

REGENXBIO Inc.  
Form 10-Q  
May 05, 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 March 31, 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 (State or other jurisdiction of incorporation or organization)	47-1851754 (I.R.S. Employer Identification No.)
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9712 Medical Center Drive, Suite 100

Rockville, MD (Address of principal executive offices)	20850 (Zip Code)
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(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 May 4, 2016, there were 26,338,329 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 MARCH 31, 2016

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## 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 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;
- 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 federal, state and foreign regulatory requirements;
- regulatory developments in the United States and foreign countries; and
- our plans for the use of our cash and cash equivalents.

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. 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.

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## PART I – FINANCIAL INFORMATION

## Item 1. Financial Statements.

## REGENXBIO INC.

## BALANCE SHEETS

(unaudited)

(in thousands, except per share data)

	March 31, 2016	December 31, 2015
<b>Assets</b>		
<b>Current assets</b>		
Cash and cash equivalents	\$ 35,511	\$ 54,116
Marketable securities	65,544	60,025
Accounts receivable	2,036	2,136
Prepaid expenses	1,189	1,020
Other current assets	1,300	851
<b>Total current assets</b>	<b>105,580</b>	<b>118,148</b>
Marketable securities	107,553	102,226
Property and equipment, net	1,328	538
Cost method investments	300	300
Other assets	223	168
<b>Total assets</b>	<b>\$ 214,984</b>	<b>\$ 221,380</b>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities</b>		
Accounts payable	\$ 1,113	\$ 1,014
Accrued expenses and other current liabilities	4,633	3,198
Advance payments	35	127
<b>Total current liabilities</b>	<b>5,781</b>	<b>4,339</b>
Deferred rent, net of current portion	602	233
<b>Total liabilities</b>	<b>6,383</b>	<b>4,572</b>
Commitments and contingencies (Note 6)		
<b>Stockholders' equity</b>		
Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at March 31, 2016; no shares authorized, issued and outstanding at December 31, 2015	—	—
Common stock; \$0.0001 par value; 100,000 shares authorized at March 31, 2016 and December 31, 2015; 26,338 and 26,313 shares issued and outstanding at March 31, 2016 and December 31, 2015, respectively	3	3
Additional paid-in capital	270,711	269,144
Accumulated other comprehensive income (loss)	275	(719)

Accumulated deficit	(62,388 )	(51,620 )
Total stockholders' equity	208,601	216,808
Total liabilities and stockholders' equity	\$214,984	\$ 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 Ended March 31,	
	2016	2015
<b>Revenues</b>		
License revenue	\$ 328	\$ 100
Reagent sales	59	104
Grant revenue	6	440
Total revenues	393	644
<b>Expenses</b>		
<b>Costs of revenues</b>		
Licensing costs (including amounts to related parties)	66	20
Costs of reagent sales (including amounts to related parties)	30	33
Research and development (including amounts to related parties)	6,183	2,791
General and administrative (including amounts to related parties)	5,479	1,716
Other operating expenses (income)	(114 )	77
Total operating expenses	11,644	4,637
Loss from operations	(11,251)	(3,993)
<b>Other Income (Expense)</b>		
Investment income	483	2
Interest expense	—	(20 )
Total other income (expense)	483	(18 )
Net loss	\$(10,768)	\$(4,011)
<b>Other Comprehensive Income</b>		
Unrealized gain on available-for-sale securities	994	—
Total other comprehensive income	994	—
Comprehensive loss	\$(9,774 )	\$(4,011)
<b>Reconciliation of net loss to net loss applicable to common stockholders</b>		
Net loss	\$(10,768)	\$(4,011)
Net decretion and dividends on convertible preferred stock	—	755
Net gain on extinguishment of convertible preferred stock	—	759
Net loss applicable to common stockholders	\$(10,768)	\$(2,497)
Basic and diluted net loss per common share	\$(0.41 )	\$(0.94 )
Weighted-average basic and diluted common shares	26,327	2,645

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





## REGENXBIO INC.

## STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	Three Months Ended March 31,	
	2016	2015
Cash flows from operating activities		
Net loss	\$(10,768)	\$(4,011)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	1,546	76
Net amortization of premiums and accretion of discounts on marketable debt securities	471	—
Depreciation and amortization	48	—
Unrealized foreign currency transaction losses (gains)	(13)	121
Imputed interest on related party promissory notes	—	13
Changes in operating assets and liabilities		
Accounts receivable	113	331
Prepaid expenses	(169)	(24)
Other current assets	(449)	—
Other assets	(55)	(11)
Accounts payable	(182)	419
Accrued expenses and other current liabilities	1,210	616
Other related party payables	—	795
Advance payments	(92)	—
Deferred rent	453	—
Net cash used in operating activities	(7,887)	(1,675)
Cash flows from investing activities		
Purchases of marketable securities	(20,429)	—
Maturities of marketable securities	10,106	—
Purchases of property and equipment	(416)	(106)
Net cash used in investing activities	(10,739)	(106)
Cash flows from financing activities		
Proceeds from exercise of stock options	21	7
Proceeds from issuance of Series C convertible preferred stock, net of transaction costs	—	26,021
Net cash provided by financing activities	21	26,028
Net increase (decrease) in cash and cash equivalents	(18,605)	24,247
Cash and cash equivalents		
Beginning of period	54,116	1,121
End of period	\$35,511	\$25,368
Supplemental cash flow information		
Cash paid for interest	\$—	\$7
Supplemental disclosures of non-cash investing and financing activities		
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
Purchases of property and equipment in accounts payable and accrued expenses	\$422	\$65

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

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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 March 31, 2016, the Company had generated an accumulated deficit of \$62,388 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 March 31, 2016, the Company had cash, cash equivalents and marketable securities of \$208,608, 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 United States generally accepted accounting principles 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 March 31, 2016, and the results of its operations and its cash flows for the interim periods ended March 31, 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.

## 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 loss for the three months ended March 31, 2015 was 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 and stock options 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 and unit were the same for all periods presented.

## Recently Announced Accounting Pronouncements

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. The standard is effective for annual and interim periods beginning after December 15, 2017, 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 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 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 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). This guidance is effective for annual and interim periods beginning after December 15, 2017. 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 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 ASU will be 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 is evaluating the application of this ASU, but has not yet determined the potential effects it may have on the Company's financial statement disclosures.

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 for entities by one year. The ASU is now 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 this ASU, but has not yet determined the potential effects it may have on the Company's financial statements.

## 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
<b>March 31, 2016</b>				
Corporate bonds	\$ 162,821	\$ 393	\$ (142 )	\$ 163,072
Commercial paper	9,998	—	—	9,998
Common equity securities	3	24	—	27
	\$ 172,822	\$ 417	\$ (142 )	\$ 173,097

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
<b>December 31, 2015</b>				
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

The Company's common equity securities consist 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. The Company is restricted from trading these securities until April 2016 pursuant to a lock-up agreement entered into in connection with Dimension's IPO. The Company has classified these shares as available-for-sale securities and recognized an unrealized gain of \$24 which is included in accumulated other comprehensive income as of March 31, 2016. Prior to Dimension's IPO, the shares were not marketable and were accounted for as a cost method investment on the Company's balance sheets.

As of March 31, 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 March 31, 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 three months ended March 31, 2016, the Company recognized net unrealized gains on available-for-sale securities of \$994, which is included in other comprehensive income. There were no realized gains or losses recognized on the sale or maturity of available-for-sale securities during the three months ended March 31, 2016, and as a result, the Company did not reclassify any amounts out of accumulated other comprehensive income for the period. The Company did not hold any available-for-sale securities during the three months ended March 31, 2015.

As of March 31, 2016 and December 31, 2015, the Company did not hold any available-for-sale securities in an unrealized loss position for more than 12 months. The aggregate fair value of securities held by the Company in an unrealized loss position for less than 12 months as of March 31, 2016 and December 31, 2015 was \$74,706 and \$155,486, respectively. As of March 31, 2016, securities held by the Company which were in an unrealized loss

position for less than 12 months consisted of 20 investment grade corporate bonds. The Company has the intent and ability to hold such securities until recovery and has determined that none of these investments were other-than-temporarily impaired as of March 31, 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
<b>March 31, 2016</b>				
Money market mutual funds (cash equivalents)	\$ —	\$ 35,511	\$ —	\$ 35,511
Corporate bonds (marketable securities)	—	163,072	—	163,072
Commercial paper (marketable securities)	—	9,998	—	9,998
Common equity securities (marketable securities)	27	—	—	27
	\$ 27	\$ 208,581	\$ —	\$ 208,608

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
<b>December 31, 2015</b>				
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.

## 5. Property and Equipment, Net

Property and equipment, net consists of the following:

	March 31, 2016	December 31, 2015
Computer equipment and software	\$ 562	\$ 458
Furniture and fixtures	309	105
Leasehold improvements	585	55
Total property and equipment	1,456	618
Accumulated depreciation and amortization	(128 )	(80 )
Property and equipment, net	\$ 1,328	\$ 538

The Company recorded \$48 of depreciation and amortization expense for the three months ended March 31, 2016. No depreciation or amortization expense was recorded for the three months ended March 31, 2015 because the Company's resources used in operations were provided by a related party (Note 9).

## 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 January 2015, the Company entered into an operating lease with FoxKiser LLP (FoxKiser) for office space in Washington, D.C. The lease agreement, which had a month-to-month term, required monthly payments of \$20. The lease was terminated in April 2015.

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 are \$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, if used by the Company, will be deferred and amortized on a straight-line basis as a reduction of rent expense over the term of lease.

The Company has entered into various short-term operating leases for office and laboratory space in Gaithersburg, Maryland, Philadelphia, Pennsylvania and New York, New York, which expire at various dates through July 2016.

As of March 31, 2016, future minimum lease payments under non-cancelable operating leases are as follows:

	Operating Leases
2016 (remainder of year)	\$ 848
2017	1,286
2018	1,325
2019	1,365
2020	1,337
Thereafter	1,614
<b>Total minimum lease payments</b>	<b>\$ 7,775</b>

## 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 not met as of March 31, 2016 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. On February 20, 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.

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

	Three Months Ended March 31, 2016 2015	
Sublicense fees	\$ 33	\$ 10
Royalties on sales of reagents	2	1
Maintenance of licensed patents	29	10
	\$ 64	\$ 21

As of March 31, 2016 and December 31, 2015, the Company had accrued \$170 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. On March 6, 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 March 31, 2016, no milestones have been achieved 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 Ended March 31, 2016 2015	
Sublicense fees	\$33	\$10
Royalties on sales of reagents	1	1
Maintenance of licensed patents	92	114
	\$126	\$125



As of March 31, 2016 and December 31, 2015, the Company had accrued \$233 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. On November 19, 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 March 31, 2016, no milestones have been achieved 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. The Company had no amounts due to ARIAD under the agreement as of March 31, 2016 and December 31, 2015.

Regents of the University of Minnesota. On November 10, 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 March 31, 2016, no milestones have been achieved 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. The Company had no amounts due to Minnesota under the agreement as of March 31, 2016 and December 31, 2015.

#### 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 March 31, 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 March 31, 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, \$16,650 upon the commencement of various stages of clinical trials, \$28,000 upon the submission of regulatory approval filings, \$73,500 upon the approval of commercial products by regulatory agencies and \$47,000 upon the achievement of specified sales targets for licensed products.

License revenue consists of the following:

Three  
Months  
Ended  
March 31,

	2016	2015
Maintenance fees for commercial licenses	\$250	\$100
Research and other license revenue	78	—
	\$328	\$100

## 8. Stock-based Compensation

### Equity Incentive Plans

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 at 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 March 31, 2016, the total number of shares of common stock authorized for issuance under the 2015 Plan and 2014 Plan is 7,178, of which 2,307 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 Company's equity incentive plans:

	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	1,098	\$ 13.07		
Exercised	(25 )	\$ 0.85		
Cancelled or forfeited	(39 )	\$ 12.41		
Outstanding at March 31, 2016	4,718	\$ 7.24	9.1	\$ 26,556
Exercisable at March 31, 2016	1,446	\$ 1.70	8.6	\$ 13,534
Vested and expected to vest at March 31, 2016	4,718	\$ 7.24	9.1	\$ 26,556

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated 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 three months ended March 31, 2016 was \$8.75. During the three months ended March 31, 2016, the total number of stock options exercised was 25, resulting in total proceeds of \$21. The total intrinsic value of options exercised during the three months ended March 31, 2016 was \$295.

Stock-based compensation expense for the three months ended March 31, 2016 and December 31, 2015, relates solely to stock options granted under the 2014 Plan and the 2015 Plan. The Company has recorded aggregate stock-based compensation expense related to the issuance of stock option awards to employees and non-employees in the statement of operations and comprehensive loss as follows:

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	Three Months Ended March 31,	
	2016	2015
Research and development	\$ 392	\$ 24
General and administrative	1,154	52
	\$	