2014 SRA; and

WHEREAS, the Parties desire that the License Agreement be amended as set forth below to reflect the rights and licenses granted to Company in accordance with the 2014 SRA;

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

1. The following definitions shall be added to Section 1.2 of the License Agreement and incorporated in its entirety:

“*Background Know-How*” means all:

- (i) Know-How that (a) was developed by Dr. Wilson, or other Penn researchers working under his direct supervision, at Penn, and (b) is related to the adeno
-

associated virus technology platform discovered by Dr. Wilson at Penn prior to September 9, 2014 or is related to the adeno associated virus technology platform discovered by Dr. Wilson at Penn during the performance of a Company sponsored research program after September 9, 2014, and (c) is owned by Penn, and (d) is necessary or useful for the practice of the Patent Rights in connection with the manufacture, use, sale, importation and/or other exploitation of the Licensed Products or the practice of the Licensed Processes in the Territory in the Field of Use, including, without, limitation, any Know-How necessary for the Company to manufacture or have manufactured the materials produced by the Penn Vector Core or Dr. Wilson's lab at Penn; and

(ii) Penn Study Data; and

(iii) any and all information, discoveries, software, methods, works of authorship, techniques, formulae, data, biological materials, processes, unpatentable inventions and other know-how, not including the Patent Rights, that (a) was developed on or after October 1, 2015 by Dr. Wilson, or other Penn researchers working under his direct supervision, at Penn, and (b) is related to Batten disease, and (c) is owned by Penn, and (d) is necessary or useful for the practice of the Patent Rights in connection with the manufacture, use, sale, importation and/or other exploitation of the Licensed Products or the practice of the Licensed Processes in the Territory in the Batten Field of Use.

“Batten’s Field of Use” means the treatment of a form of Batten disease known as late infantile ceroid lipofuscinosis (LINCL) via AAV mediated gene therapy delivery of tripeptidyl peptidase 1 (TPP1).

2. The following definitions in Section 1.2 of the License Agreement shall be amended and restated in their entirety as follows:

“Field of Use” means any and all fields of use, except with respect to (i) the Patent Rights listed in Exhibit A, Part 2 and all related Know-How and data, for which the Field of Use is limited to viral vector mediated gene therapy, and (ii) the Penn Study Data, for which the Field of Use is limited to the treatment of familial hypercholesterolemia (FH). For clarity, the Field of Use includes the Batten Field of Use.

“*Patent Rights*” means:

- (i) all of Penn’s patent rights represented by or issuing from the United States patents and patent applications (including provisional patent applications) listed in Exhibit A, as well as any continuations, continuations-in-part (to the extent the inventions claimed or disclosed in any such patent or patent applications are directed to subject matter specifically described in the patent or patent applications listed in Exhibit A), divisionals, reexaminations, renewals, re-issues, substitutions, extensions and foreign counterparts of any of the foregoing, and all other patents and patent applications that claim priority from or have common priority with any of the foregoing patents and patent applications (to the extent the inventions claimed or disclosed in any such patent or patent applications are directed to subject matter specifically described in the patent or patent applications listed in Exhibit A) and including any patents issuing from any of the foregoing; and
- (ii) all patentable inventions (to the extent they are or become available for license) that (a) were discovered by Dr. Wilson, or other Penn researchers working under his direct supervision at Penn and (b) (I) are related to the adeno associated virus technology platform discovered by Dr. Wilson at Penn prior to the Effective Date or under the SRA, or (II) are necessary or useful for the practice of Penn’s patent rights in the Batten’s Field of Use and conceived and reduced to practice on or after October 1, 2015; and (c) are owned and controlled by Penn.

“*SRA*” means each of: 1) the Sponsored Research Agreement between the Company and Penn effective as of February 24, 2009, as subsequently amended; 2) the Sponsored Research Agreement between the Company and Penn effective as of November 1, 2013 and any subsequent amendments thereof; and 3) the 2014 SRA and any future amendments thereof.

3. Section 3 is hereby amended to add Section 3.1.1 after Section 3.1 as follows:

3.1.1 Upfront Fees. In partial consideration of the licenses and rights granted to Company in this Agreement, Company shall pay Penn a one-time upfront fee of [****] within [****] of the Fourth Amendment Effective Date.

4. New Section 3.2(d) of the License Agreement is hereby added as set forth below:

“3.2(d) In addition to the royalty due under this Section 3.2, Company shall pay to Penn a [****] additional royalty percentage on Net Sales of Licensed Pharmaceutical Products in the Batten Field of Use sold by Company and its Affiliates and an additional [****] royalty percentage received by Company from third parties on Net Sales of Licensed Pharmaceutical Products in the Batten Field of Use by such third parties. For example, for Net Sales of Licensed Pharmaceutical Products using a novel AAV sold by Company or its Affiliates where annual Net Sales are greater than or equal to [****], the royalty is [****] for such Licensed Pharmaceutical Products in the Batten Field of Use. As a further example, for Net Sales of Licensed Pharmaceutical Products sold by third parties where third party annual Net Sales are greater than or equal to [****], Company shall pay Penn [****] of the royalties received by Company from third parties for such Licensed Pharmaceutical Products in the Batten Field of Use.”

5. The last paragraph of Section 3.2 of the License Agreement is amended and restated in its entirety as follows:

“Notwithstanding the foregoing (i) in no event shall the [****] paid to Penn by the Company pursuant to Sections 3.2(c) and 3.2(d) above, [****] that would be payable to Penn by the Company on such Net Sales of Licensed Pharmaceutical Products sold by Company and (ii) in no event shall [****] be payable in connection with any [****]. No royalties other than the payments set forth herein shall be due in connection with the exercise of the rights granted herein. [****].”

6. The following provision shall be added to the License Agreement as Section 3.3 and incorporated in its entirety:

3.3 Development Milestones. In partial consideration of the rights and licenses granted to Company under the Agreement, Company shall pay Penn the following milestone payments within [****] after the first achievement of each event for the first Licensed Product in the Batten Field of Use to achieve the following milestone events:

Development Milestone	Milestone Payment
1. First dosing of the first human subject in a Phase 1/2 Clinical Trial in the Batten Field of Use	[****]
2. First dosing of the first human subject in a Phase 3 Clinical Trial in the Batten Field of Use	[****]
3. BLA Acceptance in the Batten Field of Use in the United States	[****]
4. Receipt of Marketing Authorization in the Batten Field of Use outside of the United States	[****]
Total	[****]

For clarity, the milestone payments set forth in Section 3.3 are [****] with respect to the first Licensed Product in the Batten Field of Use to achieve the foregoing milestone events, regardless of the total number of Licensed Products arising in the Batten Field of Use that achieve the milestone event, and regardless of whether the milestone is achieved by Company, any Affiliate, or any sublicensee.

7. The following provision shall be added to the License Agreement as Section 3.4 and incorporated in its entirety:

3.4 Net Sales Milestones. In partial consideration of the rights and licenses granted to Company under this Agreement, Company shall pay Penn the following milestone payments within [****] following the end of the calendar year during which annual Net Sales of Licensed Products within the Batten Field of Use achieve the sales milestones indicated below, regardless of whether the milestone is achieved by Company, any Affiliate, or any sublicensee:

Milestone	Milestone Payment
1. Annual worldwide Net Sales of Licensed Products within the Batten Field of Use are [****]	[****]
2. Annual worldwide Net Sales of Licensed Products within the Batten Field of Use are [****]	[****]
Total	[****]

For clarity, the foregoing annual Net Sales milestones are [****] Licensed Products within the Batten Field of Use achieve the above annual Net Sales. In addition, in the event that the second annual Net Sales milestone is met with respect to Licensed Products within the Batten Field of Use prior to the achievement of the first annual Net Sales milestone, then the first annual Net Sales milestone with respect to Licensed Products shall also be due along with the payment of the second annual Net Sales milestone.

8. Section 3.5 of the License Agreement shall be amended as follows:

- a. The heading “A. In all Fields of Use other than the Batten Field of Use” shall be added before the table listing the Date of Sublicense Grant and Sublicensing Fees.
- b. The following shall be added after the table listing the Date of Sublicense Grant and Sublicensing Fees:

B. In the Batten Field of Use:

Date of Sublicense Grant	Sublicensing Fees
During the period commencing on the Fourth Amendment Effective Date and ending the day prior to the first dosing of the first human subject in a Phase 1/2 Clinical Trial	[****]
Any date on or after the first dosing of the first human subject in a Phase 1/2 Clinical Trial	[****]

9. The following definitions shall be added to Section 3.8 of the License Agreement and incorporated herein in their entirety:
- “*BLA*” means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for Marketing Authorization in any country or regulatory jurisdiction other than the United States.
- “*Marketing Authorization*” means all approvals, licenses, registrations or authorizations of any federal, state or local regulatory agency, department, bureau or other governmental entity, necessary for the manufacturing, use, storage, import, transport, marketing and sale of Licensed Products in a country or regulatory jurisdiction.
10. Exhibit A of the License Agreement is hereby amended and restated in its entirety to add the Patent Rights as set forth in Appendix A to this Fourth Amendment.
11. To Penn’s knowledge through its Penn Center for Innovation, as of the Fourth Amendment Effective Date, Penn does not Control any patent or patent application related to the Batten disease clinical candidate RGX-181 ([****]) (the “Batten Clinical Candidate”) transgene and expression cassette (other than the Patent Rights set forth in Appendix A to this Fourth Amendment) that would necessarily be infringed by the use or sale of a Licensed Product containing the Batten Clinical Candidate within the Batten Field of Use. If it is determined that as of the Fourth Amendment Effective Date Penn Controls a patent or patent application directly related to the Batten Clinical Candidate transgene and expression cassette (other than the Patent Rights set forth in Appendix A) that was conceived and reduced to practice solely in the Wilson Lab and that would necessarily be infringed by the use or sale of a Licensed Product containing the Batten Clinical Candidate within the Batten Field of Use, then the Parties shall amend Exhibit A to include such applicable patent or patent application For the purpose of this Section 11, “Control” means the possession by Penn (whether by ownership or license, other than pursuant to this Agreement) of the ability to grant to Company access, a license, or a sublicense (as applicable) to the applicable patent or patent application on the terms and conditions set forth herein without violating the terms of any agreement or other arrangement with any third party.

12. This Fourth Amendment amends the terms of the License Agreement and is deemed incorporated into, and governed by all other terms of, the License Agreement. To the extent that the License Agreement is explicitly amended by this Fourth Amendment, the terms of this Fourth Amendment will control where the terms of the License Agreement are contrary to or conflict with the terms of this Fourth Amendment. All other terms and conditions of the License Agreement not explicitly amended by this Fourth Amendment shall remain in full force and effect. The License Agreement, as previously amended, shall, together with this Fourth Amendment, be read and construed as a single instrument.
13. Signatures on this Fourth Amendment may be communicated by facsimile or e-mail transmission and shall be binding upon the Parties upon receipt by transmitting the same by facsimile or e-mail transmission, which signatures shall be deemed originals. If executed in counterparts, the Fourth Amendment shall be effective as if simultaneously executed.

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

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: /s/ John S. Swartley
Name: John S. Swartley, PhD
Title: Associate Vice Provost for Research
and Managing Director, PCI
Date: April 5, 2019

REGENXBIO INC.

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President and CEO
Date: April 5, 2019

Appendix A - Patent Rights

Exhibit A

Patents and Patent Applications in the Patent Rights

Exhibit A- Part 1; No Field of Use Limitation

[****]

Exhibit A, Part 2-

Field of Use limited to viral vector mediated delivery of gene therapy product.

[****]

Penn / Wilson Lab Know-How for the Familial Hypercholesterolemia and Onithine Transcarbamyase Deficiency (OTC) Programs

FH Know-How (associated with [****]).

[****]

OTC Know-How (associated with [****]).

[****]

ARE-MARYLAND NO. 24, LLC
c/o Alexandria Real Estate Equities, Inc.
385 E. Colorado Blvd., Suite 299
Pasadena, California 91101

April 12, 2019

REGENXBIO Inc.
Attention: General Counsel
9600 Blackwell Drive, Suite 210
Rockville, MD 20850

Re: **9800 Medical Center Drive—Letter Agreement**

To Whom It May Concern:

Reference is made to that certain Lease Agreement dated as of November 1, 2018 (“Lease”) between REGENXBIO Inc., a Delaware corporation, as tenant (“Tenant”), and ARE-Maryland No. 24, LLC, a Delaware limited liability company, as landlord (“Landlord”). Initially capitalized terms not specifically defined in this letter agreement are intended to have the meanings set forth for such terms in the Lease.

Pursuant to Section 2.e of Exhibit C-1 to the Lease, Tenant has requested certain Material Changes to the Base Building Work. The Material Changes, as more fully described or shown on Exhibit A attached hereto, consist of the following and are hereinafter collectively referred to in this letter agreement as the “February 2019 Material Changes”:

- a. Modifications to X bracing
- b. Relocate N Stairwell
- c. Reposition service elevator and add second dedicated service elevator
- d. Increase 5th floor in height (not greater than 3')
- e. Increase 5th floor load to 125 lbs/sf
- f. Future Penthouse / Roof Screening and Structure

For clarity, it is agreed and understood that the aforesaid second dedicated service elevator and Future Penthouse / Roof Screening and Structure shall be reserved for Tenant’s exclusive use.

Pursuant to Section 2.e of Exhibit C-1 to the Lease, Landlord hereby agrees to implement the February 2019 Material Changes. This letter agreement is intended to supplement the applicable terms and conditions of the Lease as to the manner in which the February 2019 Material Changes shall be implemented, as follows:

1. Material Change Payment. Within 30 days after the date of this letter agreement, Tenant shall pay to Landlord as Additional Rent an amount equal to \$3,975,000 ("Material Change Payment"). Tenant shall pay the Material Change Payment to Landlord by means of a wire transfer of immediately available federal funds to the Address for Rent Payment. The Material Change Payment consists of the following elements:

- 1.1 An amount equal to \$1,050,000 ("Estimated Delay Cost"), which is the product of \$350,000 multiplied by 3 months, which is the estimated period of Tenant Delay that will result from the implementation of the February 2019 Material Changes.
- 1.2 An amount equal to \$2,600,000 ("Estimated Construction Cost"), which is the estimated cost Landlord will incur to implement the February 2019 Material Changes.
- 1.3 An amount equal to \$325,000 ("Management Fee"), which represents Landlord's management fee for the implementation of the February 2019 Material Changes.

2. Reconciliation of Material Change Payment. Within a reasonable period of time (not to exceed 60 days) after Substantial Completion of the Base Building Work, Landlord will determine (a) the actual Delay Cost ("Actual Delay Cost") based on the actual period of Tenant Delay caused by the implementation of the February 2019 Material Changes (as certified by the Base Building Architect pursuant to Section 3.d of the Landlord Work Letter attached to the Lease as Exhibit C-1 ("Actual Tenant Delay"), and based on \$350,000 per month, prorated on a daily basis based on the actual number of calendar days of Tenant Delay), and (b) the actual Construction Cost ("Actual Construction Cost") incurred by Landlord to implement the February 2019 Material Changes. Landlord shall communicate that determination to Tenant by means of a reconciliation statement ("Reconciliation Statement"). The Reconciliation Statement shall state whether any adjustments need to be made to the Material Change Payment and, if so, the Reconciliation Statement shall be accompanied by reasonably detailed documentation in support of such determination. If the Estimated Delay Cost or Estimated Construction Cost, or both, exceeded the Actual Delay Cost or the Actual Construction Cost, or both, Landlord shall pay the excess to Tenant within 30 days after the date of the Reconciliation Statement. If the Estimated Delay Cost or the Estimated Construction Cost, or both, were less than the Actual Delay Cost or the Actual Construction Cost, or both, Tenant shall pay the deficiency to Landlord as Additional Rent within 30 days after the date of the Reconciliation Statement. At Tenant's request, Landlord shall make available to Tenant, on an open book basis, the documentation in support of the Reconciliation Statement.

3. Impact on Dates.

a. Estimated and Actual Tenant Delay. It is agreed by Landlord and Tenant that the estimated period of Tenant Delay that will result from the implementation of the February 2019 Material Changes is three (3) months ("Estimated Tenant Delay"). Within a reasonable period of time (not to exceed 60 days) after Substantial Completion of the Base Building Work, Landlord shall cause the Base Building Architect to certify the Actual Tenant Delay.

b. Amended Rider 1. Rider 1 to Exhibit C-1 of the Lease is hereby amended by replacing that Rider with Rider 1 attached hereto as Rider 1. In connection with the amendments to Rider 1 to Exhibit C-1 of the Lease, (i) the phrase in Section 2(1) of the Lease stating “April 30, 2019 (such 270 day period ends on Saturday, January 25, 2020)” is hereby deleted and replaced with the following phrase: “July 31, 2019 (such 270 day period ends on Sunday, April 26, 2020)”, and (ii) the phrase in Section 2(2) of the Lease stating “March 31, 2020 (such 270 day period ends on Saturday, December 26, 2020)” is hereby deleted and replaced with the following phrase: “June 30, 2020 (such 270 day period ends on Saturday, March 27, 2021)”.

c. Lease Commencement Date and Rent Commencement Dates. Landlord and Tenant agree that (i) the period of Actual Tenant Delay shall not affect the Lease Commencement Date, (ii) the Lease Commencement Date shall not be extended for any period of Actual Tenant Delay, and (iii) the Lease Commencement Date shall be determined as if there were no period of Actual Tenant Delay. To illustrate the operation of this provision, assume that June 30, 2020 is the projected Lease Commencement Date and that no Tenant Delay (other than the Actual Tenant Delay) or Force Majeure Delay has occurred. Based on these assumptions and notwithstanding any period of Actual Tenant Delay, the Lease Commencement Date shall be deemed to occur on June 30, 2020 and the Rent Commencement Date and the 4th Floor Rent Commencement Date shall be determined based on the Lease Commencement Date occurring on June 30, 2020. Except as provided in this paragraph, the Lease Commencement Date shall be subject to extension for Tenant Delay or Force Majeure Delay subject to the terms and conditions set forth in the Lease.

d. Target Commencement Date. In light of the Estimated Tenant Delay, (i) the references to “Target Commencement Date” in the first paragraph of Section 2 of the Lease (relating to a termination right in favor of Tenant) and in Section 2(2)(a) of the Lease (relating to the Per Diem Credit) shall initially mean October 1, 2020 (rather than July 1, 2020), and (ii) the date that is 270 days after the Target Commencement Date as referenced in such Lease provisions shall initially be measured from October 1, 2020 (rather than July 1, 2020), with all such dates to be finally adjusted based on the Actual Tenant Delay (and any other Tenant Delays).

4. Binding Effect; No Other Changes. This letter agreement shall be binding upon Landlord, its successors and assigns, and Tenant, its successors and assigns as permitted under Section 22 of the Lease. Except as supplemented by this letter agreement, 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 letter agreement. In the event of any conflict between the provisions of this letter agreement and the provisions of the Lease, the provisions of this letter agreement shall prevail. Regardless of whether specifically supplemented by this letter agreement, 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 letter agreement.

5. Confidentiality. The parties will keep the subject matter of this letter agreement confidential between them in accordance with the requirements and subject to the exceptions of Section 47(m) of the Lease. Landlord acknowledges and agrees that Tenant shall have the right to disclose this letter agreement in connection with a request under Sections 23 (Estoppel Certificate) or 27 (Subordination) of the Lease and the delivery of any statement or instrument thereunder, or as otherwise permitted under Section 47(m) of the Lease.

6. Counterparts/Electronic Signatures. This letter agreement 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 letter agreement and all matters related thereto, with such electronic signatures having the same legal effect as original signatures.

Please acknowledge your agreement to the terms of this letter agreement by countersigning below.

Sincerely,

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

By: Alexandria Real Estate Equities, L.P.,
a Delaware limited partnership,
as its sole member

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

By: /s/ Gary Dean

Name: Gary Dean

Title: Senior Vice President

RE Legal

[SIGNATURE CONTINUED ON NEXT PAGE]

**ACKNOWLEDGED AND AGREED AS OF
THE DATE FIRST WRITTEN ABOVE:**

REGENXBIO INC.,
a Delaware corporation

By: /s/ Kenneth Mills
Print Name: Kenneth Mills
Its: President & CEO

EXHIBIT A
MATERIAL CHANGES

The following list of MATERIAL CHANGES are the basis of this letter agreement.

- a. Modifications to X bracing – **proceed**
- b. Relocate N Stairwell – **proceed**
- c. Reposition service elevator and add second dedicated service elevator – **proceed**
- d. Central stair security required between REGENXBIO floors and future tenant floors – **proceed**
- e. Increase 5th floor 3 feet in height – **proceed**
- f. Increase 5th floor load to 125 lbs/sf – **proceed**
- g. Future Penthouse – **proceed**

The list below of PDF documents (which documents have been prepared by Gaudreau, Inc.) are the supporting documents for these Material Changes:

DATED	TITLE	NOTES
• 1/14/2019	S-001 - GENERAL NOTES, ABBREVIATIONS, AND SYMBOLS.pdf	
• 2/19/2019	S-111 - ROOF FRAMING PLAN—AREA A.pdf	
• 2/19/2019	S-112 - ROOF FRAMING PLAN—AREA B.pdf	
• 2/19/2019	S-113 - PENTHOUSE ROOF AND MECHANICAL PLATFORM FRAMING PLAN—AREA A.pdf	
• 2/19/2019	S-114 - PENTHOUSE ROOF AND MECHANICAL PLATFORM FRAMING PLAN—AREA B.pdf	
• 3/26/2019	A-101 - OVERALL FLOOR PLANS - 1ST & 2ND FLOORS.pdf	
• 3/26/2019	A-102 - OVERALL FLOOR PLANS - 3RD & 4TH FLOORS.pdf	
• 2/19/2019	A-103 - OVERALL FLOOR PLANS - 5TH FLOOR & ROOF.pdf	
• 2/19/2019	A-301 - BUILDING SECTIONS.pdf	
• 3/26/2019	A-401 - STAIR PLANS - STAIR 01.pdf	
• 3/26/2019	A-402 - STAIR SECTIONS - STAIR 01.pdf	
• 3/26/2019	A-403 - STAIR PLANS - STAIR 02.pdf	
• 3/26/2019	A-404 - STAIR SECTIONS - STAIR 02.pdf	
• 3/26/2019	A-411 - LOADING AREA ENLARGED PLAN AND DETAILS.pdf	
• 3/26/2019	A-421 - RESTROOM ENLARGED FLOOR PLAN - FIRST FLOOR.pdf	
• 3/26/2019	A-422 - RESTROOM ENLARGED FLOOR PLAN - SECOND-FIFTH FLOORS.pdf	
• 3/26/2019	A-423 - RESTROOM INTERIOR ELEVATIONS.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S101.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S102.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S103.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S104.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S105.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S106.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S107.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S108.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S109.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S110.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S601.pdf	
• 3/26/2019	1713 - 9800 MCD Bldg F - S602.pdf	

RIDER 1

**RIDER 1 TO EXHIBIT C-1
Base Building Construction Schedule**

Milestone Event	Projected Date
Base Building Design Drawings sent to Tenant for comment	November 30, 2018
Deadline for completing Final Base Building Design Drawings	December 18, 2018
Deadline for completing Final Base Building Construction Drawings	May 9, 2019
Base Building Permit issued	July 31, 2019
Landlord begins performance of Base Building Work	August 31, 2019
Completion of erection of structural steel for Base Building	April 30, 2020
Turnover Condition Date (i.e., date on which Building is watertight)	June 30, 2020
Substantial Completion of Base Building Work	September 30, 2020

FIRST AMENDMENT TO LEASE AGREEMENT

THIS FIRST AMENDMENT TO LEASE AGREEMENT (“**this First Amendment**”) is dated as of April 23, 2019 (“**Effective Date**”), by and between **ARE-MARYLAND NO. 24, LLC**, a Delaware limited liability company, having an address at 385 E. Colorado Boulevard, Suite 299, 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, as amended (collectively, the “**Lease**”), wherein Landlord leased to Tenant certain premises containing approximately 132,487 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 5,975 rentable square feet on the first floor of the Building as identified as the “**REGENXBIO EXPANSION**” on the page of **Exhibit A** attached hereto labeled “**1ST FLOOR BOMA PLAN**” (“**Expansion Premises**”), (ii) adjust certain definitions contained in the Basic Lease Provisions to reflect the addition of the Expansion Premises, (iii) modify the amount of the TI Allowance based on certain re-measurements and the addition of the Expansion Premises, and (iv) eliminate the On Site Food Service Area, all on the terms and conditions set forth in this First Amendment.

AGREEMENT

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

1. **Definitions; Recitals.** Terms used in this First Amendment but not otherwise defined shall have the meanings set forth in the Lease. The Recitals form an integral part of this First 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 Expansion Premises, (ii) **Exhibit A** to this First Amendment, which depicts the Expansion Premises, the balance of the Premises, and Landlord’s management office on the first (1st) floor of the Building (identified as “**ARE**” on the page of **Exhibit A** attached hereto labeled “**1ST FLOOR BOMA PLAN**”), 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:



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That portion of the Project, containing approximately 139,281 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 12,712 rentable square feet located on the first floor of the Building ("**1st Floor Premises**"), (ii) approximately 40,198 rentable square feet located on the third floor of the Building ("**3rd Floor Premises**"), (iii) approximately 40,198 rentable square feet located on the 4th floor of the Building ("**4th Floor Premises**"), (iv) approximately 40,198 rentable square feet located on the 5th floor of the Building ("**5th Floor Premises**"), and (v) approximately 5,975 rentable square feet located on the first floor of the Building ("**Expansion Premises**"). The 1st Floor Premises, the 3rd Floor Premises, the 5th Floor Premises, and the Expansion Premises are collectively referred to as the "**Initial Premises**," and the Initial Premises and 4th Floor Premises are collectively referred to as the "**Premises**."

and by (ii) replacing the word "2%" wherever it appears in the second (2nd) paragraph thereof, with the word "1.5%".

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) 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: 139,281 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: 459,269 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:	176,833 rentable square feet
Total:	<u>459,269 rentable square feet</u>



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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: 78.76%, 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: 30.33%, 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: 176,833 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%, subject to adjustment upon confirmation of the rentable area of the Building as provided above.

3. **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:

(b) **TI Allowance.** Landlord shall provide to Tenant a tenant improvement allowance ("**TI Allowance**") of **\$110** per rentable square foot of the Premises, or **\$15,320,910** in the aggregate (based on the Premises containing 139,281 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.



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4. **Elimination of On Site Food Service.** Landlord and Tenant have agreed to eliminate the need for the On Site Food Service and the On Site Food Service Area. Accordingly, effective as of the Effective Date, the following changes are hereby made to the Lease for the purpose of deleting references to the On Site Food Service and the On Site Food Service Area:

4.1 Section 2(f) of the Lease is hereby deleted in its entirety and replaced with the following: "Intentionally Deleted."

4.2 The second paragraph of Section 5 of the Lease is hereby amended by deleting the language beginning on line 14 (i.e., "Rent, the cost to repair. . .") to the end of that paragraph and replacing it with the following:

Rent, and the cost to repair or replace exterior glass, caulking, or brick, the cost of any tuck pointing, excluding only:

4.3 Section 39(a) of the Lease is hereby amended by deleting the penultimate sentence and replacing it with the following sentence:

In no event shall the Available Space include any space on the first floor for Landlord's management office.

4.4 Section 40(a) of the Lease is hereby amended by deleting the third sentence and replacing it with the following sentence:

In no event shall the Expansion Space include any space on the first floor for Landlord's management office.

4.5 Section 41 of the Lease is hereby amended by deleting the second sentence and replacing it with the following sentence:

The provisions of this Section shall not apply, however, to any space on the first floor for Landlord's management office.

5. **Miscellaneous.**

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

5.2 **Binding Effect.** This First 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.



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5.3 **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 First 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 First Amendment.

5.4 **Counterparts.** This First 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 E-SIGN 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 First Amendment and all matters related thereto, with such electronic signatures having the same legal effect as original signatures.

5.5 **Ratification; Conflicts.** Except as amended and/or modified by this First 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 First Amendment. In the event of any conflict between the provisions of this First Amendment and the provisions of the Lease, the provisions of this First Amendment shall prevail. Regardless of whether specifically amended by this First 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 First Amendment.

[SIGNATURES APPEAR ON NEXT PAGE]



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IN WITNESS WHEREOF, the parties hereto have executed this First 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

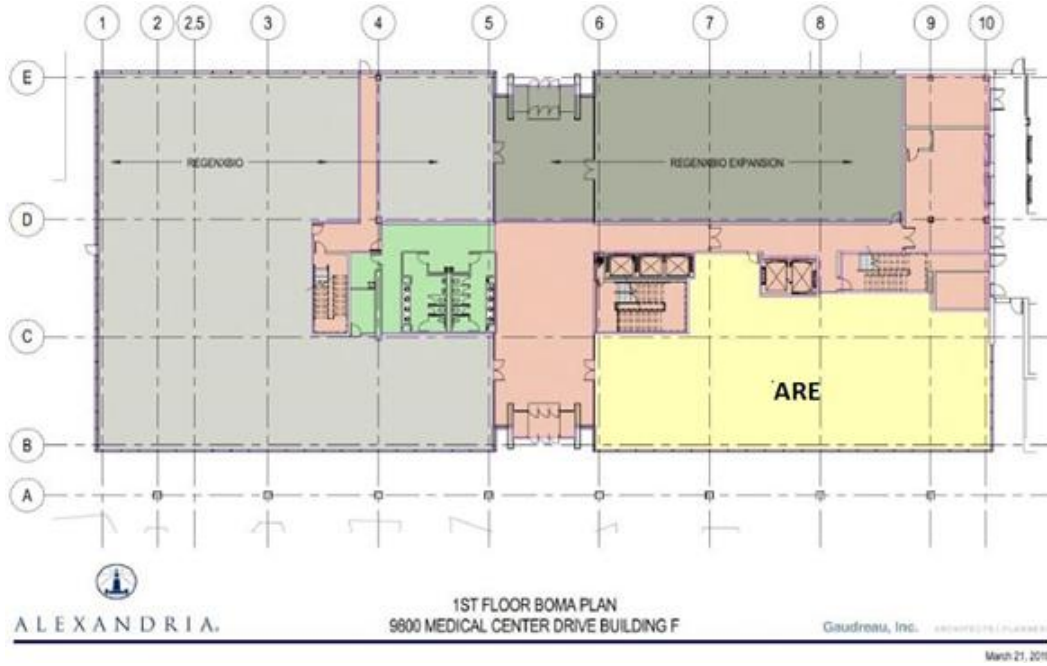
By: /s/ Jackie Clem (SEAL)
Name: Jackie Clem
Title: Senior Vice President
RE Legal Affairs



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EXHIBIT A DESCRIPTION OF PREMISES

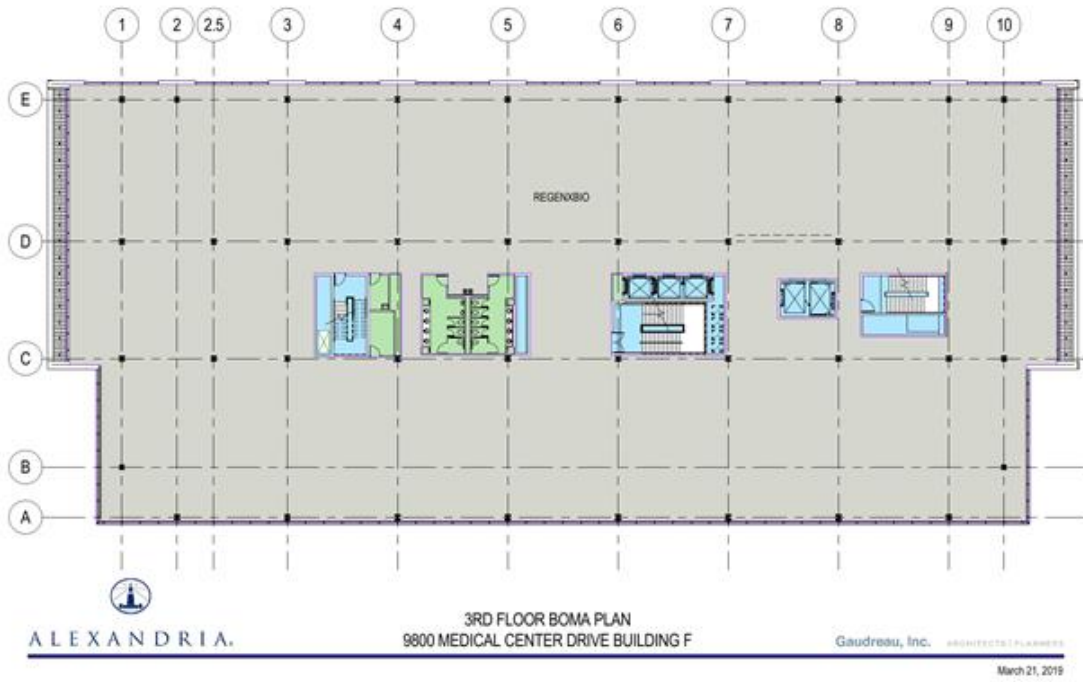


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EXHIBIT A
DESCRIPTION OF PREMISES—continued

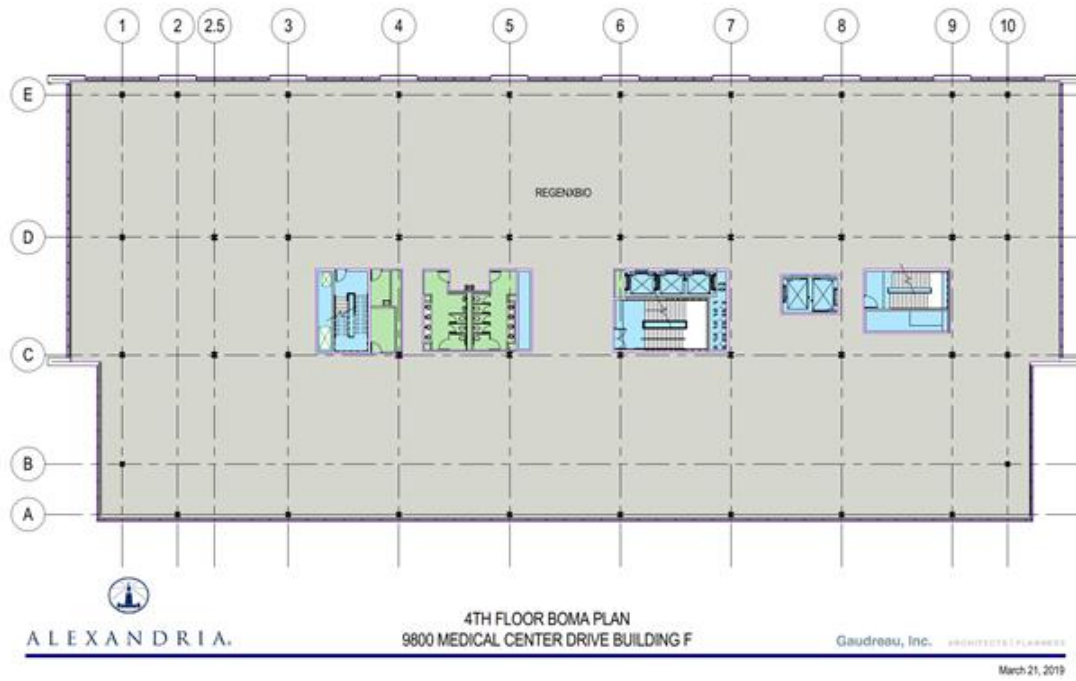


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EXHIBIT A
DESCRIPTION OF PREMISES—continued

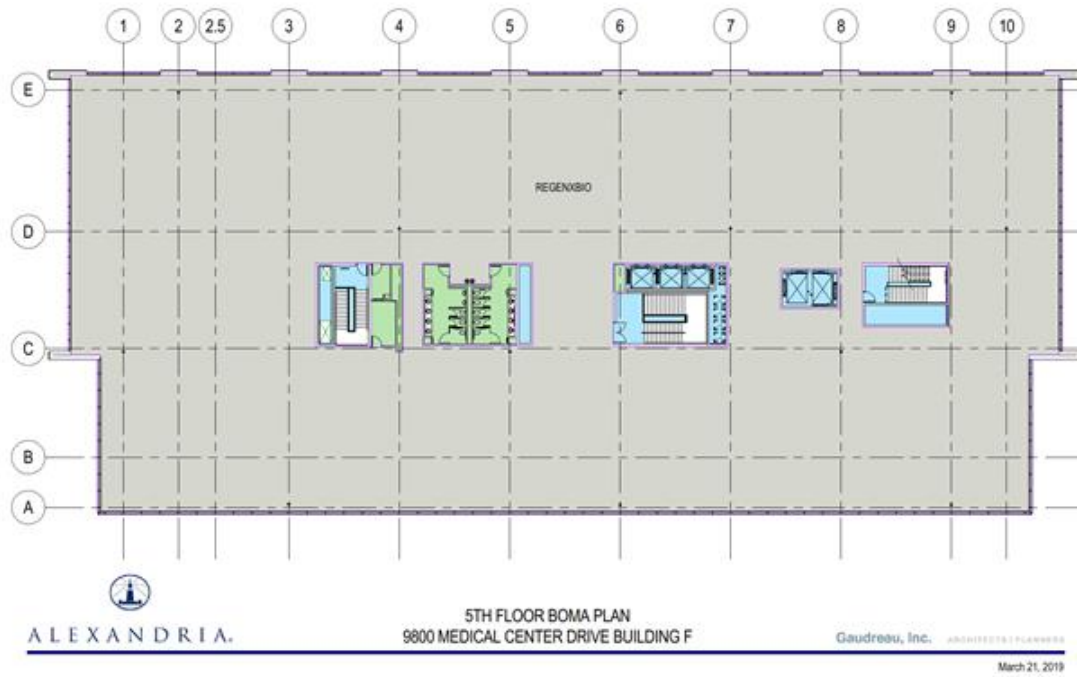


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EXHIBIT A
DESCRIPTION OF PREMISES—continued



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CERTIFICATION

I, Kenneth T. Mills, certify that:

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

Date: May 7, 2019

/s/ Kenneth T. Mills

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

CERTIFICATION

I, Vittal Vasista, certify that:

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

Date: May 7, 2019

/s/ Vittal Vasista

Vittal Vasista

Chief Financial Officer

(Principal Financial and Accounting Officer)

CERTIFICATION

In connection with the Quarterly Report of REGENXBIO Inc. (the "Registrant") on Form 10-Q for the quarter ended March 31, 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: May 7, 2019

/s/ Kenneth T. Mills

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

Date: May 7, 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.