Regulus Therapeutics Inc.
Form 10-Q
May 08, 2015
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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

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

FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2015

or

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

FOR THE TRANSITION PERIOD FROM TO

Commission file number: 001-35670

Regulus Therapeutics Inc.

(Exact name of registrant as specified in its charter)

Delaware	**26-4738379**
(State or Other Jurisdiction of Incorporation or Organization)	**(I.R.S. Employer Identification No.)**
3545 John Hopkins Ct., Suite 210	
San Diego, CA	**92121**
(Address of Principal Executive Offices)	**(Zip Code)**

858-202-6300

(Registrant s Telephone Number, Including Area Code)

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 x 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 (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes x No ¨

Indicate by check mark whether 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 x
Non-accelerated filer ¨ (Do not check if a smaller reporting company)	Smaller reporting company ¨

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

As of April 24, 2015, the registrant had 51,017,405 shares of Common Stock ($0.001 par value) outstanding.

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REGULUS THERAPEUTICS INC.

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PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

Regulus Therapeutics Inc.

CONDENSED BALANCE SHEETS

(in thousands, except share and per share data)

	March 31, 2015 (Unaudited)	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 19,600	$ 37,327
Short-term investments	129,191	122,416
Contract and other receivables	4,859	274
Prepaid and other current assets	4,867	4,934
Total current assets	158,517	164,951
Property and equipment, net	3,444	3,568
Intangibles, net	1,087	1,150
Other assets	1,800	1,811
Total assets	$ 164,848	$ 171,480
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable	$ 2,677	$ 2,188
Accrued liabilities	5,318	4,402
Accrued compensation	1,049	2,108
Current portion of deferred revenue	5,307	3,097
Convertible note payable, at fair value		23,397
Total current liabilities	14,351	35,192
Deferred revenue, less current portion	2,769	3,252
Other long-term liabilities	903	1,022
Total liabilities	18,023	39,466
Stockholders equity:		
Common stock, $0.001 par value; 200,000,000 shares authorized, 50,762,489 and 48,944,530 shares issued and outstanding at March 31, 2015 (unaudited) and December 31, 2014, respectively	51	49
Additional paid-in capital	297,191	267,929
Accumulated other comprehensive loss	(163)	(197)

Accumulated deficit	(150,254)	(135,767)
Total stockholders equity	146,825	132,014
Total liabilities and stockholders equity	$ 164,848	$ 171,480

See accompanying notes to these condensed financial statements.

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Regulus Therapeutics Inc.

CONDENSED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share data)

	Three months ended March 31,	
	2015	2014
	(Unaudited)	
Revenues:		
Revenue under strategic alliances and collaborations	$ 4,200	$ 1,631
Total revenues	4,200	1,631
Operating expenses:		
Research and development	13,427	9,604
General and administrative	3,644	2,732
Total operating expenses	17,071	12,336
Loss from operations	(12,871)	(10,705)
Other income (expense):		
Interest and other income	199	100
Interest expense	(8)	(11)
Loss from valuation of convertible note payable	(1,811)	(2,124)
Loss before income taxes	(14,491)	(12,740)
Income tax (benefit) expense	(4)	1
Net loss	$ (14,487)	$ (12,741)
Other comprehensive loss:		
Unrealized gain (loss) on short-term investments, net	58	(43)
Comprehensive loss	$ (14,434)	$ (12,784)
Net loss per share, basic and diluted	$ (0.29)	$ (0.30)
Shares used to compute basic and diluted net loss per share	50,071,165	42,690,200

See accompanying notes to these condensed financial statements.

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Regulus Therapeutics Inc.

Condensed Statements of Cash Flows

(In thousands)

	Three months ended March 31,	
	2015	2014
	(Unaudited)	
Operating activities		
Net loss	$ (14,487)	$ (12,741)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation and amortization expense	394	354
Loss from valuation of convertible note payable	1,811	2,124
Stock-based compensation	3,103	1,400
Amortization of premium on investments, net	425	442
Loss on disposal of long-term assets	50	18
Change in operating assets and liabilities:		
Contracts and other receivables	(2,469)	(61)
Prepaid and other current assets	78	(385)
Accounts payable	471	1,519
Accrued liabilities	899	(39)
Accrued compensation	(1,059)	(438)
Deferred revenue	(389)	(1,161)
Deferred rent and other liabilities	(79)	(61)
Net cash used in operating activities	(11,252)	(9,029)
Investing activities		
Purchases of short-term investments	(37,157)	(39,780)
Maturities and sales of short-term investments	30,010	29,372
Purchases of property and equipment	(236)	(75)
Acquisition of intangibles	(7)	
Net cash used in investing activities	(7,390)	(10,483)
Financing activities		
Proceeds from issuance of common stock, net	257	9,728
Principal payments on other long-term obligations	(37)	(34)
Proceeds from exercise of stock options	695	487
Net cash provided by financing activities	915	10,181
Net decrease in cash and cash equivalents	(17,727)	(9,331)

Cash and cash equivalents at beginning of period	37,327	17,807
Cash and cash equivalents at end of period	$ 19,600	$ 8,476
Supplemental disclosure of cash flow information		
Interest paid	$ 8	$ 11
Income taxes paid	$ 1	$ 1
Supplemental disclosure of non-cash investing and financing activities		
Amounts accrued for property and equipment	$ 14	$ 301

See accompanying notes to these condensed financial statements.

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Regulus Therapeutics Inc.

NOTES TO CONDENSED FINANCIAL STATEMENTS

(Unaudited)

1. Basis of Presentation and Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited condensed financial statements have been prepared in accordance with U.S. generally accepted accounting principles (GAAP) for interim financial information and the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. In management s opinion, the accompanying financial statements reflect all adjustments, consisting of normal recurring adjustments, considered necessary for a fair presentation of the results for the interim periods presented.

Interim financial results are not necessarily indicative of results anticipated for the full year. These unaudited condensed financial statements should be read in conjunction with the Company s audited financial statements and footnotes included in our Annual Report on Form 10-K for the year ended December 31, 2014, from which the balance sheet information herein was derived.

Use of Estimates

Our condensed financial statements are prepared in accordance with GAAP, which requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues and expenses and the disclosure of contingent assets and liabilities in our financial statements and accompanying notes. Although these estimates are based on our knowledge of current events and actions we may undertake in the future, actual results may ultimately differ from these estimates and assumptions.

Revenue Recognition

Our revenues generally consist of upfront payments for licenses or options to obtain licenses in the future, milestone payments and payments for other research services under strategic alliance and collaboration agreements. We recognize revenues when all four of the following criteria are met: (1) persuasive evidence that an arrangement exists; (2) delivery of the products and/or services has occurred; (3) the selling price is fixed or determinable; and (4) collectability is reasonably assured.

Multiple element arrangements, such as our strategic alliance agreements with Sanofi and AstraZeneca AB (AstraZeneca) and our collaboration agreement with Biogen Inc. (Biogen), formerly Biogen Idec MA Inc., are analyzed to determine whether the deliverables within the agreement can be separated or whether they must be accounted for as a single unit of accounting. Deliverables under the agreement will be accounted for as separate units of accounting provided that (i) a delivered item has value to the customer on a stand-alone basis; and (ii) if the agreement includes a general right of return relative to the delivered item, delivery or performance of the undelivered item is considered probable and substantially in the control of the vendor. The allocation of consideration amongst the deliverables under the agreement is derived using a best estimate of selling price if vendor specific objective evidence and third-party evidence of fair value is not available. If the delivered element does not have stand-alone value, the arrangement is then accounted for as a single unit of accounting, and we recognize the consideration received under

the arrangement as revenue on a straight-line basis over our estimated period of performance, which for us is often the expected term of the research and development plan.

Milestones

We apply the milestone method of accounting to recognize revenue from milestone payments when earned, as evidenced by written acknowledgement from the collaborator or other persuasive evidence that the milestone has been achieved and the payment is non-refundable, provided that the milestone event is substantive. A milestone event is defined as an event (i) that can only be achieved based in whole or in part on either our performance or on the occurrence of a specific outcome resulting from our performance; (ii) for which there is substantive uncertainty at the inception of the arrangement that the event will be achieved; and (iii) that would result in additional payments being due to us. Events for which the occurrence is either contingent solely upon the passage of time or the result of a counterparty s performance are not considered to be milestone events. A milestone event is substantive if all of the following conditions are met: (i) the consideration is commensurate with either our performance to achieve the milestone, or the enhancement of the value to the delivered item(s) as a result of a specific outcome resulting from our performance to achieve the milestone; (ii) the consideration relates solely to past performance; and (iii) the consideration is reasonable relative to all the deliverables and payment terms (including other potential milestone consideration) within the arrangement.

We assess whether a milestone is substantive at the inception of each arrangement. If a milestone is deemed non-substantive, we will account for that milestone payment in accordance with the multiple element arrangements guidance and recognize revenue consistent with the related units of accounting for the arrangement over the related performance period.

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Deferred Revenue

Amounts received prior to satisfying the above revenue recognition criteria are recorded as deferred revenue in the accompanying balance sheets. Amounts not expected to be recognized within the next 12 months are classified as non-current deferred revenue.

Stock-Based Compensation

We account for stock-based compensation expense related to stock options granted to employees and members of our board of directors by estimating the fair value of each stock option on the date of grant using the Black-Scholes model. We recognize stock-based compensation expense using the accelerated multiple-option approach. Under the accelerated multiple-option approach (also known as the graded-vesting method), we recognize compensation expense over the requisite service period for each separately vesting tranche of the award as though the award was in substance multiple awards, resulting in accelerated expense recognition over the vesting period. For performance-based awards granted to employees (i) the fair value of the award is determined on the grant date, (ii) we assess the probability of the individual milestones under the award being achieved and (iii) the fair value of the shares subject to the milestone is expensed over the implicit service period commencing once management believes the performance criteria is probable of being met.

We account for stock options granted to non-employees using the fair value approach. Stock options granted to non-employees are subject to periodic revaluation over their vesting terms.

Fair Value Option

Applicable accounting policies permit entities to choose, at specified election dates, to measure specified items at fair value if the decision about the election is: 1) applied instrument by instrument, 2) irrevocable, and 3) applied to an entire instrument.

In July 2012, we amended and restated the $5.0 million convertible promissory note originally issued in February 2010 to Glaxo Group Limited (GSK) (the 2010 GSK Note), which resulted in a debt extinguishment for accounting purposes. Concurrently with the debt extinguishment, we elected the fair value option for the 2010 GSK Note. The difference between the carrying value of the 2010 GSK Note and the fair value of the amended and restated 2010 GSK Note was recorded as a loss on extinguishment of debt to non-operating earnings. Thereafter, any change to the fair value of the amended note was recorded as gain (loss) from valuation of convertible note payable to non-operating earnings.

The amended and restated 2010 GSK Note provided for a rollover into a new promissory note, effective as of the closing date of a qualifying initial public offering (the Post-IPO GSK Note). In October 2012, upon our initial public offering, the Post-IPO GSK Note was established in the principal amount of $5.4 million, which was equivalent to the original principal amount of $5.0 million plus accrued but unpaid interest of approximately $0.4 million. The 2010 GSK Note was simultaneously cancelled and obligations thereunder were terminated. In January 2015, the principal balance of the Post-IPO GSK Note was converted into common stock.

Clinical Trial and Pre-Clinical Study Accruals

We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on the facts and circumstances known to us at that time. Our accrued expenses for pre-clinical studies and clinical trials are based on estimates of costs incurred and fees that may be associated with services provided by clinical trial

investigational sites, clinical research organizations (CROs) and other clinical trial-related vendors. Payments under certain contracts with such parties depend on factors such as successful enrollment of patients, site initiation and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If possible, we obtain information regarding unbilled services directly from these service providers. However, we may be required to estimate these services based on other information available to us. If we underestimate or overestimate the activity or fees associated with a study or service at a given point in time, adjustments to research and development expenses may be necessary in future periods. Historically, our estimated accrued liabilities have approximated actual expense incurred. Subsequent changes in estimates may result in a material change in our accruals.

Recent Accounting Pronouncements

In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers* (ASU 2014-19). Adoption of ASU No. 2014-09 requires that an entity recognize revenue 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. This update is effective for annual reporting periods beginning after December 15, 2016 and interim periods therein and requires expanded disclosures. We are currently evaluating the impact of adoption on our financial position, results of operations and cash flows.

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In August 2014, the FASB issued ASU No. 2014-15, *Presentation of Financial Statements Going Concern*, which requires management to assess an entity s ability to continue as a going concern, and to provide related footnote disclosure in certain circumstances. This standard is effective for annual reporting periods ending after December 15, 2016 and interim periods thereafter. Early application is permitted. The adoption of this guidance will have no impact on our financial position, results of operations or cash flows.

2. Net Loss Per Share

Basic net loss per share is calculated by dividing the net loss by the weighted average number of common shares outstanding for the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by dividing the net loss by the weighted-average number of common share equivalents outstanding for the period determined using the treasury-stock method. Dilutive common stock equivalents are comprised of options outstanding under our stock option plan and convertible note payable. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted shares outstanding due to our net loss position.

Potentially dilutive securities not included in the calculation of diluted net loss per share because to do so would be anti-dilutive are as follows (in common equivalent shares):

	Three months ended March 31,	
	2015	**2014**
Common stock options	2,813,279	2,350,252
Convertible note payable		1,425,127
Total	2,813,279	3,775,379

3. Investments

We invest our excess cash in commercial paper and debt instruments of financial institutions and corporations. As of March 31, 2015, our short-term investments had a weighted average maturity of less than two years.

The following tables summarize our short-term investments (in thousands):

As of March 31, 2015	**Maturity (in years)**	**Amortized cost**	**Unrealized Gains**	**Unrealized Losses**	**Estimated fair value**
Corporate debt securities	2 or less	$ 106,842	$ 6	$ (114)	$ 106,734
Certificates of deposit	2 or less	17,960			17,960
Commercial paper	1 or less	4,495	2		4,497
Total		$ 129,297	$ 8	$ (114)	$ 129,191

As of December 31, 2014	**Maturity (in years)**	**Amortized cost**	**Unrealized Gains**	**Unrealized Losses**	**Estimated fair value**

Corporate debt securities	2 or less	$ 105,085	$ 2	$ (167)	$ 104,920
Certificates of deposit	2 or less	14,600			14,600
Commercial paper	1 or less	2,895	1		2,896
Total		$ 122,580	$ 3	$ (167)	$ 122,416

4. Fair Value Measurements

We have certain financial assets and liabilities recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements.

Accounting standards define fair value as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants as of the measurement date. Market participants are buyers and sellers in the principal market that are (i) independent, (ii) knowledgeable, (iii) able to transact, and (iv) willing to transact. The accounting standards provide an established hierarchy for inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the most observable inputs be used when available. Observable inputs are inputs that market participants would use in

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valuing the asset or liability and are developed based on market data obtained from independent sources. Unobservable inputs are inputs that reflect our assumptions about the factors that market participants would use in valuing the asset or liability. The accounting standard prioritize the inputs used in measuring the fair value into the following hierarchy:

Level 1 includes financial instruments for which quoted market prices for identical instruments are available in active markets.

Level 2 includes financial instruments for which there are inputs other than quoted prices included within Level 1 that are observable for the instrument such as quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets with insufficient volume or infrequent transactions (less active markets) or model-driven valuations in which significant inputs are observable or can be derived principally from, or corroborated by, observable market data.

Level 3 includes financial instruments for which fair value is derived from valuation techniques in which one or more significant inputs are unobservable, including management s own assumptions.

The following table presents our fair value hierarchy for assets and liabilities measured at fair value on a recurring basis at March 31, 2015 and December 31, 2014 (in thousands):

	Fair value as of March 31, 2015			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$ 18,991	$ 18,991	$	$
Corporate debt securities	106,734		106,734	
Certificates of deposit	17,960		17,960	
Commercial paper	4,497		4,497	
	$ 148,182	$ 18,991	$ 129,191	$

	Fair value as of December 31, 2014			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$ 37,072	$ 37,072	$	$
Corporate debt securities	104,920		104,920	
Certificates of deposit	14,600		14,600	
Commercial paper	2,896		2,896	
	$ 159,488	$ 37,072	$ 122,416	$
Liabilities:				

Convertible note payable	$ 23,397	$	$	$ 23,397

We obtain pricing information from quoted market prices or quotes from brokers/dealers. We generally determine the fair value of our investment securities using standard observable inputs, including reported trades, broker/dealer quotes, bids and/or offers. Refer to Note 3 for information regarding our investments.

The following table presents a reconciliation of the liability measured at fair value using significant unobservable inputs (Level 3) from December 31, 2014 to March 31, 2015 (in thousands):

	Fair Value Measurements Using Significant Unobservable Inputs (Level 3)
Balance at December 31, 2014	$ 23,397
Change in estimated fair value of convertible note payable	1,811
Convertible note converted to shares of common stock	(25,208)
Balance at March 31, 2015	$

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We used an income approach in the form of a convertible bond valuation model to value the convertible note payable. The convertible bond model considered the debt and option characteristics of the note. The key inputs to the model as of December 31, 2014 were volatility of 110%, risk-free rate of 0.10%, and credit spread of 9.7%. The volatility inputs were based on historical and implied volatility of peer companies. Peer companies were materially consistent with those used to determine volatility for stock-based compensation. Beginning in 2014, our historical volatility was included with the peer companies for purposes of estimating volatility. As of December 31, 2014, the volatility input included 60% weighting of our historical volatility and 40% weighting of historical and implied volatility of peer companies. The risk-free rate inputs were based on the yield of US Treasury Strips as of each date. The credit spread inputs were based on an analysis of our creditworthiness and market rates for comparable straight debt instruments. On January 29, 2015, the principal balance of the convertible note payable was converted into 1,356,738 shares of common stock, at a conversion price of $4.00 per share. A final valuation upon conversion at January 29, 2015 was performed, considering only the option characteristics of the note as its conversion was certain. Key inputs of volatility, risk-free rate and credit spread were considered, however, the final valuation was substantially driven by the number of shares of common stock issued upon conversion (1,356,738) and our stock price on the date of conversion ($18.58). Upon issuance of the common stock, the fair value of the convertible note was classified into stockholders equity.

We recorded a loss from the change in valuation of convertible note payable of $1.8 million and $2.1 million on the condensed statements of operations and comprehensive loss for the three months ended March 31, 2015 and 2014, respectively.

5. Convertible Note Payable

In October 2012, in conjunction with our initial public offering the amended and restated 2010 GSK Note was rolled over into a new promissory note, and the Post-IPO GSK Note was established in the principal amount of $5.4 million, with a maturity date of October 9, 2015. On January 29, 2015, the principal amount outstanding under the Post-IPO GSK Note of $5.4 million was converted into 1,356,738 shares of our common stock at a conversion price of $4.00 per share. At March 31, 2015 and December 31, 2014, the fair value of the Post-IPO GSK Note was zero and $23.4 million, respectively, and is classified as Convertible note payable, at fair value on the condensed balance sheets.

6. Stockholders Equity

Shares Reserved for Future Issuance

The following shares of common stock are reserved for future issuance:

	March 31, 2015
Common stock options outstanding	6,605,989
Common stock options available for future grant	1,901,972
Employee Stock Purchase Plan	1,291,834
Total shares reserved for future issuance	9,799,795

The following table summarizes our stock option activity under all equity incentive plans for the three months ended March 31, 2015 (shares in thousands):

	Number of options	Weighted average exercise price
Options outstanding at December 31, 2014	6,643	$ 6.95
Granted	413	$ 16.39
Exercised	(423)	$ 1.64
Canceled/forfeited/expired	(27)	$ 11.36
Options outstanding at March 31, 2015	6,606	$ 7.86

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Stock-Based Compensation

The following table summarizes the weighted average assumptions used to estimate the fair value of stock options and performance stock awards granted to employees under our 2012 Equity Incentive Plan and the shares purchasable under our 2012 Employee Stock Purchase Plan during the periods presented:

	Three months ended March 31,	
	2015	2014
Stock options		
Risk-free interest rate	1.7%	1.9%
Volatility	75.1%	74.0%
Dividend yield	0%	0%
Expected term (years)	6.1	6.1
Performance stock options		
Risk-free interest rate	1.8%	2.2%
Volatility	76.4%	70.1%
Dividend yield	0%	0%
Expected term (years)	6.0	6.4
Employee stock purchase plan shares		
Risk-free interest rate	0.1%	0.1%
Volatility	72.9%	65.2%
Dividend yield	0%	0%
Expected term (years)	0.5	0.5

The following table summarizes the allocation of our stock-based compensation expense for all stock awards during the periods presented (in thousands):

	Three months ended March 31,	
	2015	2014
Research and development	$ 1,956	$ 804
General and administrative	1,147	596
Total	$ 3,103	$ 1,400

7. Strategic Alliances and Collaborations

The following table summarizes our total revenues from our strategic alliances and collaborations during the periods presented (in thousands):

	Three months ended March 31,	
	2015	2014

Sanofi	$ 18	$ 925
AstraZeneca	3,582	465
GSK		144
Biogen	600	87
Other		10
Total	$ 4,200	$ 1,631

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Sanofi

In July 2012, we amended and restated our collaboration and license agreement with Sanofi to expand the potential therapeutic applications of the *micro*RNA alliance targets to be developed under such agreement. We determined that the elements within the strategic alliance agreement with Sanofi should be treated as a single unit of accounting because the delivered elements did not have stand-alone value to Sanofi. The following elements were delivered as part of the strategic alliance with Sanofi: (1) a license for up to four *micro*RNA targets; and (2) a research license under our technology alliance.

In June 2013, the original research term expired, upon which we and Sanofi entered into an option agreement pursuant to which Sanofi was granted an exclusive right to negotiate the co-development and commercialization of certain of our unencumbered *micro*RNA programs and we were granted the exclusive right to negotiate with Sanofi for co-development and commercialization of certain miR-21 anti-miRs in oncology and Alport Syndrome. In July 2013, we received an upfront payment of $2.5 million, of which $1.25 million is creditable against future amounts payable by Sanofi to us under any future co-development and commercialization agreement we enter pursuant to the option agreement. Revenue associated with the creditable portion of this option payment remained deferred as of March 31, 2015, and will remain deferred until its application to a creditable transaction. The non-creditable portion of this payment, $1.25 million, was recognized as revenue over the option period from the effective date of the option agreement in June 2013 through the expiration of the option period in January 2014.

In conjunction with the option agreement, we agreed to continue specified research on the miR-21 programs during the option period. We re-evaluated our remaining estimated period of performance from the original research term through the term of the option agreement and amortized the remaining deferred revenue of $10.1 million associated with the initial $25.0 million upfront payment from June 2013 through the expiration of the option period in January 2014.

In February 2014, we and Sanofi entered into a second amended and restated collaboration and license agreement (the 2014 Sanofi Amendment) to renew our strategic alliance to discover, develop and commercialize *micro*RNA therapeutics to focus on specific orphan disease and oncology targets. Under the terms of our renewed alliance, Sanofi will have opt-in rights to our preclinical fibrosis program targeting miR-21 for the treatment of Alport Syndrome, our preclinical program targeting miR-21 for oncology indications, and our preclinical program targeting miR-221/222 for hepatocellular carcinoma (HCC). We are responsible for developing each of these programs to proof-of-concept, at which time Sanofi has an exclusive option on each program. If Sanofi chooses to exercise its option on any of these programs, Sanofi will reimburse us for a significant portion of our preclinical and clinical development costs and will also pay us an option exercise fee for any such program, provided that $1.25 million of the $2.5 million upfront option fee paid to us by Sanofi in connection with the June 2013 option agreement will be creditable against such option exercise fee. In addition, we will be eligible to receive clinical and regulatory milestone payments and potentially commercial milestone payments for these programs. We also continue to be eligible to receive royalties on *micro*RNA therapeutic products commercialized by Sanofi and will have the right to co-promote these products.

In connection with the 2014 Sanofi Amendment, we entered into a Common Stock Purchase Agreement (the Purchase Agreement), pursuant to which we sold 1,303,780 shares of our common stock to Aventisub LLC (formerly Aventis Holdings, Inc.) (Aventis), an entity affiliated with Sanofi, in a private placement at a price per share of $7.67 for an aggregate purchase price of $10.0 million. Under the terms of the Purchase Agreement, Aventis was not permitted to sell, transfer, make any short sale of, or grant any option for the sale of any common stock for the 12-month period following its effective date. The Purchase Agreement and the 2014 Sanofi Amendment were negotiated concurrently and were therefore evaluated as a single agreement. Based upon restricted stock studies of similar duration and a Black-Scholes valuation to measure the discount for lack of marketability, approximately $0.4 million of the proceeds

from the Purchase Agreement was attributed to the 2014 Sanofi Amendment, and represents consideration for the value of the program targeting miR-221/222 for HCC. As this element does not have stand-alone value, we are recognizing the $0.4 million into revenue ratably over the estimated period of performance of the miR-221/222 program. As of March 31, 2015, deferred revenue associated with the Purchase Agreement and the 2014 Sanofi Amendment was $0.3 million, which we are expecting to recognize over the remaining estimated period of performance of approximately five years.

We are eligible to receive milestone payments of up to $101.8 million for proof-of-concept option exercise fees (net of $1.25 million creditable, as noted above), $15.0 million for clinical milestones and up to $300.0 million for regulatory and commercial milestones. In addition, we are entitled to receive royalties based on a percentage of net sales of any products from the miR-21 and miR-221/222 programs which, in the case of sales in the United States, will be in the middle of the 10 to 20% range, and, in the case of sales outside of the United States, will range from the low end to the middle of the 10 to 20% range, depending upon the volume of sales. If we exercise our option to co-promote a product, we will continue to be eligible to receive royalties on net sales of each product in the United States at the same rate, unless we elect to share a portion of Sanofi s profits from sales of such product in the United States in lieu of royalties.

We have evaluated the contingent event-based payments under the 2014 Sanofi Amendment and determined that the milestone payments meet the definition of substantive milestones. Accordingly, revenue for these achievements will be recognized in their

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entirety in the period when the milestone is achieved and collectability is reasonably assured. Other contingent event-based payments under the 2014 Sanofi Amendment for which payment is contingent upon the results of Sanofi s performance will not be accounted for using the milestone method. Such payments will be recognized as revenue over the remaining estimated period of performance, if any, and when collectability is reasonably assured.

AstraZeneca

In August 2012, we entered into a collaboration and license agreement with AstraZeneca. Under the terms of the agreement, we have agreed to collaborate with AstraZeneca to identify, research and develop compounds targeting three *micro*RNA alliance targets primarily in the fields of cardiovascular diseases, metabolic diseases and oncology. Pursuant to the agreement, we granted AstraZeneca an exclusive, worldwide license to thereafter develop, manufacture and commercialize lead compounds designated by AstraZeneca in the course of the collaboration activities against the alliance targets for all human therapeutic uses. Under the terms of the agreement we are required to use commercially reasonable efforts to perform all research, development and manufacturing activities described in the research plan, at our cost, until the acceptance of an investigational new drug application (IND) or the end of the research term, which extends until the fourth anniversary of the date of the agreement, and may be extended only by mutual written agreement of us and AstraZeneca. Following the earlier to occur of the acceptance of an IND in a major market or the end of the research term, AstraZeneca will assume all costs, responsibilities and obligations for further development, manufacture and commercialization of alliance product candidates.

Under the terms of the agreement, we received an upfront payment of $3.0 million in October 2012. We determined the elements within the agreement should be treated as a single unit of accounting because the delivered element, the license, does not have stand-alone value. As a result, we are recognizing revenue related to the upfront payment on a straight-line basis over our estimated period of performance, which is four years based on the expected term of the research and development plan.

In March 2015, we earned a $2.5 million preclinical milestone payment upon AstraZeneca s selection of RG-125, a GalNAc-conjugated anti-miR targeting *micro*RNA-103/107, as a lead compound under the agreement. If all three targets are successfully developed and commercialized through pre-agreed sales targets, we could receive additional milestone payments of up to $495.5 million, including preclinical milestones of up to $2.5 million upon selection of a lead compound, up to $123.0 million for clinical milestones, and up to $370.0 million for commercialization milestones. In addition, we are entitled to receive royalties based on a percentage of net sales which will range from the mid-single digits to the low end of the 10 to 20% range, depending upon the product and the volume of sales, which royalties may be reduced in certain, limited circumstances.

In January 2015, we entered into a letter agreement with AstraZeneca to amend the collaboration and license agreement. Under the terms of the letter agreement, we have agreed to perform additional miR-103/107 program research and development activities related to RG-125. AstraZeneca has agreed to fund 50% of the costs for additional activities, as outlined in the letter agreement. In accordance with the collaboration and license agreement, AstraZeneca will fund 100% of the costs for product manufacturing activities outlined in the letter agreement necessary to support a Phase I clinical study. We have recognized revenue of $0.6 million for the three months ended March 31, 2015 for services outlined in the letter agreement. As of March 31, 2015, deferred revenue associated with the letter agreement was $2.7 million, which we are expecting to recognize in the period services are provided.

We have evaluated the contingent event-based payments under our collaboration and license agreement with AstraZeneca and determined that the preclinical payments meet the definition of substantive milestones. Accordingly, revenue for these achievements will be recognized in its entirety in the period when the milestone is achieved and collectability is reasonably assured. Other contingent event-based payments under the agreement for which payment is

contingent upon the results of AstraZeneca s performance will not be accounted for using the milestone method. Such payments will be recognized as revenue over the remaining estimated period of performance, if any, and when collectability is reasonably assured.

Concurrently with the collaboration and license agreement, we entered into a Common Stock Purchase Agreement (CSPA) with AstraZeneca, pursuant to which we agreed to sell to AstraZeneca an aggregate of $25.0 million of our common stock in a private placement concurrently with our initial public offering, at a price per share equal to the initial public offering price. In October 2012, in accordance with the CSPA, we sold AstraZeneca 6,250,000 shares of our common stock at a price per share of $4.00. Further, the CSPA stipulated that AstraZeneca could not sell, transfer, make any short sale of, or grant any option for the sale of any common stock for a 365-day period following the effective date of our initial public offering. Accounting standards for multiple element arrangements contains a presumption that separate contracts negotiated and/or entered into at or near the same time with the same entity were negotiated as a package and should be evaluated as a single agreement. We valued the discount applied to the shares of common stock due to the one-year restriction. Based upon restricted stock studies of similar duration and a Black-Scholes valuation to measure a discount for lack of marketability, $4.3 million was attributed to the collaboration and license agreement. We continue to recognize the $4.3 million into revenue ratably over the estimated period of performance of the collaboration. As of March 31, 2015, deferred revenue associated with the collaboration and license agreement and CSPA was $2.5 million, which we are expecting to recognize over the remaining contractual term and corresponding estimated period of performance of approximately 1.5 years.

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GSK

In April 2008, we entered into a strategic alliance with GSK to discover, develop and commercialize novel *micro*RNA-targeted therapeutics to treat inflammatory diseases (the immuno-inflammatory alliance). In February 2010, we and GSK expanded the strategic alliance to include hepatitis C virus infection (HCV) to discover, develop and commercialize *micro*RNA therapeutics targeting miR-122 for the treatment of HCV (the HCV alliance). In June 2012, we amended our immuno-inflammatory alliance to extend the target selection period for the fourth collaboration target. We determined that the elements within the immuno-inflammatory alliance should be treated as a single unit of accounting because the delivered elements, the opt-in licenses for *micro*RNA product candidates, did not have stand-alone value to GSK. As a result of the extension of the target selection period, we extended the amortization period for the remaining deferred revenue to approximately eight years, which represented our new estimated period of performance.

In June 2013, the HCV alliance was amended to state that RG-101, and other formulations thereof, will be developed by us independently of our alliance for the treatment of HCV. This amendment removed any further milestone or royalty obligations owed by GSK to us as it relates to RG-101. Concurrently with the amendment, we recorded the remaining $1.1 million in deferred revenue associated with the upfront payment from the HCV alliance, as our estimated period of performance was complete.

In October 2014, we received written notice from GSK of its election to terminate the product development and commercialization agreement. Concurrently with the notice of termination, we recorded the remaining $3.1 million in deferred revenue associated with the upfront payment, as our estimated period of performance was complete. The effective date of the termination was January 15, 2015 in accordance with the terms of the agreement.

Biogen

In August 2012, we entered into a collaboration and license agreement with Biogen pursuant to which we and Biogen agreed to collaborate on *micro*RNA biomarkers for multiple sclerosis (MS). Under the terms of the agreement, in August 2012 we received an upfront payment of $0.8 million. We were also eligible to receive research milestone payments up to an aggregate of $1.3 million. We considered the elements within the collaboration and license agreement as a single unit of accounting because the delivered element, the license, did not have stand-alone value. As a result, we recognized revenue relating to the upfront payment of $0.8 million on a straight-line basis over our estimated period of performance, which was approximately two years based on the expected term of the research and development plan.

In June 2013, we amended the collaboration and license agreement to provide for revised terms with respect to the initial phase of the research plan and related milestone payment provisions. The amendment did not modify the maximum dollar amount we were originally eligible to receive in connection with the agreement, or our estimated period of performance. In October 2013 and November 2013, we received research milestone payments totaling $0.3 million under the August 2012 collaboration and license agreement.

In August 2014, we entered into a new collaboration and license agreement with Biogen to collaborate on *micro*RNA biomarkers for MS and simultaneously executed an agreement terminating the August 2012 collaboration and license agreement. As a result of the termination agreement, we recognized $0.1 million in deferred revenue associated with the upfront payment, as our estimated period of performance was complete. Pursuant to the terms of the August 2014 collaboration and license agreement, we received an upfront payment of $2.0 million in August 2014. We are also eligible to receive research-based milestone payments up to an aggregate of $0.7 million. We determined that the elements within the August 2014 collaboration and license agreement should be treated as a single unit of accounting

because the delivered element, the license, does not have stand-alone value to Biogen. As a result, we are recognizing revenue relating to the upfront payment of $2.0 million on a straight-line basis over the estimated period of performance, which is approximately one year based on the expected term of the research and development plan. In January 2015, we earned a $0.1 million research milestone payment under the August 2014 collaboration and license agreement.

We have evaluated the contingent event-based payments under our collaboration and license agreement with Biogen and determined that the research payments meet the definition of substantive milestones. Accordingly, revenue for these achievements will be recognized in their entirety in the period when the milestone is achieved and collectability is reasonably assured. As of March 31, 2015, deferred revenue associated with the collaboration and license agreement was $0.7 million which we are expecting to recognize over the remaining contractual term and corresponding estimated period of performance of approximately four months.

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8. Related Party Transactions

We have entered into several agreements with related parties in the ordinary course of business to license intellectual property and to procure administrative and research and development support services.

In August 2013, we entered into an amendment to the Amended and Restated License and Collaboration Agreement with Isis and Alnylam dated January 1, 2009, as amended in June 2010 and October 2011 (as amended, the Amendment). Under the terms of the Amendment, the parties agreed to our use of certain Alnylam-controlled intellectual property concerning the use and manufacture of GalNAc conjugates (GalNAc Process Technology) on a non-exclusive basis. We will generally not be permitted to sublicense or otherwise transfer the GalNAc Process Technology and other Alnylam licensed intellectual property rights relating to GalNAc conjugate technology without the prior written consent of Alnylam, subject to certain limited exceptions for sublicenses to third party collaboration partners. There were no financial terms related to this Amendment. We did not purchase GalNAc-related materials from Alnylam in the three months ended March 31, 2015 and 2014.

In February 2015, we entered into a letter agreement with Alnylam pursuant to which we and Alnylam agreed to the financial terms for certain technology acquired by Alnylam within the licensed patent rights under the Amended and Restated License and Collaboration Agreement (the Additional patent Rights). In addition to any royalties payable by us to Alnylam pursuant to the terms of the Amended and Restated Collaboration Agreement, we agreed to pay Alnylam an additional low single-digit royalty on net sales of certain products utilizing the Additional Patent Rights, with the exact royalty percentage payable being dependent on the total amount of net sales during the calendar year. We also agreed to pay Alnylam milestone payments on certain products utilizing the additional patent rights of up to $33.0 million per product upon the achievement of certain regulatory milestone events.

In September 2014, we entered into an agreement with Sanofi-Aventis Deutschland GmbH (Sanofi Deutschland), a contract manufacturing subsidiary of Sanofi, for the manufacture of certain drug substance requirements and other services to support our preclinical and clinical activities associated with the RG-012 program. Pursuant to this agreement, we may engage Sanofi Deutschland from time-to-time to manufacture RG-012 drug product on our behalf.

The following table summarizes the amounts included in our operating expenses as a result of costs incurred from services provided under the Sanofi agreement (in thousands):

	Three months ended March 31,	
	2015	2014
Expenses incurred for services performed or out-of-pocket expenses under the Sanofi agreement	$ 400	$

ITEM 2. MANAGEMENT S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

The interim unaudited condensed financial statements and this Management s Discussion and Analysis of Financial Condition and Results of Operations should be read in conjunction with the financial statements and notes thereto for the year ended December 31, 2014 and the related Management s Discussion and Analysis of Financial Condition and Results of Operations, both of which are contained in our Annual Report on Form 10-K for the year ended December 31, 2014, or Annual Report, filed with the Securities and Exchange Commission on February 19, 2015. Past operating results are not necessarily indicative of results that may occur in future periods.

FORWARD-LOOKING STATEMENTS

This quarterly report on Form 10-Q and the documents incorporated by reference herein may contain forward-looking statements within the meaning of the federal securities laws made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Our actual results could differ materially from those anticipated in these forward-looking statements as a result of various factors, including those set forth below under Part II, Item 1A, Risk Factors in this quarterly report on Form 10-Q. Except as required by law, we assume no obligation to update these forward-looking statements, whether as a result of new information, future events or otherwise. These statements, which represent our current expectations or beliefs concerning various future events, may contain words such as may, will, expect, anticipate, intend, plan, believe, estimate or other words indicating future results though not a forward-looking statements necessarily contain these identifying words. Such statements may include, but are not limited to, statements concerning the following:

- the initiation, cost, timing, progress and results of, and our expected ability to undertake certain activities and accomplish certain goals with respect to our research and development activities, preclinical studies and future clinical trials;

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our ability to obtain and maintain regulatory approval of our future product candidates, and any related restrictions, limitations, and/or warnings in the label of an approved product candidate;

our ability to obtain funding for our operations;

our plans to research, develop and commercialize our future product candidates;

our strategic alliance partners election to pursue development and commercialization;

our ability to attract collaborators with development, regulatory and commercialization expertise;

our ability to obtain and maintain intellectual property protection for our future product candidates;

the size and growth potential of the markets for our future product candidates, and our ability to serve those markets;

our ability to successfully commercialize, and our expectations regarding future therapeutic and commercial potential with respect to, our future product candidates;

the rate and degree of market acceptance of our future product candidates;

our ability to develop sales and marketing capabilities, whether alone or with potential future collaborators;

regulatory developments in the United States and foreign countries;

the performance of our third-party suppliers and manufacturers;

the success of competing therapies that are or become available;

the loss of key scientific or management personnel;

our ability to successfully secure and deploy capital;

our ability to satisfy our debt obligations;

our expectations regarding the time during which we will be an emerging growth company under the Jumpstart our Business Startups Act of 2012, or the JOBS Act;

our use of the proceeds from our prior or subsequent public offerings;

the accuracy of our estimates regarding expenses, future revenues, capital requirements and need for additional financing; and

the additional risks and other factors described under the caption Risk Factors under Part II, Item 1A of this quarterly report on Form 10-Q.

OVERVIEW

We are a biopharmaceutical company focused on discovering and developing first-in-class drugs that target *micro*RNAs to treat a broad range of diseases. We were formed in 2007 when Alnylam Pharmaceuticals, Inc. and Isis Pharmaceuticals, Inc. contributed significant intellectual property, know-how and financial and human capital to pursue the development of drugs targeting *micro*RNAs pursuant to a license and collaboration agreement. We have established strategic alliances with AstraZeneca AB and Sanofi to discover, develop and commercialize *micro*RNA therapeutics. Under these strategic alliances, we are eligible to receive approximately $900.0 million in aggregate milestone payments upon successful commercialization of *micro*RNA therapeutics for the programs contemplated by our agreements. These payments include up to $107.8 million upon achievement of preclinical and investigational new drug, or IND, milestones, up to $138.0 million upon achievement of clinical development milestones, up to $180.0 million upon achievement of regulatory milestones and up to $490.0 million upon achievement of commercialization milestones.

*micro*RNAs are naturally occurring ribonucleic acid, or RNA, molecules that play a critical role in regulating key biological pathways. Scientific research has shown that the improper balance, or dysregulation, of *micro*RNAs is directly linked to many diseases. To date, more than 500 *micro*RNAs have been identified in humans, each of which is believed to interact with a specific set of genes that control key aspects of cell biology. Since most diseases are multi-factorial and involve multiple targets in a pathway, the ability to modulate gene networks by targeting a single *micro*RNA provides a new therapeutic approach for treating complex diseases.

RNA plays an essential role in the process used by cells to encode and translate genetic information from DNA to proteins. RNA is comprised of subunits called nucleotides and is synthesized from a DNA template by a process known as transcription. Transcription generates different types of RNA, including messenger RNAs that carry the information for proteins in the sequence of their nucleotides. In contrast, *micro*RNAs are small RNAs that do not code for proteins but rather are responsible for regulating gene expression by affecting the translation of target messenger RNAs. By interacting with many messenger RNAs, a single *micro*RNA can regulate several genes that are instrumental for the normal function of a biological pathway.

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We believe that *micro*RNA therapeutics have the potential to become a new and major class of drugs with broad therapeutic application for the following reasons:

*micro*RNAs until recently, have not been a focus of pharmaceutical research;

*micro*RNAs play a critical role in regulating biological pathways by controlling the translation of many target genes;

*micro*RNA therapeutics target entire disease pathways which may result in more effective treatment of complex multi-factorial diseases; and

*micro*RNA therapeutics may be synergistic with other therapies because of their different mechanism of action.

We believe we have assembled the leading position in the *micro*RNA field, including expertise in *micro*RNA biology and oligonucleotide chemistry, a broad intellectual property estate, relationships with key opinion leaders and disciplined drug discovery and development processes. We refer to these assets as our *micro*RNA product platform. We are using our *micro*RNA product platform to develop chemically modified, single-stranded oligonucleotides that we call anti-miRs to modulate *micro*RNAs and return diseased cells to their healthy state. We believe *micro*RNAs may be transformative in the field of drug discovery and that anti-miRs may become a new and major class of drugs with broad therapeutic application much like small molecules, biologics and monoclonal antibodies. In addition to our *micro*RNA product platform, we have established Regulus *micro*Markers[SM], a division focused on identifying *micro*RNAs as biomarkers of human disease to support our therapeutic pipeline, collaborators and strategic partners. Regulus *micro*Markers[SM] utilizes a clinically-validated, highly reproducible, proprietary technology platform to identify *micro*RNAs as potential biomarkers for disease and we control key intellectual property and know-how related to the division. We believe that *micro*RNA biomarkers may be used to select optimal patient segments in clinical trials and to monitor disease progression or relapse. We believe these *micro*RNA biomarkers can be applied toward drugs that we develop and drugs developed by other companies with which we partner or collaborate, including small molecules and monoclonal antibodies. We have formed a research collaboration with Biogen focused on the discovery of *micro*RNAs as biomarkers for multiple sclerosis and have also entered into an arrangement with another leading, commercial-stage pharmaceutical company to explore *micro*RNAs as biomarkers for specific patient populations. We also maintain several academic research collaborations focused on the identification of *micro*RNAs as biomarkers in multiple disease areas.

Clinical Map Initiative Goals

To advance our *micro*RNA therapeutics pipeline and biomarkers platform over the next several years, we have outlined specific goals under our Clinical Map Initiative strategy. Under this initiative, we are developing RG-101, our wholly-owned GalNAc-conjugated anti-miR targeting *micro*RNA-122 for the treatment of HCV and RG-012, an anti-miR targeting *micro*RNA-21 for the treatment of Alport syndrome, a life-threatening kidney disease driven by genetic mutations with no approved therapy. We are also advancing several programs toward clinical development in oncology, fibrosis and metabolic diseases, both independently and with our strategic alliance partners AstraZeneca and Sanofi. Under our strategic alliance with AstraZeneca, they recently selected RG-125, a GalNAc-conjugated anti-miR targeting microRNA-103/107 for the treatment of nonalcoholic steatohepatitis, or NASH, in patients with type 2 diabetes/pre-diabetes. This represents our third candidate to be nominated for clinical development and

achieves a key goal under our Clinical Map Initiative for 2015.

Our additional goals under the Clinical Map Initiative for 2015 are focused on advancing our clinical-stage programs, RG-101, RG-012, and RG-125, and expanding our biomarkers efforts to support our growing therapeutics portfolio. Specifically, we intend to initiate Phase II studies investigating RG-101 in combination with oral direct-acting antiviral agents, and further as a single agent with interim data expected by the end of 2015. In the RG-012 program, we intend to enroll up to 120 Alport syndrome patients in our global ATHENA natural history of disease study, which is designed to characterize the natural decline of renal function (as measured by established renal markers) in Alport syndrome patients over time. We believe the data from ATHENA will provide the clinical basis for the design of a Phase II proof-of-concept study to monitor the therapeutic effect of RG-012 on the decline in renal function in patients with Alport syndrome. In addition, we plan to initiate a Phase I study in the first half of 2015 to evaluate the safety and tolerability of RG-012 in healthy volunteers and to initiate a Phase II proof-of-concept study thereafter. In the RG-125 program, we and AstraZeneca are working toward the filing of an IND application with the goal to initiate a Phase I study in humans by the end of 2015. We are also continuing to advance several programs toward clinical development in oncology, fibrosis and other disease areas of interest to us and our strategic alliance partners.

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FINANCIAL OPERATIONS OVERVIEW

Revenues

Our revenues generally consist of upfront payments for licenses or options to obtain licenses in the future, milestone payments and payments for other research services under strategic alliance and collaboration agreements.

In the future, we may generate revenue from a combination of license fees and other upfront payments, research and development payments, milestone payments, product sales and royalties in connection with strategic alliances. We expect that any revenue we generate will fluctuate from quarter-to-quarter as a result of the timing of our achievement of preclinical, clinical, regulatory and commercialization milestones, if at all, the timing and amount of payments relating to such milestones and the extent to which any of our products are approved and successfully commercialized by us or our strategic alliance partners. If our strategic alliance partners do not elect or otherwise agree to fund our development costs pursuant to our strategic alliance agreements, or we or our strategic alliance partners fail to develop product candidates in a timely manner or obtain regulatory approval for them, our ability to generate future revenues, and our results of operations and financial position would be adversely affected.

Research and development expenses

Research and development expenses consist of costs associated with our research activities, including our drug discovery efforts, the development of our therapeutic programs, and our Regulus *micro*Markers[SM] division. Our research and development expenses include:

- employee-related expenses, including salaries, benefits, travel and stock-based compensation expense;
- external research and development expenses incurred under arrangements with third parties, such as contract research organizations, or CROs, contract manufacturing organizations, or CMOs, other clinical trial related vendors, consultants and our scientific advisors;
- license fees; and
- facilities, depreciation and other allocated expenses, which include direct and allocated expenses for rent and maintenance of facilities, depreciation of leasehold improvements and equipment, and laboratory and other supplies.

We expense research and development costs as incurred. We account for nonrefundable advance payments for goods and services that will be used in future research and development activities as expenses when the service has been performed or when the goods have been received.

To date, we have conducted research on many different *micro*RNAs with the goal of understanding how they function and identifying those that might be targets for therapeutic modulation. At any given time we are working on multiple targets, primarily within our therapeutic areas of focus. Our organization is structured to allow the rapid deployment and shifting of resources to focus on the best targets based on our ongoing research. As a result, in the early phase of our development programs, our research and development costs are not tied to any specific target. However, we are

currently spending the vast majority of our research and development resources on our lead development programs.

Since our conversion to a corporation in January 2009, we have grown from 15 research and development personnel to 69 and have spent a total of approximately $151.3 million in research and development expenses through March 31, 2015.

We expect our research and development expenses to increase for the foreseeable future as we continue to advance our pre-clinical research programs toward the clinic, including initiating additional pre-clinical studies, continuing to conduct our ongoing clinical studies, and initiating additional clinical studies and other IND-enabling activities. The process of conducting preclinical studies and clinical trials necessary to obtain regulatory approval is costly and time consuming. We, or our strategic alliance partners, may never succeed in achieving marketing approval for any of our product candidates. The probability of success for each product candidate may be affected by numerous factors, including preclinical data, clinical data, competition, manufacturing capability and commercial viability. Under our strategic alliance with Sanofi, we are responsible for the development of product candidates through proof-of-concept, after which time Sanofi would be responsible for the costs of clinical development and commercialization and all related costs, in the event it exercises its option to such program. Under our strategic alliance agreement with AstraZeneca, we are responsible for certain research and development activities with respect to each alliance target under a mutually agreed upon research and development plan until the earlier to occur of acceptance of an IND application (or its foreign equivalent) in a major market or the end of the research term under the agreement. We also have several independent programs for which we are responsible for all of the research and development costs, unless and until we partner any of these programs in the future.

Most of our product development programs are at an early stage, and successful development of future product candidates from these programs is highly uncertain and may not result in approved products. Completion dates and completion costs can vary

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significantly for each future product candidate and are difficult to predict. We anticipate we will make determinations as to which programs to pursue and how much funding to direct to each program on an ongoing basis in response to our ability to maintain or enter into new strategic alliances with respect to each program or potential product candidate, the scientific and clinical success of each future product candidate, as well as ongoing assessments as to each future product candidate s commercial potential. We will need to raise additional capital and may seek additional strategic alliances in the future in order to advance our various programs.

General and administrative expenses

General and administrative expenses consist primarily of salaries and related benefits, including stock-based compensation, related to our executive, finance, legal, business development and support functions. Other general and administrative expenses include allocated facility-related costs not otherwise included in research and development expenses, travel expenses and professional fees for auditing, tax and legal services. We expect that general and administrative expenses will increase in the future as we expand our operating activities and incur additional costs associated with being a publicly-traded company. These increases will likely include legal fees, accounting fees, directors and officers liability insurance premiums and fees associated with investor relations.

Other income (expense), net

Other income (expense) consists primarily of interest income and expense, and on occasion income or expense of a non-recurring nature, including changes in the valuation of convertible note payable from period to period. We earn interest income from interest-bearing accounts and money market funds for cash and cash equivalents and marketable securities, such as interest-bearing bonds, for our short-term investments. Interest expense has historically represented interest payable under convertible note payable and equipment and tenant improvement financing arrangements.

CRITICAL ACCOUNTING POLICIES AND ESTIMATES

The preparation of our financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities, and the revenues and expenses incurred during the reported periods. We believe that the estimates, assumptions and judgments involved in the accounting policies described in Management s Discussion and Analysis of Financial Condition and Results of Operations in Item 7 and under Note 1 to our financial statements contained in our Annual Report have the greatest potential impact on our financial statements, so we consider them to be our critical accounting policies and estimates. There were no material changes to our critical accounting policies and estimates as disclosed in our Annual Report.

RESULTS OF OPERATIONS

Comparison of the three months ended March 31, 2015 and 2014

The following table summarizes our results of operations for the three months ended March 31, 2015 and 2014, together with the changes in those items (in thousands):

	Three months ended March 31,		
	2015	2014	Increase/(Decrease)
Revenue under strategic alliances	$ 4,200	$ 1,631	$ 2,569
Research and development expenses	13,427	9,604	3,823

General and administrative expenses	3,644	2,732	912
Loss from changes in valuation of convertible note payable	(1,811)	(2,124)	(313)

Revenue under strategic alliances

Our revenues are generated from ongoing strategic alliance and collaborations, and generally consist of upfront payments for licenses or options to obtain licenses in the future, milestone payments and payments for other research services. The following table summarizes our total revenues for the periods indicated (in thousands):

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	Three months ended March 31,	
	2015	2014
AstraZeneca	$ 3,582	$ 465
Sanofi	18	925
Biogen	600	87
GSK		144
Other		10
Total revenue under strategic alliances	$ 4,200	$ 1,631

Revenue under strategic alliances was $4.2 million for the three months ended March 31, 2015, compared to $1.6 million for the three months ended March 31, 2014.

Revenue under the AstraZeneca collaboration and license agreement increased to $3.6 million for the three months ended March 31, 2015, compared to $0.5 million for the three months ended March 31, 2014. This change was primarily a result of earning a $2.5 million preclinical milestone payment for the clinical candidate selection of RG-125, a GalNAc-conjugated anti-miR targeting microRNA-103/107 for the treatment of NASH in patients with type 2 diabetes/pre-diabetes in March 2015. In January 2015, we and AstraZeneca entered into a letter agreement to perform additional research and development activities and to provide manufacturing support for RG-125. Revenue of $0.6 million was recognized under the side letter for the three months ended March 31, 2015.

In February 2014, we and Sanofi entered into a second amended and restated collaboration and license agreement to renew our strategic alliance to discover, develop and commercialize *micro*RNA therapeutics to focus on specific orphan disease and oncology targets. Revenue recognized from our strategic alliance with Sanofi decreased to less than $0.1 million for the three months ended March 31, 2015, compared to $0.9 million for the three months ended March 31, 2014. Revenue recognized in these periods reflected the amortization of payments received from Sanofi over our estimated period of performance.

In August 2014, we and Biogen entered into a new collaboration and license agreement to collaborate on *micro*RNA biomarkers for MS. Revenue recognized from our agreement with Biogen increased to $0.6 million for the three months ended March 31, 2015 compared to $0.1 million for the three months ended March 31, 2014. This change was primarily a result of amortization of the $2.0 million upfront payment received in August 2014 which is being recognized over the estimated one-year period of performance. In January 2015, we earned a $0.1 million research milestone under the collaboration and license agreement.

Revenue recognized from our product development and commercialization agreement with GSK decreased to zero for the three months ended March 31, 2015, compared to $0.1 million for the three months ended March 31, 2014. The decrease resulted from the termination of the product development and commercialization agreement in January 2015.

As of March 31, 2015, we had $8.1 million of deferred revenue, which consisted of payments received through our strategic alliances that have not yet been recognized in accordance with our revenue recognition policies and remaining estimated period of performance.

Research and development expenses

Research and development expenses were $13.4 million for the three months ended March 31, 2015 compared to $9.6 million for the three months ended March 31, 2014. This change was primarily driven by an increase in costs for

RG-101 and RG-125 of $0.4 million and $1.2 million, respectively, offset by a decrease in costs of $0.4 million for RG-012. Additionally, there was an increase in lab supplies and salaries and related benefits, including stock-based compensation, of $0.8 million and $1.5 million, respectively. Employees engaged in research and development activities increased to 69 employees as of March 31, 2015, compared to 59 as of March 31, 2014. We expect our research and development expenses to continue to increase for the remainder of 2015 compared to 2014 as we continue clinical studies and initiate additional pre-clinical and clinical programs.

General and administrative expenses

General and administrative expenses were $3.6 million for the three months ended March 31, 2015 compared to $2.7 million for the three months ended March 31, 2014. This increase was primarily driven by an increase in salaries and related benefits, including stock based compensation, of $0.8 million.

Loss from valuation of convertible note payable

We recorded a loss from changes in value of convertible notes payable of $1.8 million and $2.1 million in the statements of operations and comprehensive loss for the three months ended March 31, 2015 and 2014, respectively. Changes in value were driven by changes in our stock price during the respective periods.

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LIQUIDITY AND CAPITAL RESOURCES

Since our inception through March 31, 2015, we have received $69.0 million principally from upfront payments, research funding and preclinical milestones from our strategic alliances and collaborations, $257.1 million from the sale of our equity and convertible debt securities, including $70.0 million in net proceeds from our initial public offering and concurrent private placement of our common stock in October 2012, $45.8