

REGENXBIO Inc.
Form 10-Q
November 09, 2016

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2016

OR

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

Commission File Number 001-37553

REGENXBIO Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	47-1851754
(State or other jurisdiction of	(I.R.S. Employer
incorporation or organization)	Identification No.)

9600 Blackwell Road, Suite 210

Rockville, MD	20850
(Address of principal executive offices)	(Zip Code)

(240) 552-8181

(Registrant's telephone number, including area code)

Not Applicable

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

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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

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

Large accelerated filer

Accelerated filer

Non-accelerated filer (Do not check if a smaller reporting company) Smaller reporting company

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

As of November 4, 2016, there were 26,475,379 outstanding shares of the registrant's common stock, \$0.0001 par value per share.

REGENXBIO INC.

QUARTERLY REPORT ON FORM 10-Q

FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2016

TABLE OF CONTENTS

PART I—FINANCIAL INFORMATION

Item 1.	<u>Financial Statements (Unaudited)</u>	2
	<u>Balance Sheets as of September 30, 2016 and December 31, 2015</u>	2
	<u>Statements of Operations and Comprehensive Loss for the Three and Nine Months Ended September 30, 2016 and 2015</u>	3
	<u>Statements of Cash Flows for the Nine Months Ended September 30, 2016 and 2015</u>	4
	<u>Notes to the Financial Statements</u>	5
Item 2.	<u>Management’s Discussion and Analysis of Financial Condition and Results of Operations</u>	17
Item 3.	<u>Quantitative and Qualitative Disclosures About Market Risk</u>	25
Item 4.	<u>Controls and Procedures</u>	25

PART II—OTHER INFORMATION

Item 1.	<u>Legal Proceedings</u>	27
Item 1A.	<u>Risk Factors</u>	27
Item 2.	<u>Unregistered Sales of Equity Securities and Use of Proceeds</u>	67
Item 3.	<u>Defaults Upon Senior Securities</u>	67
Item 4.	<u>Mine Safety Disclosures</u>	67
Item 5.	<u>Other Information</u>	67
Item 6.	<u>Exhibits</u>	67
	<u>Signatures</u>	68

INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains “forward-looking statements” that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q, including statements regarding our future results of operations and financial position, strategy and plans, and our expectations for future operations, are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek” the negative version of these words and similar expressions are intended to identify forward-looking statements. We have based these forward-looking statements on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, strategy, short- and long-term business operations and objectives, and financial needs. These forward-looking statements are subject to a number of risks, uncertainties, assumptions and other important factors, including those described in the sections titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” included in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2015, filed with the Securities and Exchange Commission on March 3, 2016. In light of these risks, uncertainties, assumptions and other factors, the forward-looking events and circumstances discussed in this Quarterly Report on Form 10-Q or our Annual Report on Form 10-K may not occur, and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

Forward-looking statements include, but are not limited to, statements about:

- the timing of enrollment, commencement and completion of our clinical trials;
- the timing and success of preclinical studies and clinical trials conducted by us and our development partners;
- 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.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, level of activity, performance or achievements. All of our development timelines could be subject to adjustment depending on recruitment rates, regulatory agency review, and other factors that could delay the initiation and completion of our clinical trials. Any forward-looking statement made by us in this Quarterly Report on Form 10-Q speaks only as of the date of this report. Except as required by law, we disclaim any duty to update any of these forward-looking statements after the date such statements are made, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

We encourage you to read the discussion and analysis of our financial condition and our financial statements contained in this Quarterly Report on Form 10-Q. We also encourage you to read Item 1A of Part II this Quarterly Report on Form 10-Q, entitled “Risk Factors,” which contains a more complete discussion of the risks and uncertainties

associated with our business. In addition to the risks described above and in Item 1A of Part II of this Quarterly Report on Form 10-Q, other unknown or unpredictable factors also could affect our results. There can be no assurance that the actual results or developments anticipated by us will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, us. Therefore no assurance can be given that the outcomes stated in such forward-looking statements and estimates will be achieved.

PART I – FINANCIAL INFORMATION

Item 1. Financial Statements.

REGENXBIO INC.

BALANCE SHEETS

(unaudited)

(in thousands, except per share data)

	September 30, 2016	December 31, 2015
Assets		
Current assets		
Cash and cash equivalents	\$ 28,108	\$ 54,116
Marketable securities	63,662	60,025
Accounts receivable	679	2,136
Prepaid expenses	2,171	1,020
Other current assets	2,000	851
Total current assets	96,620	118,148
Marketable securities	93,087	102,226
Property and equipment, net	5,804	538
Cost method investments	—	300
Restricted cash	225	—
Other assets	239	168
Total assets	\$ 195,975	\$ 221,380
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	\$ 5,376	\$ 1,014
Accrued expenses and other current liabilities	9,006	3,198
Advance payments	—	127
Total current liabilities	14,382	4,339
Deferred rent, net of current portion	1,367	233
Total liabilities	15,749	4,572
Commitments and contingencies (Note 6)		
Stockholders' equity		
Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued		
and outstanding at September 30, 2016 and December 31, 2015	—	—
Common stock; \$0.0001 par value; 100,000 shares authorized at September 30, 2016		
and December 31, 2015; 26,475 and 26,313 shares issued and outstanding at		
September 30, 2016 and December 31, 2015, respectively	3	3
Additional paid-in capital	274,349	269,144
Accumulated other comprehensive income (loss)	853	(719)
Accumulated deficit	(94,979)	(51,620)

Total stockholders' equity	180,226	216,808
Total liabilities and stockholders' equity	\$ 195,975	\$ 221,380

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

REGENXBIO INC.

STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited)

(in thousands, except per share data)

	Three Months		Nine Months Ended	
	Ended September 30, 2016	2015	September 30, 2016	2015
Revenues				
License revenue	\$65	\$65	\$2,638	\$635
License revenue from related party	—	1,000	—	2,000
Reagent sales	47	61	213	200
Grant revenue	13	14	42	305
Total revenues	125	1,140	2,893	3,140
Expenses				
Costs of revenues				
Licensing costs (including amounts to related parties)	13	213	528	527
Costs of reagent sales (including amounts to related parties)	22	44	101	94
Research and development (including amounts to related parties)	12,560	5,664	29,423	12,471
General and administrative (including amounts to related parties)	6,200	2,567	17,848	7,671
Other operating expenses (income)	(2)	(1)	(136)	15
Total operating expenses	18,793	8,487	47,764	20,778
Loss from operations	(18,668)	(7,347)	(44,871)	(17,638)
Other Income (Expense)				
Investment income	514	15	1,512	23
Interest expense	—	—	—	(20)
Total other income (expense)	514	15	1,512	3
Net loss	\$(18,154)	\$(7,332)	\$(43,359)	\$(17,635)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities	332	(26)	1,572	(26)
Total other comprehensive income (loss)	332	(26)	1,572	(26)
Comprehensive loss	\$(17,822)	\$(7,358)	\$(41,787)	\$(17,661)
Reconciliation of net loss to net loss applicable to common stockholders				
Net loss	\$(18,154)	\$(7,332)	\$(43,359)	\$(17,635)
Net accretion and dividends on convertible preferred stock	—	—	—	(1,747)
Net gain on extinguishment of convertible preferred stock	—	—	—	759
Net loss applicable to common stockholders	\$(18,154)	\$(7,332)	\$(43,359)	\$(18,623)
Basic and diluted net loss per common share	\$(0.69)	\$(1.52)	\$(1.64)	\$(5.48)
Weighted-average basic and diluted common shares	26,469	4,809	26,386	3,397

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

3

REGENXBIO INC.

STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	Nine Months Ended September 30,	
	2016	2015
Cash flows from operating activities		
Net loss	\$(43,359)	\$(17,635)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	5,031	2,059
Net amortization of premiums and accretion of discounts on marketable debt securities	1,502	1
Depreciation and amortization	264	43
Realized gains on sales of marketable securities	(20)	—
Unrealized foreign currency transaction gains	(2)	(4)
Imputed interest on related party promissory notes	—	13
Changes in operating assets and liabilities		
Accounts receivable	1,459	1,277
Prepaid expenses	(1,151)	(1,372)
Other current assets	(1,149)	(127)
Other assets	(71)	(128)
Accounts payable	3,595	317
Accrued expenses and other current liabilities	4,591	758
Due to related party under services agreement	—	(34)
Other related party payables	—	(3,412)
Advance payments	(127)	(26)
Deferred rent	1,302	198
Net cash used in operating activities	(28,135)	(18,072)
Cash flows from investing activities		
Restricted cash	(225)	—
Purchases of marketable securities	(32,262)	(19,065)
Maturities of marketable securities	38,131	—
Sales of marketable securities	23	—
Purchases of property and equipment	(3,714)	(394)
Net cash provided by (used in) investing activities	1,953	(19,459)
Cash flows from financing activities		
Proceeds from exercise of stock options	174	100
Proceeds from issuance of Series C convertible preferred stock, net of transaction costs	—	26,021
Proceeds from issuance of Series D convertible preferred stock, net of transaction costs	—	67,998
Proceeds from initial public offering of common stock, net of underwriting discounts and commissions	—	148,233
Issuance costs for initial public offering	—	(618)
Net cash provided by financing activities	174	241,734
Net increase (decrease) in cash and cash equivalents	(26,008)	204,203

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Cash and cash equivalents		
Beginning of period	54,116	1,121
End of period	\$28,108	\$205,324
Supplemental cash flow information		
Cash paid for interest	\$—	\$7
Supplemental disclosures of non-cash investing and financing activities		
Purchases of property and equipment in accounts payable and accrued expenses	\$1,816	\$34
Issuance costs for initial public offering in accounts payable and accrued expenses	\$—	\$2,431
Conversion of accrued service fees to related party into Series C convertible preferred stock	\$—	\$2,403
Conversion of related party promissory notes into Series C convertible preferred stock	\$—	\$1,389
Conversion of convertible preferred stock into common stock upon initial public offering	\$—	\$111,392

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

REGENXBIO INC.

NOTES TO FINANCIAL STATEMENTS

(unaudited)

(in thousands, except per share data)

1. Nature of Business

REGENXBIO Inc. (the Company) was formed on July 16, 2008 in the state of Delaware as ReGenX, LLC, and on December 22, 2009, changed its name to ReGenX Biosciences, LLC. On September 16, 2014, the Company converted from a limited liability company (LLC) to a C-corporation, and changed its name to REGENXBIO Inc. The Company is a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. The Company's proprietary 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, in the development of product candidates for a variety of diseases with unmet needs.

Initial Public Offering

On September 22, 2015, the Company completed its initial public offering (IPO) whereby the Company sold 7,245 shares of common stock (inclusive of 945 shares of common stock sold by the Company pursuant to the full exercise of an option to purchase additional shares granted to the underwriters in connection with the offering) at a price of \$22.00 per share. The shares began trading on The Nasdaq Global Select Market on September 17, 2015. The aggregate net proceeds received by the Company from the offering were \$145,184, net of underwriting discounts and commissions and offering expenses payable by the Company. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 16,298 shares of common stock.

Liquidity and Risks

As of September 30, 2016, the Company had generated an accumulated deficit of \$94,979 since inception. As the Company continues to incur losses, transition to 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 profitability, and unless and until it does, the Company will continue to need to raise additional capital. As of September 30, 2016, the Company had cash, cash equivalents and marketable securities of \$184,857, which management believes is sufficient to fund operations for at least the next 12 months.

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 preclinical manufacturing to commercial production of products.

2. Summary of Significant Accounting Policies

Basis of Presentation and Unaudited Interim Financial Information

The accompanying financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). Any reference in these notes to applicable guidance is meant to refer to the authoritative GAAP as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB). The interim unaudited financial statements have been prepared on the same basis as the annual audited financial statements as of and for the year ended December 31, 2015 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2015, filed with the Securities and Exchange Commission (SEC) on March 3, 2016. Certain information and footnote disclosures required by GAAP which are normally included in the Company's annual financial statements have been condensed or omitted pursuant to SEC rules and regulations for interim reporting. In the opinion of management, the accompanying 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 September 30, 2016, and the results of its operations and its cash flows for the interim periods ended September 30, 2016 and 2015.

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 financial statements should be read in conjunction with the audited financial statements as of and for the year ended December 31, 2015, and the notes thereto, which are included in the Company's Annual Report on Form 10-K.

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 materially differ 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 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 financial statements. Estimates are used in the following areas, among others: stock-based compensation expense, accrued research and development expenses and the fair value of financial instruments.

Restricted Cash

Restricted cash includes money market mutual funds used to collateralize an irrevocable letter of credit as required by the Company's lease agreement for its office space in New York, New York.

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

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

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

Net Loss Per Share

The Company computes net loss per share in conformity with the two-class method required for participating securities. The Company considers all series of convertible preferred stock outstanding prior to the IPO to be participating securities. The holders of convertible preferred stock outstanding prior to the IPO were entitled to receive preferential dividends in the event that a dividend was to be paid to the holders of common stock, and did not have a contractual obligation to share in the losses of the Company. As such, the Company's net losses for the three and nine months ended September 30, 2015 were not allocated to these participating securities. In connection with the IPO, all outstanding shares of convertible preferred stock were automatically converted into shares of common stock.

Basic net loss per share is calculated by dividing net loss applicable to holders of common stock by the weighted-average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting weighted-average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period,

determined using the treasury-stock method. For purposes of the diluted net loss per share calculation, convertible preferred stock, outstanding stock options and withholdings under the employee stock purchase plan are considered to be common stock equivalents but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net loss per share until the contingency has been fully met. Accordingly, basic and diluted net loss per share were the same for all periods presented.

Recently Announced Accounting Pronouncements

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes the revenue recognition requirements in ASC 605, Revenue Recognition. This ASU is based on the principle that revenue is recognized to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. In August 2015, the FASB issued ASU No. 2015-14, Revenue from Contracts with Customers (Topic 606), which deferred the effective date of the guidance under ASU No. 2014-09 by one year. In April 2016, the FASB issued ASU 2016-10, Revenue from Contracts with Customers (Topic 606): Identifying Performance Obligations and Licensing, which clarifies various aspects of Topic 606, including the identification of performance obligations and the implementation of licensing guidance. In May 2016, the FASB issued ASU 2016-12, Revenue from Contracts with Customers (Topic 606): Narrow-Scope Improvements and Practical Expedients, which clarifies various additional aspects of Topic 606, including the assessment of collectability, presentation of sales taxes and other similar taxes collected from customers, the measurement date for transactions with non-cash consideration as well as transitional issues and other technical corrections regarding the adoption of new standards under Topic 606. The standards are effective for annual and interim reporting periods beginning after December 15, 2017. Earlier application is permitted only as of annual reporting periods beginning after December 15, 2016, including interim reporting periods within that reporting period. The Company is evaluating the application of these ASU's, but has not yet determined the potential effects they may have on the Company's financial statements.

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 and net investment in leases that are not accounted for at fair value through net income to 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 annual and interim periods beginning after December 15, 2019, with early adoption permitted for annual and interim periods beginning after December 15, 2018. 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.

In March 2016, the FASB issued ASU 2016-09, Compensation—Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting, which is intended to simplify several aspects of the accounting for share-based payment awards including income tax consequences, classification of awards as either equity or liabilities and classification within the statement of cash flows. The standard is effective for annual and interim periods beginning after December 15, 2016, 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 financial position or results of operations.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842) which supersedes FASB ASC Topic 840, Leases (Topic 840) and provides principles for the recognition, measurement, presentation and disclosure of leases for both lessees and lessors. The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest

method or on a straight-line basis over the term of the lease, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of classification. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases. The standard is effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted upon issuance. The Company is evaluating the application of this ASU, but has not yet determined the potential effects it may have on the Company's financial statements.

In January 2016, the FASB issued ASU 2016-01, Financial Instruments—Overall (Topic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities, which supersedes the current guidance to classify equity securities with readily determinable fair values into different categories and requires equity securities to be measured at fair value with changes in the fair value recognized through net income (loss). The standard is effective for annual and interim periods beginning after December 15, 2017. 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.

In August 2014, the FASB issued ASU 2014-15, Presentation of Financial Statements—Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern, requiring management to evaluate whether events or conditions could impact an entity’s ability to continue as a going concern and to provide disclosures if necessary. Management will be required to perform the evaluation within one year after the date that the financial statements are issued. Disclosures will be required if conditions give rise to substantial doubt and the type of disclosure will be determined based on whether management’s plans will be able to alleviate the substantial doubt. The standard is effective for the first annual period ending after December 15, 2016, and for annual periods and interim periods thereafter with early application permitted. The Company does not believe that, upon its adoption, the application of this standard will have a material impact on the Company’s financial statement disclosures.

3. Marketable Securities

The following table presents a summary of the Company’s marketable securities, which consist solely of available-for-sale securities:

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
September 30, 2016				
Corporate bonds	\$ 155,596	\$ 292	\$ (34)	\$ 155,854
Common equity securities	300	595	—	895
	\$ 155,896	\$ 887	\$ (34)	\$ 156,749

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2015				
Corporate bonds	\$ 157,977	\$ 4	\$ (759)	\$ 157,222
Commercial paper	4,990	—	—	4,990
Common equity securities	3	36	—	39
	\$ 162,970	\$ 40	\$ (759)	\$ 162,251

As of December 31, 2015, the Company’s common equity securities consisted of shares of common stock of Dimension Therapeutics, Inc. (Dimension), which became a publicly traded company in October 2015. The Company obtained these shares in connection with a license granted to Dimension in October 2013. As of September 30, 2016, the Company had sold all of its shares of Dimension common stock.

As of September 30, 2016, the Company’s common equity securities consisted of shares of common stock of Audentes Therapeutics, Inc. (Audentes), which became a publicly traded company in July 2016. The Company obtained these shares in connection with a license granted to Audentes in July 2013. The Company is restricted from trading these securities until January 2017 pursuant to a lock-up agreement entered into in connection with Audentes’ IPO. The Company has classified these shares as available-for-sale securities and recognized an unrealized gain of \$595 which is included in accumulated other comprehensive income as of September 30, 2016. Prior to Audentes’ IPO, the shares

were not marketable and were accounted for as a cost method investment on the Company's balance sheets.

As of September 30, 2016 and December 31, 2015, 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. As of September 30, 2016 and December 31, 2015, the balance in the Company's accumulated other comprehensive income (loss) consisted solely of net unrealized gains and losses on available-for-sale securities. For the nine months ended September 30, 2016, the Company recognized net unrealized gains on available-for-sale securities of \$1,572, which is included in other comprehensive income for the period. The Company recognized realized gains of \$20 on the sale or maturity of available-for-sale securities during the nine months ended September 30, 2016, which were reclassified out of accumulated other comprehensive income (loss) during the period. The realized gains on available-for-sale securities related solely to the sale of Dimension common stock during the period.

The aggregate fair value of securities held by the Company in an unrealized loss position for less than 12 months as of September 30, 2016 and December 31, 2015 was \$40,512 and \$155,486, respectively. The Company did not hold any securities in an unrealized loss position for more than 12 months as of December 31, 2015. The aggregate fair value of securities held by the Company in an unrealized loss position for more than 12 months as of September 30, 2016 was \$4,116. The aggregate unrealized loss

for those securities in an unrealized loss position for more than 12 months as of September 30, 2016 was \$6. As of September 30, 2016, securities held by the Company which were in an unrealized loss position consisted of eleven investment grade corporate bonds. 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 September 30, 2016 or December 31, 2015.

4. Fair Value of Financial Instruments

Financial instruments reported at fair value on a recurring basis include cash equivalents and marketable securities. Cash equivalents consist solely of money market mutual funds. Marketable securities consist of corporate debt securities, including corporate bonds and commercial paper, as well as common equity securities as disclosed in Note 3. The following tables present the fair value of cash equivalents and marketable securities in accordance with the hierarchy discussed in Note 2:

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
September 30, 2016				
Money market mutual funds (cash equivalents)	\$ —	\$ 28,108	\$ —	\$ 28,108
Corporate bonds (marketable securities)	—	155,854	—	155,854
Common equity securities (marketable securities)	895	—	—	895
	\$ 895	\$ 183,962	\$ —	\$ 184,857

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2015				
Money market mutual funds (cash equivalents)	\$ —	\$ 54,104	\$ —	\$ 54,104
Corporate bonds (marketable securities)	—	157,222	—	157,222
Commercial paper (marketable securities)	—	4,990	—	4,990
Common equity securities (marketable securities)	39	—	—	39
	\$ 39	\$ 216,316	\$ —	\$ 216,355

Management estimates that the carrying amounts of its accounts receivable, accounts payable and accrued expenses and other current liabilities approximate fair value due to the short-term nature of those instruments.

The Company has determined that it is not practicable to estimate the fair value of cost method investments. The Company has not identified any events or changes in circumstances that would have an adverse effect on the fair value of its cost method investments reported as of December 31, 2015.

5. Property and Equipment, Net

Property and equipment, net consists of the following:

	September 30, 2016	December 31, 2015
Computer equipment and software	\$ 865	\$ 458
Lab equipment	756	—
Furniture and fixtures	870	105
Leasehold improvements	3,657	55
Total property and equipment	6,148	618
Accumulated depreciation and amortization	(344)	(80)
Property and equipment, net	\$ 5,804	\$ 538

6. Commitments and Contingencies

Lease Agreements

The Company recognizes rent expense on a straight-line basis over the term of its operating leases commencing on the date the Company takes possession of the leased property. Tenant improvement allowances which are considered to be lease incentives from the lessor are recorded as deferred rent and amortized as a reduction of rent expense over the term of the lease from the possession date.

In March 2015, the Company entered into a 5.5-year, non-cancelable operating lease for office space in Rockville, Maryland. The lease commenced in April 2015, and expires in September 2020. The Company has options to extend the lease for up to 6 years. Initial monthly payments required under the lease were \$24 beginning in October 2015 and escalate annually in accordance with the lease.

In September 2015, and again in November 2015, the Company amended its operating lease in Rockville, Maryland to include additional office and laboratory space and extend the term of the lease for its existing space to October 2020. The lease for the additional space commenced in April 2016, and has a 5-year term expiring in March 2021. The Company has options to extend the lease for the additional space to be coterminous with the Company's existing lease at that facility. Initial monthly payments required under the lease for the additional space were \$41 and escalate annually in accordance with the lease. The Company received a \$286 tenant improvement allowance from the landlord which will be deferred and amortized on a straight-line basis as a reduction of rent expense over the term of lease.

In January 2016, the Company entered into a 7.5-year, non-cancelable operating lease for additional office space in Rockville, Maryland. The lease commenced in February 2016, and expires in September 2023. Initial monthly payments required under the lease are \$38 beginning seven months from the commencement date and escalate annually in accordance with the lease agreement. The Company received a \$725 tenant improvement allowance from the landlord which will be deferred and amortized on a straight-line basis as a reduction of rent expense over the term of lease.

In May 2016, the Company entered into a 51-month, non-cancelable operating lease for additional office space in New York, New York. The lease commenced in July 2016, and expires in October 2020. Initial monthly payments required under the lease are \$25 beginning three months from the commencement date and escalate annually in accordance with the lease agreement. Under the terms of the lease agreement, the Company has provided the landlord with an irrevocable letter of credit of \$225 which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease. As of September 30, 2016, the Company has recorded restricted cash of \$225 as collateral to the financial institution which issued the letter of credit.

The Company entered into a short-term operating lease for office space in Gaithersburg, Maryland which expired in October 2016.

As of September 30, 2016, future minimum lease payments under non-cancelable operating leases are as follows:

	Operating Leases
2016 (remainder of year)	\$ 387
2017	1,589
2018	1,637
2019	1,685

2020	1,611
Thereafter	1,614
Total minimum lease payments	\$ 8,523

Licenses Granted to the Company

Licenses granted to the Company may require the Company to make future payments relating to sublicense fees, milestone fees for milestones achieved in the future and royalties on future sales of licensed products. Additionally, the Company may be responsible for the cost of the maintenance of the intellectual property as incurred by its licensors. Up-front fees to obtain licensed technology are included in research and development expenses and patent maintenance costs are included in general and administrative expenses in the statements of operations and comprehensive loss. Sublicense fees are based on a specified percentage of license fees earned by the Company and are included in licensing costs in the statements of operations and comprehensive loss. Royalties on sales of licensed reagents for use in research and development are included in costs of reagent sales in the statements of operations and comprehensive loss. The Company has not commercialized any product candidates or paid any royalties under these agreements other than for the sales of licensed reagents.

The Trustees of the University of Pennsylvania. In February 2009, the Company entered into a license agreement, as amended, with The Trustees of the University of Pennsylvania (Penn) for exclusive, worldwide rights to certain patents owned by Penn underlying the Company's NAV® Technology Platform. Under the terms of the agreement, in consideration for the license, the Company issued to Penn 24.5 percent of the then outstanding membership interest in the LLC on a fully diluted basis after issuance. The Company is obligated to pay Penn royalties on net sales and sublicense fees, if any. Additionally, the Company is obligated to reimburse Penn for certain costs incurred related to the maintenance of the licensed patents.

In April 2016, the Company entered into an agreement with Penn whereby the Company will fund clinical trial activities performed by Penn for RGX-501, the Company's product candidate for homozygous familial hypercholesterolemia (HoFH). In connection with the agreement, the Company amended its license from Penn to include exclusive license rights to data, results and other information generated in connection with the RGX-501 clinical trial.

Expenses incurred by the Company related to its license from Penn were as follows:

	Three Months Ended September 30, 2016		Nine Months Ended September 30, 2015	
Sublicense fees	\$7	\$107	\$264	\$264
Royalties on sales of reagents	2	2	11	8
Maintenance of licensed patents	42	29	85	118
	\$51	\$138	\$360	\$390

As of September 30, 2016 and December 31, 2015, the Company had accrued \$22 and \$440, respectively, in expenses payable to Penn under the license agreement, which are included in accounts payable and accrued expenses on the Company's balance sheets. Until September 30, 2015, the Company considered Penn to be a related party. See Note 9 for further information on related party transactions with Penn.

GlaxoSmithKline LLC. In March 2009, the Company entered into a license agreement, as amended, with GlaxoSmithKline LLC (GSK) for exclusive, worldwide rights to certain patents underlying the Company's NAV® Technology Platform which are owned by Penn and exclusively licensed to GSK. Under the terms of the agreement, in consideration for the license, the Company issued to GSK 19.9 percent of the then outstanding membership interest in the LLC on a fully diluted basis after issuance. The Company is obligated to pay GSK royalties on net sales and sublicense fees, if any. Additionally, the Company is obligated to reimburse GSK for certain costs incurred and invoiced to the Company related to the maintenance of the licensed patents. The Company is obligated to pay GSK up to \$1,500 upon the achievement of various milestones. As of September 30, 2016, no milestones have been achieved, or deemed probable of achievement, and accordingly no milestone payments were payable to GSK.

Expenses incurred by the Company related to its license from GSK were as follows:

	Three Months	Nine Months
--	-----------------	----------------

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	Ended September 30, 2016		Ended September 30, 2015	
Sublicense fees	\$7	\$107	\$264	\$264
Royalties on sales of reagents	1	1	7	5
Maintenance of licensed patents	89	57	319	474
	\$97	\$165	\$590	\$743

As of September 30, 2016 and December 31, 2015, the Company had accrued \$74 and \$526, respectively, in expenses payable to GSK under the license agreement, which are included in accounts payable and accrued expenses on the Company's balance sheets. Until September 30, 2015, the Company considered GSK to be a related party. See Note 9 for further information on related party transactions with GSK.

ARIAD Pharmaceuticals, Inc. In November 2010, the Company entered into a license agreement with ARIAD Pharmaceuticals, Inc. (ARIAD), for exclusive, worldwide rights to certain patents owned and exclusively licensed by ARIAD. In consideration for the license, the Company issued Class A Units of the LLC to ARIAD with a fair value of \$726. Under the terms of the agreement, the Company is obligated to pay ARIAD royalties on net sales, and sublicense fees, if any. Additionally, the Company is obligated to pay ARIAD up to \$2,300 and annual maintenance fees of \$50 upon the achievement of various milestones. As of September 30, 2016, no milestones have been achieved, or deemed probable of achievement, and accordingly no milestone payments or maintenance fees were payable to ARIAD. Additionally, the Company has not incurred any royalties or sublicense fees payable to ARIAD since the

inception of the agreement. There were no amounts due to ARIAD under the agreement as of September 30, 2016 and December 31, 2015.

Regents of the University of Minnesota. In November 2014, the Company entered into a license agreement with Regents of the University of Minnesota (Minnesota), for an exclusive license under certain patent rights to commercialize products covered by the licensed patent rights in any country or territory in which a licensed patent has been issued and is unexpired, or a licensed patent application is pending. In consideration for the license, the Company paid an up-front fee of \$25 and reimbursed Minnesota for patent maintenance expenses of \$9. Under the terms of the agreement, the Company is obligated to pay Minnesota annual maintenance fees between \$5 and \$15 per year on each anniversary date of the agreement. Additionally, the Company is obligated to pay royalties on net sales and sublicense fees, if any, and up to \$125 per licensed product upon the achievement of various milestones. As of September 30, 2016, no milestones have been achieved, or deemed probable of achievement, and accordingly no milestone payments were payable to Minnesota. Additionally, the Company has not incurred any royalties or sublicense fees payable to Minnesota since the inception of the agreement. As of September 30, 2016 and December 31, 2015, the Company had accrued \$25 and \$0, respectively, in patent maintenance expenses payable to Minnesota under the license agreement.

Guarantees and Indemnifications

In the normal course of business, the Company enters into agreements that contain a variety of representations and provide for general indemnification. The Company's exposure under these agreements is unknown because it involves claims that may be made against the Company in the future. To date, the Company has not paid any claims or been required to defend any action related to its indemnification obligations. As of September 30, 2016 and December 31, 2015, the Company did not have any material indemnification claims that were probable or reasonably possible and consequently has not recorded any related liabilities.

7. Significant Agreements

See Note 6 for license agreements granted to the Company.

Licenses Granted by the Company

The Company has granted a number of intellectual property licenses to other biotechnology and pharmaceutical companies. The terms of the licenses vary, however licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the Company's NAV® Technology. License agreements generally have a term equal to the life of the underlying patents and are terminable only at the option of the licensee. License agreements may require licensees to pay non-refundable up-front fees, option fees and annual maintenance fees. Additional contingent consideration under the licenses may include sublicense fees, milestone fees and royalties on net sales of commercialized 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-teen percentage of net sales by licensees.

Milestone fees are payable to the Company upon the achievement of specific clinical and regulatory developments by licensees. As of September 30, 2016, the Company's license agreements, excluding additional licenses that could be

granted upon the exercise of options by licensees, could result in aggregate milestone fees payable to the Company of up to \$500 upon the submission of preclinical regulatory filings, \$23,650 upon the commencement of various stages of clinical trials, \$37,000 upon the submission of regulatory approval filings, \$91,500 upon the approval of commercial products by regulatory agencies and \$92,000 upon the achievement of specified sales targets for licensed products.

License revenue consists of the following:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Up-front fees and option fees for commercial licenses	\$—	\$1,000	\$2,000	\$2,000
Maintenance fees for commercial licenses	65	65	395	245
Milestone fees	—	—	—	250
Research and other license revenue	—	—	243	140
	\$65	\$1,065	\$2,638	\$2,635

8. Stock-based Compensation

Stock-based Compensation Expense

The Company's stock-based compensation expense by award type is as follows:

	Three Months Ended September 30, 2016		Nine Months Ended September 30, 2015	
Stock options	\$1,800	\$1,349	\$5,002	\$2,059
Employee stock purchase plan	29	—	29	—
	\$1,829	\$1,349	\$5,031	\$2,059

The Company has recorded aggregate stock-based compensation expense in the statement of operations and comprehensive loss as follows:

	Three Months Ended September 30, 2016		Nine Months Ended September 30, 2015	
Research and development	\$814	\$1,101	\$1,824	\$1,413
General and administrative	1,015	248	3,207	646
	\$1,829	\$1,349	\$5,031	\$2,059

Stock Options

In September 2014, the Board of Directors adopted the 2014 Stock Plan (2014 Plan). In June 2015, the Board of Directors adopted the 2015 Equity Incentive Plan (2015 Plan), which became effective on September 16, 2015, the date on which the registration statement for the IPO was declared effective. The 2015 Plan replaced the 2014 Plan, and as of the effective date of the 2015 Plan, no further awards may be issued under the 2014 Plan. Any options or awards outstanding under the 2014 Plan as of the effective date of the 2015 Plan remained outstanding and effective. The initial amount of shares authorized for issuance under the 2015 Plan was 2,952. The number of authorized shares under the 2015 Plan automatically increases annually on January 1, beginning January 1, 2016, by the lesser of (i) 4% of the total number of shares of common stock outstanding on December 31 of the prior year, or (ii) a number of common shares determined by the Board of Directors. Effective January 1, 2016, the Board of Directors authorized an additional 1,053 shares to be issued under the 2015 Plan. As of September 30, 2016, the total number of shares of common stock authorized for issuance under the 2015 Plan and 2014 Plan is 7,178, of which 1,848 remain available for future grants under the 2015 Plan.

The 2014 Plan and 2015 Plan provide for the issuance of stock options, stock appreciation rights, restricted and unrestricted stock awards and performance cash awards to employees, members of the Board of Directors and consultants of the Company. No stock appreciation rights, restricted or unrestricted stock awards or performance cash awards have been granted under the 2014 Plan and 2015 Plan since the inception of the plans. Stock options under the 2014 Plan and 2015 Plan generally expire ten years following the date of grant. Options typically vest over a four year

period, but vesting provisions can vary by award based on the discretion of the Board of Directors. Certain awards issued by the Company include performance conditions that must be achieved in order for vesting to occur. Stock options under the 2014 Plan and 2015 Plan carry an exercise price at least equal to the estimated fair value of the Company's common stock on the date of grant.

Shares of common stock underlying awards previously issued under the 2014 Plan and 2015 Plan which are reacquired by the Company, withheld by the Company in payment of the purchase price, exercise price or withholding taxes, expired, cancelled due to forfeiture or otherwise terminated other than by exercise, are added to the number of shares of common stock available for issuance under the 2015 Plan. Shares available for issuance under the 2015 Plan may be either authorized but unissued shares of the Company's common stock or common stock reacquired by the Company and held in treasury. The 2015 Plan expires in June 2025, ten years from the date it was adopted by the Board of Directors, unless earlier terminated.

The following table summarizes stock option activity under the 2014 Plan and 2015 Plan:

	Shares	Weighted- average Exercise Price	Weighted- average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (a)
Outstanding at December 31, 2015	3,684	\$ 5.52	9.1	\$ 44,472
Granted	1,696	\$ 12.30		
Exercised	(162)	\$ 1.08		
Cancelled or forfeited	(178)	\$ 14.62		
Outstanding at September 30, 2016	5,040	\$ 7.62	8.7	\$ 58,033
Exercisable at September 30, 2016	1,898	\$ 3.26	8.3	\$ 23,110
Vested and expected to vest at September 30, 2016	5,040	\$ 7.62	8.7	\$ 58,033

(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 of options granted during the nine months ended September 30, 2016 was \$8.18. During the nine months ended September 30, 2016, the total number of stock options exercised was 162, resulting in total proceeds of \$174. The total intrinsic value of options exercised during the nine months ended September 30, 2016 was \$1,692.

Stock Options Granted to Employees. The fair value of options granted to employees was estimated at the date of grant using the Black-Scholes valuation model with the following weighted-average assumptions:

	Nine Months Ended September 30, 2016 2015	
Expected volatility	75 %	67 %
Expected term (in years)	6.2	6.1
Risk-free interest rate	1.5 %	1.7 %
Expected dividend yield		