REGENXBIO Inc.
Form 10-Q
August 08, 2017
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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 June 30, 2017

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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer		Accelerated filer
Non-accelerated filer	(Do not check if a smaller reporting company)	Smaller reporting company
		Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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

As of August 4, 2017, there were 30,894,443 outstanding shares of the registrant's common stock, $0.0001 par value per share.

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REGENXBIO INC.

QUARTERLY REPORT ON FORM 10-Q

FOR THE QUARTERLY PERIOD ENDED JUNE 30, 2017

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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 (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). The words "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "c "potential," "predict," "seek," "should," "would" or 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, 2016, which we filed with the Securities and Exchange Commission (the SEC) on March 7, 2017. 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.

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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.

As used in this Quarterly Report on Form 10-Q, the terms “REGENXBIO,” “Registrant,” “we,” “us,” and “our” mean REGENXBIO Inc. and its subsidiary, on a consolidated basis, unless the context indicates otherwise.

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PART I – FINANCIAL INFORMATION

Item 1. Financial Statements.

REGENXBIO INC.

CONSOLIDATED BALANCE SHEETS

(unaudited)

(in thousands, except per share data)

	June 30, 2017	December 31, 2016
Assets		
Current assets		
Cash and cash equivalents	$57,649	$24,840
Marketable securities	104,434	64,714
Accounts receivable	50	1,032
Prepaid expenses	2,432	1,775
Other current assets	1,252	1,010
Total current assets	165,817	93,371
Marketable securities	46,417	69,412
Property and equipment, net	11,524	9,324
Restricted cash	225	225
Other assets	393	400
Total assets	$224,376	$172,732
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$3,948	$1,543
Accrued expenses and other current liabilities	7,514	8,126
Total current liabilities	11,462	9,669
Deferred rent, net of current portion	1,217	1,326
Total liabilities	12,679	10,995
Commitments and contingencies (Note 6)		
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at June 30, 2017 and December 31, 2016	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at June 30, 2017 and December 31, 2016; 30,853 and 26,477 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	3	3
Additional paid-in capital	363,393	276,354

Accumulated other comprehensive loss	(646)	(33)
Accumulated deficit	(151,053)	(114,587)
Total stockholders' equity	211,697	161,737
Total liabilities and stockholders' equity	$224,376	$172,732

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

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REGENXBIO INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(unaudited)

(in thousands, except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Revenues				
License revenue	$6,555	$2,245	$7,010	$2,573
Reagent sales	—	107	—	166
Grant revenue	7	23	7	29
Total revenues	6,562	2,375	7,017	2,768
Expenses				
Costs of revenues				
Licensing costs	1,311	449	1,402	515
Costs of reagent sales	6	49	6	79
Research and development	13,917	10,680	30,536	16,863
General and administrative	6,355	6,169	12,977	11,648
Other operating expenses (income)	29	(20)	74	(134)
Total operating expenses	21,618	17,327	44,995	28,971
Loss from operations	(15,056)	(14,952)	(37,978)	(26,203)
Other Income				
Investment income	583	515	1,512	998
Total other income	583	515	1,512	998
Net loss	$(14,473)	$(14,437)	$(36,466)	$(25,205)
Other Comprehensive Income (Loss)				
Unrealized gain (loss) on available-for-sale securities, net of reclassifications of $480 for the six months ended June 30, 2017	(74)	246	(613)	1,240
Total other comprehensive income (loss)	(74)	246	(613)	1,240
Comprehensive loss	$(14,547)	$(14,191)	$(37,079)	$(23,965)
Net loss applicable to common stockholders	$(14,473)	$(14,437)	$(36,466)	$(25,205)
Basic and diluted net loss per common share	$(0.47)	$(0.55)	$(1.27)	$(0.96)
Weighted-average basic and diluted common shares	30,662	26,362	28,678	26,344

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

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REGENXBIO INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	Six Months Ended June 30,	
	2017	2016
Cash flows from operating activities		
Net loss	$(36,466)	$(25,205)
Adjustments to reconcile net loss to net cash used in operating activities		
Stock-based compensation expense	5,074	3,202
Net amortization of premiums and accretion of discounts on marketable debt securities	945	987
Depreciation and amortization	1,257	104
Realized gains on sales of marketable securities	(480)	—
Other non-cash adjustments	40	—
Changes in operating assets and liabilities		
Accounts receivable	982	1,353
Prepaid expenses	(657)	(460)
Other current assets	(242)	(586)
Other assets	(86)	(98)
Accounts payable	2,723	930
Accrued expenses and other current liabilities	(31)	2,982
Advance payments	—	(127)
Deferred rent	(89)	601
Net cash used in operating activities	(27,030)	(16,317)
Cash flows from investing activities		
Purchases of marketable securities	(46,593)	(32,261)
Maturities of marketable securities	28,010	26,131
Sales of marketable securities	780	—
Purchases of property and equipment	(4,609)	(1,491)
Restricted cash	—	(225)
Net cash used in investing activities	(22,412)	(7,846)
Cash flows from financing activities		
Proceeds from exercise of stock options	329	130
Proceeds from issuance of common stock under employee stock purchase plan	147	—
Proceeds from public offering of common stock, net of underwriting discounts and commissions	81,994	—
Issuance costs for public offering of common stock	(219)	—
Net cash provided by financing activities	82,251	130
Net increase (decrease) in cash and cash equivalents	32,809	(24,033)
Cash and cash equivalents		

Beginning of period	24,840	54,116
End of period	$57,649	$30,083
Supplemental disclosures of non-cash investing and financing activities		
Purchases of property and equipment in accounts payable and accrued expenses	$—	$939
Issuance costs for public offering of common stock in accounts payable and accrued expenses	$193	$—

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

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REGENXBIO INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

1. Nature of Business

REGENXBIO Inc. (the Company) is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. The Company's proprietary adeno-associated virus (AAV) gene delivery platform (NAV® Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. The Company's NAV® Technology Platform is being applied by the Company, as well as by third-party licensees, in the development of product candidates for a variety of diseases with unmet needs. The Company was formed in 2008 in the state of Delaware and is headquartered in Rockville, Maryland.

Follow-on Public Offering

On March 27, 2017, the Company completed a follow-on public offering whereby the Company sold 3,700,000 shares of common stock at a price of $20.50 per share. In connection with the offering, the Company granted the underwriters an option to purchase up to 555,000 additional shares of common stock at the public offering price. The underwriters exercised the option in full and purchased the additional shares on April 26, 2017. The aggregate net proceeds received by the Company from the offering, inclusive of the underwriters' option exercise, were $81.5 million, net of underwriting discounts and commissions and offering expenses payable by the Company.

Liquidity and Risks

As of June 30, 2017, the Company had generated an accumulated deficit of $151.1 million 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 June 30, 2017, the Company had cash, cash equivalents and marketable securities of $208.5 million, which management believes is sufficient to fund operations for at least the next 12 months from the date these consolidated financial statements were issued.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, development by the Company or its competitors of technological innovations, risks of failure of clinical trials, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to transition from clinical manufacturing to commercial production of products.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying consolidated financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). 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 consolidated financial statements have been prepared on the same basis as the annual audited consolidated financial statements as of and for the year ended December 31, 2016 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2016, which was filed with the SEC on March 7, 2017. Certain information and footnote disclosures required by GAAP which are normally included in the Company's annual consolidated financial statements have been omitted pursuant to SEC rules and regulations for interim reporting. In the opinion of management, the accompanying consolidated financial statements reflect all adjustments, which include all normal and recurring adjustments necessary for the fair statement of the Company's financial position as of June 30, 2017, and the results of its operations and its cash flows for the interim periods ended June 30, 2017 and 2016.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year, any other interim periods, or any future year or period. These interim consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2016, and the notes thereto, which are included in the Company's Annual Report on Form 10-K.

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The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiary, REGENXBIO EU Ltd., which was formed in June 2017. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Actual results could 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 consolidated financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the consolidated financial statements. 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

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, outstanding stock options, outstanding restricted stock units 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.

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Recently Announced Accounting Pronouncements

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. Additionally, the standard allows companies to elect not to estimate forfeitures of share-based awards for purposes of recognizing stock-based compensation expense, and account for forfeitures as they occur. The standard is effective for annual and interim periods beginning after December 15, 2016, with early adoption permitted upon issuance. The Company has adopted this standard effective January 1, 2017, and upon adoption, has elected not to estimate forfeitures of share-based awards, which requires retrospective application in the consolidated financial statements. The Company has estimated a forfeiture rate of zero on all share-based awards granted since its inception. Additionally, the adoption of this standard had no material impact on the Company's tax provision or classification of the Company's share-based awards. Accordingly, the application of this standard had no material impact on the Company's financial position or results of operations.

In May 2014, the FASB issued ASU 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 2015-14, Revenue from Contracts with Customers (Topic 606), which deferred the effective date of the guidance under ASU 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. In December 2016, the FASB issued ASU 2016-20, Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers, which clarifies various additional narrow aspects of 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 has elected not to early adopt these standards. The Company is currently evaluating these standards and their potential impact, and believes the standards will significantly impact the Company's revenue recognition polices for license revenue. Aspects of Topic 606 which may potentially impact the Company's policies and disclosures for license revenue include, but are not limited to, the identification of performance obligations, determination and allocation of the contract price, including variable consideration, implementation of the licensing guidance and the determination of the proper method of revenue recognition for intellectual property licenses. While the new standards will impact the Company's revenue recognition policies and disclosures, management's evaluation is not yet complete and management has not yet determined the potential effects these new standards may have on the Company's financial position or results of operations.

In May 2017, the FASB issued ASU 2017-09, Compensation – Stock Compensation (Topic 718): Scope of Modification Accounting, which clarifies when modification accounting should be applied for changes to terms or conditions of a share-based award. The standard is effective for annual and interim periods beginning after

December 15, 2017, with early adoption permitted, and is to be applied prospectively upon adoption. 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 November 2016, the FASB issued ASU 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash. The standard requires that the statement of cash flows explain the change during the period in the total of cash, cash equivalents and restricted cash. As a result, amounts generally described as restricted cash should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. The standard is effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted, and is to be applied retrospectively to each period presented. 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 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

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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 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 consolidated 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.

3. Marketable Securities

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

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
June 30, 2017				
Corporate bonds	$ 146,069	$ 18	$ (229)	$ 145,858
Commercial paper	4,993	—	—	4,993
	$ 151,062	$ 18	$ (229)	$ 150,851

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value

December 31, 2016				
Corporate bonds	$ 133,424	$ 82	$ (298)	$ 133,208
Common equity securities	300	618	—	918
	$ 133,724	$ 700	$ (298)	$ 134,126

Common equity securities reported as of December 31, 2016 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 sold all of its shares of Audentes common stock during the first quarter of 2017.

As of June 30, 2017 and December 31, 2016, 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 June 30, 2017 and December 31, 2016, the balance in the Company’s accumulated other comprehensive loss consisted solely of net unrealized gains and losses on available-for-sale securities, net of income tax effects and reclassification adjustments for realized gains and losses. During the three and six months ended June 30, 2017, the Company recognized net unrealized losses on available-for-sale securities of $0.1 million and $0.1 million, respectively, and income tax expense of $0 in other comprehensive loss for the periods. The Company recognized realized gains of $0 and $0.5 million on the sale or maturity of available-for-sale securities during the three and six months ended June 30, 2017, respectively, which were reclassified out of accumulated other comprehensive loss during the periods and are included in investment income in the consolidated statements of operations and comprehensive loss. During the three and six months ended June 30, 2016, the Company recognized net unrealized gains on available-for-sale securities of

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$0.2 million and $1.2 million, respectively, and income tax expense of $0 in other comprehensive income for the periods. The Company did not recognize any realized gains or losses on the sale or maturity of marketable securities for the three or six months ended June 30, 2016.

The Company did not hold any securities in an unrealized loss position for more than 12 months as of June 30, 2017 or December 31, 2016. The aggregate fair value of securities held by the Company in an unrealized loss position for less than 12 months as of June 30, 2017 and December 31, 2016 was $127.2 million and $102.5 million, respectively. As of June 30, 2017, securities held by the Company which were in an unrealized loss position consisted of 45 investment grade corporate bond positions. The Company has the intent and ability to hold such securities until recovery and has determined that none of its investments were other-than-temporarily impaired as of June 30, 2017 or December 31, 2016.

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, 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 (in thousands):

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
June 30, 2017				
Money market mutual funds (cash equivalents)	$ —	$ 57,649	$ —	$ 57,649
Corporate bonds (marketable securities)	—	145,858	—	145,858
Commercial paper (marketable securities)	—	4,993	—	4,993
	$ —	$ 208,500	$ —	$ 208,500

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2016				

Money market mutual funds (cash equivalents)	$ —	$ 24,840	$ —	$ 24,840
Corporate bonds (marketable securities)	—	133,208	—	133,208
Common equity securities (marketable securities)	918	—	—	918
	$ 918	$ 158,048	$ —	$ 158,966

There were no transfers of financial instruments between levels of the fair value hierarchy during the six months ended June 30, 2017.

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.

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5. Property and Equipment, Net

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

	June 30, 2017	December 31, 2016
Computer equipment and software	$1,276	$ 1,038
Lab equipment	5,896	2,780
Furniture and fixtures	1,271	1,213
Leasehold improvements	4,961	4,917
Total property and equipment	13,404	9,948
Accumulated depreciation and amortization	(1,880)	(624)
Property and equipment, net	$11,524	$ 9,324

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 at 9712 Medical Center Drive 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. Monthly payments under the lease began in October 2015 and escalate annually in accordance with the lease agreement.

In September 2015 and November 2015, the Company amended the 9712 Medical Center Drive lease to include additional office and laboratory space at 9714 Medical Center Drive and extend the term of the lease for its existing space at that facility to October 2020. The lease term 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. Monthly payments under the lease escalate annually in accordance with the lease agreement. The Company received a $0.3 million 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 its corporate headquarters at 9600 Blackwell Road in Rockville, Maryland. The lease commenced in February 2016, and expires in September 2023. Monthly payments under the lease began in September 2016 and escalate annually in accordance with the lease agreement. The Company received a $0.7 million 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 at 400 Madison Avenue in New York, New York. The lease commenced in July 2016, and expires in October 2020. Monthly payments under the lease began in October 2016 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 $0.2 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease. As of June 30, 2017, the Company has recorded restricted cash of $0.2 million as collateral to the financial institution which issued the letter of credit.

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As of June 30, 2017, future minimum lease payments under non-cancelable operating leases are as follows (in thousands):

	Operating Leases
2017 (remainder of year)	$ 802
2018	1,638
2019	1,687
2020	1,626
2021	665
Thereafter	952
Total minimum lease payments	$ 7,370

In July 2017, the Company amended the 9712 and 9714 Medical Center Drive lease to include additional office and laboratory space and extend the term of the lease for its existing space at that facility to September 2021. The lease term for the additional space commences partially in August 2017 and partially in February 2018 and is coterminous with the existing space at that facility.

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 consolidated 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 consolidated 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 consolidated 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, which has been amended from time to time, with The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn) for exclusive, worldwide rights to certain patents owned by Penn underlying the Company’s NAV® Technology Platform. Under the terms of the agreement, in consideration for the license, the Company issued to Penn a 24.5% equity interest in the Company 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 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Sublicense fees	$656	$225	$701	$257
Royalties on sales of reagents	4	7	4	9
Maintenance of licensed patents	85	14	169	43
	$745	$246	$874	$309

As of June 30, 2017 and December 31, 2016, the Company had accrued $0.7 million and $0.2 million, respectively, in expenses payable to Penn under the license agreement, which are included in accounts payable and accrued expenses on the Company's consolidated balance sheets.

GlaxoSmithKline LLC. In March 2009, the Company entered into a license agreement, which was amended in April 2009, 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

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license, the Company issued to GSK a 19.9% equity interest in the Company 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 $1.5 million upon the achievement of various milestones. As of June 30, 2017, 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 (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Sublicense fees	$656	$225	$701	$257
Royalties on sales of reagents	2	4	2	6
Maintenance of licensed patents	14	137	159	230
	$672	$366	$862	$493

As of June 30, 2017 and December 31, 2016, the Company had accrued $0.7 million and $0.4 million, respectively, in expenses payable to GSK under the license agreement, which are included in accounts payable and accrued expenses on the Company’s consolidated balance sheets.

Regents of the University of Minnesota. In November 2014, the Company entered into a license agreement, which was amended in November 2016, 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, and reimbursed Minnesota for patent maintenance expenses, for a total of less than $0.1 million. Under the terms of the agreement, the Company is obligated to pay Minnesota annual maintenance fees of less than $0.1 million 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 $0.1 million per licensed product upon the achievement of various milestones. In November 2016, the license with Minnesota was amended to include additional patent rights. In consideration for the additional patent rights, the Company paid an up-front fee of less than $0.1 million.

As of June 30, 2017, 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 license agreement. As of June 30, 2017 and December 31, 2016, the Company had accrued less than $0.1 million in patent maintenance expenses and up-front fees 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 June 30, 2017 and December 31, 2016, the Company did not have any material indemnification claims that were probable or reasonably possible and consequently has not recorded any related liabilities.

European Patent Office Proceeding

In June 2017, a third party filed an opposition with the European Patent Office challenging the validity of a European patent owned by Penn for the AAV8 vector, which the Company has exclusively licensed. This matter is in its very early stages and the Company is unable to estimate the timing or outcome of this matter but intends to assist Penn in vigorously defending this patent. As of June 30, 2017, the Company has not recorded any liabilities related to this matter.

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

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may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the Company's NAV® Technology Platform. License agreements generally have a term 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 June 30, 2017, 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 $0.5 million upon the submission of preclinical regulatory filings, $29.2 million upon the commencement of various stages of clinical trials, $48.5 million upon the submission of regulatory approval filings, $110.5 million upon the approval of commercial products by regulatory agencies and $92.0 million upon the achievement of specified sales targets for licensed products.

License revenue consists of the following (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Up-front fees and option fees for commercial licenses	$6,000	$2,000	$6,000	$2,000
Maintenance fees for commercial licenses	140	80	390	330
Research and other license revenue	415	165	620	243
	$6,555			