Regulus Therapeutics Inc.
Form 10-Q
August 02, 2017
Table of Contents

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 JUNE 30, 2017
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.)
10614 Science Center Drive 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, 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 x
Non-accelerated filer ¨ (Do not check if a smaller reporting company)	Smaller reporting company ¨
	Emerging growth company x

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. x
Indicate by check mark whether registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ¨ No ý
As of July 28, 2017, the registrant had 103,785,730 shares of Common Stock ($0.001 par value) outstanding.

Table of Contents

REGULUS THERAPEUTICS INC.
TABLE OF CONTENTS

Table of Contents

PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS

Regulus Therapeutics Inc.
CONDENSED BALANCE SHEETS
(in thousands, except share and per share data)

	June 30, 2017 (Unaudited)	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 12,538	$ 14,941
Short-term investments	27,549	61,170
Contract and other receivables	690	1,657
Prepaid materials, net	6,149	5,552
Prepaid expenses and other current assets	3,018	4,154
Total current assets	49,944	87,474
Property and equipment, net	10,753	11,830
Intangibles, net	823	1,015
Other assets	340	342
Total assets	$ 61,860	$ 100,661
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,901	$ 5,840
Accrued liabilities	4,717	5,577
Accrued compensation	1,433	2,318
Current portion of deferred revenue	72	72
Total current liabilities	11,123	13,807
Term loan, less debt issuance costs	19,830	19,802
Deferred revenue, less current portion	1,957	1,993
Deferred rent, less current portion	8,672	8,840
Other long-term liabilities	283	144
Total liabilities	41,865	44,586
Commitments and Contingencies		
Stockholders' equity:		
Common stock, $0.001 par value; 200,000,000 shares authorized, 53,182,330 and 52,924,805 shares issued and outstanding at June 30, 2017 (unaudited) and December 31, 2016, respectively	53	53
Additional paid-in capital	335,612	329,496
Accumulated other comprehensive loss	(88)	(123)
Accumulated deficit	(315,582)	(273,351)
Total stockholders' equity	19,995	56,075
Total liabilities and stockholders' equity	$ 61,860	$ 100,661

See accompanying notes to these condensed financial statements.

Table of Contents

Regulus Therapeutics Inc.
CONDENSED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(In thousands, except share and per share data)

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
	(Unaudited)			
Revenues:				
Revenue under strategic alliances and collaborations	$18	$483	$36	$972
Total revenues	18	483	36	972
Operating expenses:				
Research and development	14,278	18,007	30,030	34,772
General and administrative	7,057	3,664	11,016	8,767
Total operating expenses	21,335	21,671	41,046	43,539
Loss from operations	(21,317)	(21,188)	(41,010)	(42,567)
Other income (expense):				
Interest and other income	184	180	398	372
Interest and other expense	(603)	(90)	(1,149)	(114)
Loss before income taxes	(21,736)	(21,098)	(41,761)	(42,309)
Income tax benefit	128	8	132	13
Net loss	$(21,608)	$(21,090)	$(41,629)	$(42,296)
Other comprehensive loss:				
Unrealized gain on short-term investments, net	10	9	36	50
Comprehensive loss	$(21,598)	$(21,081)	$(41,593)	$(42,246)
Net loss per share, basic and diluted	$(0.41)	$(0.40)	$(0.78)	$(0.80)
Weighted average shares used to compute basic and diluted net loss per share	53,182,330	52,782,643	53,086,887	52,746,657

See accompanying notes to these condensed financial statements.

Table of Contents

Regulus Therapeutics Inc.
CONDENSED STATEMENTS OF CASH FLOWS
(In thousands)

	Six months ended June 30,	
	2017	2016
	(Unaudited)	
Operating activities		
Net loss	$(41,629)	$(42,296)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation and amortization expense	1,357	928
Stock-based compensation	5,247	5,986
Amortization of premium on investments, net	181	359
Other	190	45
Change in operating assets and liabilities:		
Contracts and other receivables	967	9,878
Prepaid materials	(597)	2,834
Prepaid expenses and other assets	1,106	(1,970)
Accounts payable	(907)	787
Accrued liabilities	(814)	(1,063)
Accrued compensation	(885)	(529)
Deferred revenue	(36)	(972)
Deferred rent and other liabilities	(187)	(262)
Net cash used in operating activities	(36,007)	(26,275)
Investing activities		
Purchases of short-term investments	(4,453)	(30,231)
Sales and maturities of short-term investments	37,929	47,522
Purchases of property and equipment	(124)	(266)
Acquisition of intangibles	(16)	(34)
Net cash provided by investing activities	33,336	16,991
Financing activities		
Proceeds from borrowing under term loan, net	—	19,819
Proceeds from issuance of common stock	265	363
Proceeds from exercise of common stock options	3	265
Principal payments on other long-term obligations	—	(83)
Net cash provided by financing activities	268	20,364
Net (decrease) increase in cash and cash equivalents	(2,403)	11,080
Cash and cash equivalents at beginning of period	14,941	15,960
Cash and cash equivalents at end of period	$12,538	$27,040
Supplemental disclosure of cash flow information		
Net changes in restricted cash	$—	$(794)
Interest paid	$(946)	$(54)
Income taxes paid	$(1)	$(1)
Supplemental disclosure of non-cash investing and financing activities		
Allowance for tenant improvements	$—	$6,545
Amounts accrued for property and equipment	$110	$221
Amounts accrued for patent expenditures	$—	$7
Unpaid debt issuance costs	$—	$38

See accompanying notes to these condensed financial statements.

5

Table of Contents

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 audited financial statements and footnotes included in our Annual Report on Form 10-K for the year ended December 31, 2016, from which the balance sheet information herein was derived.

We have incurred losses in each year since our inception. We expect to continue to incur significant expenses and operating losses for the foreseeable future in connection with our research and preclinical and clinical development of our product candidates. In order to continue to fund our research and development activities, we will need to seek additional capital. This may occur through strategic alliance and licensing arrangements and/or future public or private debt or equity financings. Sufficient funding may not be available, or if available, may be on terms that significantly dilute or otherwise adversely affect the rights of existing stockholders. If adequate funds are not available in the future, we may need to delay, reduce the scope of or put on hold one or more or our clinical and/or preclinical programs while we seek strategic alternatives.

In May 2017, we implemented a corporate restructuring to streamline our operations, reduce our operating expenses, extend our cash runway and focus our resources on our most promising programs. In connection with the restructuring, we committed to a reduction in our total workforce by approximately 30% percent, to approximately 65 employees. We completed the workforce reduction in June 2017. We recorded charges of approximately $3.2 million for employee severance and other related termination benefits, including $1.3 million in net adjustments to non-cash stock-based compensation. All payments associated with the corporate restructuring were paid in full as of June 30, 2017.

As of June 30, 2017, we had cash, cash equivalents and short-term investments of $40.1 million. We adopted Financial Accounting Standards Board, or FASB, Accounting Standards Update, or ASU, 2014-15, Presentation of Financial Statements - Going Concern, effective December 31, 2016. We have evaluated and concluded that there are no conditions or events, considered individually or in the aggregate, that raise substantial doubt about our ability to continue as a going concern for a period of one year following the date that these financial statements are issued.

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. An estimated loss contingency is accrued in our financial statements if it is probable that a liability has been incurred and the amount of the loss can be reasonably estimated. 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 of 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 agreement with Sanofi, 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

Table of Contents

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, which approximates effort over our estimated period of performance, which for us is typically 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 acknowledgment 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 using a method consistent with the related units of accounting for the arrangement over the estimated performance period.

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 option pricing 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.

Clinical Trial and Preclinical Study Accruals

We make estimates of our accrued expenses for clinical trial and preclinical study activities as of each balance sheet date in our financial statements based on the facts and circumstances known to us at that time. These accruals are based upon estimates of costs incurred and fees that may be associated with services provided by clinical trial investigational sites, clinical research organizations ("CROs") and for other clinical trial-related activities. 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 for these services, 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 activities 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.

Table of Contents

Prepaid Materials

We capitalize the purchase of certain raw materials and related supplies for use in the manufacturing of drug product in our clinical development programs, as we have determined that these materials have alternative future use. We can use these raw materials and related supplies in multiple clinical drug products, and therefore have future use independent of the development status of any particular drug program until it is utilized in the manufacturing process. We periodically review these capitalized materials for indicators of impairment, including shelf life, continued alternative future use and obsolescence, and write down the asset to its net realizable value in the period it which it is identified. As of June 30, 2017 and December 31, 2016, our prepaid materials balance was $6.1 million, net of a $0.6 million reserve, and $5.6 million, net of a $0.6 million reserve, respectively.

Recent Accounting Pronouncements

In May 2014, the FASB issued ASU No. 2014-09, Revenue from Contracts with Customers (Topic 606), which outlines a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers and supersedes most current revenue recognition guidance, including industry-specific guidance. ASU 2014-09 outlines a five-step process for revenue recognition that focuses on transfer of control, as opposed to transfer of risk and rewards, and also requires enhanced disclosures regarding the nature, amount, timing and uncertainty of revenues and cash flows from contracts with customers. Major provisions include determining which goods and services are distinct and require separate accounting (performance obligations), how variable consideration (which may include change orders and claims) is recognized, whether revenue should be recognized at a point in time or over time and ensuring the time value of money is considered in the transaction price.

The FASB issued supplemental adoption guidance and clarification to ASU No. 2014-09 in March 2016, April 2016 and May 2016 within ASU No. 2016-08, Revenue from Contracts with Customers: Principal vs. Agent Considerations, ASU No. 2016-10, Revenue from Contracts with Customers: Identifying Performance Obligations and Licensing and ASU No. 2016-12, Revenue from Contracts with Customers: Narrow-Scope Improvements and Practical Expedients, respectively. ASU No. 2014-09 and the related supplemental ASUs are effective for fiscal years beginning after December 15, 2017 and interim periods therein. The ASU permits two methods of adoption: the full retrospective method or the modified retrospective method. We plan to apply the modified retrospective method upon adoption in the first quarter of 2018 and currently do not anticipate that the adoption of this ASU will have a material impact with regard to our current contracts.

In January 2016, the FASB issued ASU No. 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities, which eliminates the requirement for public companies to disclose the method(s) and significant assumptions used to estimate the fair value for financial instruments measured at amortized cost on the balance sheet. Additionally, the standard requires public companies to use the exit price notion when measuring the fair value of financial instruments for disclosure purposes. Furthermore, the standard requires presentation of financial assets and liabilities by measurement category and form of financial asset on the balance sheet or accompanying notes to the financial statements. The standard is effective for annual reporting periods beginning after December 15, 2017, including interim periods within those annual reporting periods. Early application is permitted. The adoption of this guidance will have no impact on our financial statements.

In February 2016, the FASB issued ASU No. 2016-02, Leases, which increases transparency and comparability among organizations by requiring recognition of lease assets and lease liabilities on the balance sheet and disclosure of key information about leasing arrangements. The standard is effective for annual reporting periods beginning after December 15, 2018, including interim periods within those annual reporting periods. Early application is permitted. We are currently evaluating the impact of adoption on our financial statements.

In March 2016, the FASB issued ASU No. 2016-09, Compensation – Stock Compensation: Improvements to Employee Share-Based Payment Accounting, which was intended to simplify various aspects of accounting for share-based payment transactions. The new guidance requires immediate recognition of all excess tax benefits and deficiencies in the income statement, requires classification of excess tax benefits as an operating activity as opposed to a financing activity in the statements of cash flows and allows the Company to make an accounting policy election to either estimate the number of awards expected to vest or account for forfeitures when they occur. The standard is effective

for annual reporting periods beginning after December 15, 2016, and interim periods within those annual reporting periods. We applied this standard in the first quarter of 2017 using the modified retrospective method of adoption. In conjunction with this adoption, we applied an accounting policy election to account for forfeitures as they occur. Upon adoption, we reversed a deferred tax asset related to the balance of unrecognized excess tax benefits of $7.4 million, with an offsetting adjustment to the valuation allowance. Under the modified retrospective method of adoption, we recorded an adjustment of $0.6 million to accumulated deficit with a corresponding offset to additional paid-in capital.

Table of Contents

In August 2016, the FASB issued ASU No. 2016-15, Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments, which addresses the presentation and classification of certain cash receipts and cash payments in the statement of cash flows under Accounting Standards Codification 230. The standard is effective for annual reporting periods beginning after December 15, 2017, and interim periods within those annual reporting periods. The adoption of this guidance will have no impact on our financial statements.

In November 2016, the FASB issued ASU No. 2016-18, Statement of Cash Flows: Restricted Cash, which requires restricted cash and restricted cash equivalents to 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 reporting periods beginning after December 15, 2017, and interim periods within those annual reporting periods. Early application is permitted. Upon its adoption in 2018, we will include $1.3 million of restricted cash in our disclosed balance of cash and cash equivalents at the beginning of the period for 2016. We do not expect any additional impact on our financial statements.

In May 2017, the FASB issued ASU No. 2017-09, Compensation - Stock Compensation: Scope of Modification Accounting, which provides clarity and guidance around which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. The standard is effective for annual reporting periods beginning after December 15, 2017, and interim periods within those annual reporting periods. The adoption of this guidance will have no impact on our financial statements unless we have modification accounting in accordance with Topic 718.

2. Net Loss Per Share

Basic net loss per share is calculated by dividing 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 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 plans. For all periods presented, there is no difference in the number of shares used to calculate basic and diluted net loss per share.

Potentially dilutive securities not included in the calculation of diluted net loss per share because to do so would be anti-dilutive consisted of 2,140,111 and 2,869,643 shares attributable to common stock options for the three and six months ended June 30, 2017, respectively, compared to 3,055,143 and 2,639,630 shares attributable to common stock options for the same periods in 2016.

3. Investments

We invest our excess cash primarily in commercial paper and debt instruments of financial institutions, corporations, U.S. government-sponsored agencies and the U.S. Treasury. As of June 30, 2017, our short-term investments had a weighted average maturity of less than one year.

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

	Maturity (in years)	Amortized cost	Unrealized Gains	Unrealized Losses	Estimated fair value
As of June 30, 2017					
Corporate debt securities	1 or less	$ 21,264	$ —	$ (9)	$ 21,255
Certificates of deposit	1 or less	3,785	—	—	3,785
U.S. Treasury securities	1 or less	2,010	—	(1)	2,009
Debt securities of U.S. government-sponsored agencies	1 or less	500	—	—	500
Total		$ 27,559	$ —	$ (10)	$ 27,549

Table of Contents

	Maturity (in years)	Amortized cost	Unrealized Gains	Unrealized Losses	Estimated fair value
As of December 31, 2016					
Corporate debt securities	2 or less	$ 49,185	$ 12	$ (77)	$ 49,120
Certificates of deposit	1 or less	9,291	—	—	9,291
Commercial paper	1 or less	1,247	—	—	1,247
U.S. Treasury securities	1 or less	1,001	—	(1)	1,000
Debt securities of U.S. government-sponsored agencies	1 or less	512	—	—	512
Total		$ 61,236	$ 12	$ (78)	$ 61,170

4. Fair Value Measurements

We have certain financial assets 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 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 standards 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.

Financial Assets Measured at Fair Value

The following table presents our fair value hierarchy for assets measured at fair value on a recurring basis as of June 30, 2017 and December 31, 2016 (in thousands):

	Fair value as of June 30, 2017			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$10,630	$10,630	$—	$ —
Corporate debt securities	21,255	—	21,255	—
Certificates of deposit	3,785	—	3,785	—
U.S. treasury securities	2,009	—	2,009	—
Debt securities of U.S. government-sponsored agencies	500	—	500	—
	$38,179	$10,630	$27,549	$ —

Table of Contents

	Fair value as of December 31, 2016			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$13,578	$13,578	$—	$ —
Corporate debt securities	49,120	—	49,210	—
Certificates of deposit	9,291	—	9,291	—
Commercial paper	1,247	—	1,247	—
U.S. treasury securities	1,000	—	1,000	—
Debt securities of U.S. government-sponsored agencies	512	—	512	—
	$74,748	$13,578	$61,260	$ —

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.

5. Term Loan

On June 17, 2016, we entered into a loan and security agreement ("Loan Agreement") with Oxford Finance, LLC, ("Oxford"), pursuant to which Oxford agreed to lend us up to $30.0 million, issuable in two separate term loans of $20.0 million (the "Term A Loan") and $10.0 million (the "Term B Loan"). On June 22, 2016, we received $20.0 million in proceeds from the Term A Loan, net of debt issuance costs. The ability to borrow on the Term B Loan expired on March 31, 2017. We refer to all amounts outstanding under the Loan Agreement as the Term Loan.

The outstanding Term Loan will mature on June 1, 2020 (the “Maturity Date”) and we will have interest-only payments through June 1, 2018, followed by 24 equal monthly payments of principal and unpaid accrued interest. The Term Loan will bear interest at a floating per annum rate equal to (i) 8.51% plus (ii) the greater of (a) the 30 day U.S. Dollar LIBOR rate reported in The Wall Street Journal on the last business day of the month that immediately precedes the month in which the interest will accrue and (b) 0.44%.

We have the option to prepay all, but not less than all, of the borrowed amount, provided that we will be obligated to pay a prepayment fee equal to (i) 2% of the outstanding principal balance of the Term Loan if prepayment is made prior to the second anniversary of the funding date of the Term Loan, provided no prepayment fee would have been due in connection with a prepayment made on or prior to the first anniversary of the funding date of the Term Loan in connection with an acquisition of our company, or (ii) 1% of the Term Loan prepaid thereafter and prior to the Maturity Date. We will be required to make a final payment of 5.5% of the principal balance outstanding, payable on the earlier of (i) the Maturity Date, (ii) acceleration of the Term Loan, or (iii) the prepayment of the Term Loan.

We may use the proceeds from the Term Loan solely for working capital and to fund our general business requirements. Our obligations under the Loan Agreement are secured by a first priority security interest in substantially all of our current and future assets, other than our intellectual property. We have also agreed not to encumber our intellectual property assets, except as permitted by the Loan Agreement. The Loan Agreement defines certain events of default, including instances of a material adverse change in our operations, that may require prepayment of the outstanding Term Loan. No such events have occurred or are anticipated as of June 30, 2017.

As of June 30, 2017, we had $20.0 million outstanding under the Term Loan. The Term Loan was recorded at its initial carrying value of $20.0 million, less debt issuance costs of approximately $0.2 million. In connection with the Term Loan, the debt issuance costs have been recorded as a debt discount in our consolidated balance sheets, which are being accreted to interest expense over the life of the Term Loan using an effective interest rate of 8.98%. The exit fee is being accrued over the life of the Term Loan through interest expense.

As of June 30, 2017, we were in compliance with all covenants under the Loan Agreement.

Table of Contents

Future principal payments for the Term Loan due under the Loan Agreement are as follows (in thousands):

2017	$—
2018	5,000
2019	10,000
2020	5,000
	$20,000

6. Stockholders' Equity

Shares Reserved for Future Issuance

The following shares of common stock were reserved for future issuance as of June 30, 2017:

Common stock options outstanding	10,819,036
Common stock available for future grant under 2012 Equity Incentive Plan	917,027
Common stock available for future grant under 2015 Inducement Plan	23,126
Employee Stock Purchase Plan	1,844,527
Total common shares reserved for future issuance	13,603,716

The following table summarizes our stock option activity under all equity incentive plans for the six months ended June 30, 2017 (shares in thousands):

	Number of options	Weighted average exercise price
Options outstanding at December 31, 2016	8,931	$ 6.70
Granted	4,255	$ 1.44
Exercised	(8)	$ 0.38
Canceled/forfeited/expired	(2,359)	$ 5.86
Options outstanding at June 30, 2017	10,819	$ 4.82

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 2015 Inducement Plan and the shares purchasable under our Employee Stock Purchase Plan during the periods presented:

Table of Contents

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Stock options				
Risk-free interest rate	1.9 %	1.5 %	2.0 %	1.4 %
Volatility	89.5 %	79.1%	89.4 %	79.7%
Dividend yield	—	—	—	—
Expected term (years)	6.1	5.8	6.1	5.9
Performance stock options				
Risk-free interest rate	—	0.4 %	2.1 %	1.4 %
Volatility	—	78.5%	89.9 %	79.3%
Dividend yield	—	—	—	—
Expected term (years)	0	5.5	5.6	6.0
Employee stock purchase plan shares				
Risk-free interest rate	0.9 %	0.5 %	0.8 %	0.4 %
Volatility	111.5%	82.6%	113.8%	81.0%
Dividend yield	—	—	—	—
Expected term (years)	0.5	0.5	0.5	0.5

The following table summarizes the allocation of our stock-based compensation expense for all stock awards during the periods presented, including the adjustments to stock-based compensation expense associated with our May 2017 corporate restructuring (in thousands):

	Three months ended June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Research and development	$650	$1,299	$1,758	$2,731
Research and development-restructuring related adjustments	(1,399)	—	(1,399)	—
General and administrative	935	939	2,209	3,255
General and administrative-restructuring related adjustments	2,679	—	2,679	—
Total	$2,865	$2,238	$5,247	$5,986

In connection with our May 2017 corporate restructuring, we recorded a reversal of stock-based compensation in research and development expenses of $1.4 million for each of the three and six months ended June 30, 2017, as a result of the cancellation of unvested stock options. We recorded additional stock-based compensation in general and administrative expenses of $2.7 million for each of the three and six months ended June 30, 2017, as a result of termination provisions within certain employment agreements.

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 June 30,		Six months ended June 30,	
	2017	2016	2017	2016
Sanofi	$18	$18	$36	$36
AstraZeneca	—	465	—	936
Total	$18	$483	$36	$972

Sanofi

13

Table of Contents

In July 2012, we amended and restated our collaboration and license agreement with Sanofi to expand the potential therapeutic applications of the microRNA 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 microRNA 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 microRNA 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 June 30, 2017, 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 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 microRNA 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 clinical 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. We are eligible to receive royalties on microRNA 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 allocated consideration into revenue ratably over the estimated period of performance of the miR-221/222 program. As of June 30, 2017, deferred revenue associated with the Purchase Agreement and the 2014 Sanofi Amendment was $0.2 million, which we are expecting to recognize over the remaining estimated period of performance of approximately three 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 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.

Table of Contents

AstraZeneca

In August 2012, we entered into a collaboration and license agreement with AstraZeneca. Under the terms of the agreement, we agreed to collaborate with AstraZeneca to identify, research and develop compounds targeting three microRNA 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 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 were 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 expired in August 2016.

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, did not have stand-alone value. As a result, we recognized revenue related to the upfront payment on a straight-line basis over the period of performance, which was four years based on the term of the research and development plan which expired in August 2016.

In connection with the collaboration and license agreement and concurrently with our initial public offering, we sold AstraZeneca 6,250,000 shares of our common stock in a private placement at a price per share of $4.00. Under the terms of the Common Stock Purchase Agreement ("CSPA"), 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. The CSPA and collaboration and license agreement 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 a discount for lack of marketability, $4.3 million was attributed to the collaboration and license agreement. We recognized the $4.3 million into revenue ratably over the period of performance of the research and development plan under the collaboration, which expired in August 2016.

In March 2015, we earned a $2.5 million preclinical milestone and in December 2015, we earned a $10.0 million clinical milestone. We determined the milestones to be substantive and recognized revenue upon achievement of each milestone.

In June 2017, AstraZeneca delivered written notice of their election to terminate the collaboration and license agreement. Effective upon the termination of the agreement, AstraZeneca’s rights with respect to RG-125(AZD4076) will revert back to us. In accordance with the Agreement, the termination will become effective in June 2018, 12 months following the date of delivery of the notice by AstraZeneca.

8. Related Party Transactions

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

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 engaged Sanofi Deutschland to manufacture RG-012 drug product and perform stability studies on our behalf. Expenses incurred under the agreement for services performed or out-of-pocket expenses were less than $0.1 million for the three and six months ended June 30, 2017, respectively, compared to less than $0.1 million and $0.8 million for the same periods in 2016.

In February 2015, we entered into a letter agreement with Alnylam Pharmaceuticals, Inc. ("Alnylam") pursuant to which we and Alnylam agreed to the financial terms for certain technology acquired by Alnylam within the licensed patent rights under our Amended and Restated License and Collaboration Agreement (the “Additional Patent Rights”) with Alnylam and Ionis Pharmaceuticals, Inc. In addition to any royalties payable by us to Alnylam pursuant to the terms of the Amended and Restated License and 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. There was no activity under this agreement for the three or six months ended June 30, 2017 and 2016.

Table of Contents

9. Subsequent Events

In July 2017, we completed an underwritten public offering of 50,600,000 shares of common stock at an offering price of $0.91 per share. We received net proceeds from the offering of approximately $43.0 million after deducting underwriting discounts, commissions and other estimated offering expenses payable by us.

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, 2016 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, 2016, or Annual Report, filed with the Securities and Exchange Commission on March 3, 2017. 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 all 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 clinical trials;
- our ability to obtain and maintain regulatory approval of our 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 product candidates;
- our strategic alliance partners' election to pursue development and commercialization of any programs or product candidates that are subject to our collaboration and license agreements with such partners;
- our ability to attract collaborators with relevant development, regulatory and commercialization expertise;
- future activities to be undertaken by our strategic alliance partners, collaborators and other third parties;
- our ability to obtain and maintain intellectual property protection for our product candidates;
- the size and growth potential of the markets for our 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 product candidates;
- the rate and degree of market acceptance of our 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 may become available;
- the loss of key scientific or management personnel;
- our ability to successfully secure and deploy capital;

Table of Contents

- our ability to satisfy our debt obligations;
- the expected benefits to be achieved from our May 2017 restructuring, including with respect to the expected reduction in our operating expenses and the extension of our cash runway;
- 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;
- the accuracy of our estimates regarding future expenses, future revenues, capital requirements and need for additional financing; and
- the risks and other forward-looking statements described under the caption “Risk Factors” under Part II, Item 1A of this quarterly report on Form 10-Q.

OVERVIEW

We are a clinical-stage biopharmaceutical company focused on discovering and developing first-in-class drugs targeting microRNAs to treat diseases with significant unmet medical need. We were formed in 2007 when Alnylam Pharmaceuticals, Inc., or Alnylam, and Ionis Pharmaceuticals, Inc., or Ionis, contributed significant intellectual property, know-how and financial and human capital to pursue the development of drugs targeting microRNAs pursuant to a license and collaboration agreement. Our most advanced program, under our strategic alliance with Sanofi, is RG-012, an anti-miR targeting miR-21 for the treatment of Alport syndrome, a life-threatening kidney disease driven by genetic mutations, currently with no approved therapy available.

microRNAs are naturally occurring ribonucleic acid, or RNA, molecules that play a critical role in regulating key biological pathways. Scientific research has shown that an imbalance, or dysregulation, of microRNAs is directly linked to many diseases. Furthermore, many different infectious pathogens interact and bind to host microRNA to survive. To date, over 500 microRNAs have been identified in humans, each of which can bind to multiple messenger RNAs that control key aspects of cell biology. Since many diseases are multi-factorial, involving multiple targets and pathways, the ability to modulate multiple pathways by targeting a single microRNA 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, microRNAs are RNAs that do not code for proteins but rather are responsible for regulating gene expression by modulating the translation and decay of target messenger RNAs. By interacting with many messenger RNAs, a single microRNA can regulate the expression of multiple genes involved in the normal function of a biological pathway. Many pathogens, including viruses, bacteria and parasites, also use host microRNAs to regulate the cellular environment for survival. In some instances, the host microRNAs are essential for the replication and/or survival of the pathogen. For example, miR-122 is a microRNA expressed in human hepatocytes and is a key factor for the replication of the hepatitis C virus, or HCV.

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

- microRNAs play a critical role in regulating biological pathways by controlling the translation of many target genes;
- microRNA therapeutics regulate disease pathways which may result in more effective treatment of complex multi-factorial diseases;
- many human pathogens, including viruses, bacteria and parasites, use microRNAs (host and pathogen encoded) to enable their replication and suppression of host immune responses; and
- microRNA therapeutics may be synergistic with other therapies because of their different mechanism of action.

We believe we have assembled the leading position in the microRNA field, including expertise in microRNA biology and oligonucleotide chemistry, a broad intellectual property estate, relationships with key opinion leaders and a disciplined drug discovery and development process. We are using our microRNA expertise to develop chemically modified, single-stranded oligonucleotides that we call anti-miRs to modulate microRNAs and address underlying disease. We believe microRNAs may play a critical role in complex disease and that targeting them with anti-miRs may become a source of a new and major class of drugs with broad therapeutic application, much like small

molecules, biologics and monoclonal antibodies.

Table of Contents

We believe that microRNA biomarkers may be used to select optimal patient segments in clinical trials and to monitor disease progression or relapse. We believe these microRNA biomarkers can be applied toward drugs that we develop and drugs developed by other companies with which we partner or collaborate. We have completed a research collaboration with Biogen Inc. focused on the discovery of microRNAs as biomarkers for multiple sclerosis and have also completed research for another leading, commercial-stage pharmaceutical company to explore microRNAs as biomarkers for specific patient populations. We also maintain several academic research collaborations focused on the identification of microRNAs as biomarkers in multiple disease areas.

Development Stage Pipeline

We currently have multiple programs in various stages of clinical and preclinical development.

RG-012: In 2015, we completed a Phase I study to evaluate the safety, tolerability, and pharmacokinetics, or PK, of subcutaneous dosing of RG-012 in healthy volunteers. Forty healthy volunteer subjects were enrolled in this first-in-human, single ascending dose study. In May 2017, we completed a Phase I multiple-ascending dose, or MAD, study in 24 healthy volunteers (six-week repeat dosing) to determine safety, tolerability and PK of RG-012 prior to chronic dosing in patients. In both Phase I studies, RG-012 was well-tolerated, and there were no serious adverse events, or SAEs, reported. We also continue to enroll Alport syndrome patients in our global ATHENA natural history of disease study, which is designed to characterize the disease-related decline of renal function (as measured by established blood markers for renal function) in these patients over time. In mid-2017, we are planning to initiate HERA, the Phase II randomized (1:1), double-blinded, placebo-controlled study evaluating the safety and efficacy of RG-012 in 40 Alport syndrome patients. In parallel, a renal biopsy study is also planned to evaluate RG-012 renal tissue PK, target engagement and downstream effects on genomic disease biomarkers. Data from the renal biopsy study is anticipated by year-end and interim data from HERA is anticipated mid-2018.

RGLS4326: In December 2016, we nominated RGLS4326 as a clinical candidate targeting microRNA-17 (miR-17) for the treatment of autosomal dominant polycystic kidney disease, or ADPKD. IND-enabling toxicology, repeat pharmacology and manufacturing work have been completed as scheduled to support regulatory submissions as part of the investigational new drug, or IND, package. We anticipate filing an IND or foreign equivalent regulatory filing by the end of 2017.

RG-101: In June 2016, we received verbal notice from the U.S. Food and Drug Administration, or FDA, that our IND for RG-101 for the treatment of chronic HCV infection was placed on clinical hold. The FDA initiated the clinical hold after a second RG-101 treated patient experienced an SAE of jaundice. In December 2016, we submitted a complete response to the FDA's initial request for information, which included identification of a potential mechanism of hyperbilirubinemia. We also submitted a proposal to mitigate this risk. In January 2017, we received written communication from the FDA that the clinical development program for RG-101 remained on clinical hold. The FDA requested the complete safety and efficacy data from on-going RG-101 clinical and preclinical studies before reconsidering the clinical hold. The FDA also requested additional expert review of liver safety data considering the proposed mechanism of hyperbilirubinemia. In June 2017, we announced our plan to discontinue clinical development of RG-101 upon completion of the follow-up phase of the remaining RG-101 clinical study, which occurred in July 2017. Comprehensive preclinical investigation and detailed analysis of clinical data from the RG-101 program have identified the direct inhibition of a hepatocyte conjugated bilirubin transporter as the likely mechanism for the cases of hyperbilirubinemia in the RG-101 program. We believe that a combination of factors, including inhibition of conjugated bilirubin transport by RG-101, impaired baseline bilirubin transport in HCV patients and the preferential uptake of RG-101 by hepatocytes contributed to this mechanism. Additional patient-specific contributing factors cannot be excluded. Applying the learnings from the RG-101 program, alternative compounds targeting miR-122 have been identified that maintain potent HCV antiviral activity while lacking inhibition of the bilirubin transporter. We believe these compounds have the potential for rapid clinical proof-of-concept of a novel, markedly shortened treatment regimen for HCV and will be considered for further development pending an updated global commercial

market assessment for HCV.

RG-125(AZD4076): In June 2017, AstraZeneca delivered written notice of their election to terminate the collaboration and license agreement. Effective upon the termination of the agreement, AstraZeneca's rights with respect to RG-125(AZD4076) for the treatment of non-alcoholic steatohepatitis, or NASH, in Type 2 Diabetes/Pre-diabetes will revert to us. In accordance with the Agreement, the termination will become effective in June 2018, which is 12 months following the date of delivery of the notice by AstraZeneca.

RGLS5040: In November 2016, we nominated RGLS5040 as a clinical candidate targeting microRNA-27 (miR-27) for the treatment of cholestatic diseases. In June 2017, we discontinued development of RGLS5040 based on a positioning of the compound with respect to the competitive landscape coupled with the results from repeat pharmacology studies as part of IND-enabling work. We continue to work on developing therapeutics for genetic forms of cholestatic disease as part of our overall research activities targeting unmet diseases of the liver and kidney.

Table of Contents

Preclinical Pipeline

A major focus of our preclinical research is targeting dysregulated microRNAs implicated in diseases of high unmet medical need where we know we can effectively deliver to the target tissue or organ, such as the liver and kidney. Multiple microRNAs have been identified as being dysregulated in NASH and these are in the process of target validation including the evaluation of tool compounds in animal models of NASH. Profiling of primary tumor cells from glioblastoma multiforme, or GBM, a rapidly fatal form of brain cancer, has identified miR-10b as a microRNA target with the potential to inhibit tumor growth. We are investigating local and systemic delivery of anti-miR-10b oligonucleotides in preclinical models to evaluate potential for advancing this program to clinical testing in GBM. We also have early discovery programs investigating additional microRNA targets for infectious diseases.

FINANCIAL OPERATIONS OVERVIEW

Revenue

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, payments for research and development services, milestone payments, product sales and royalties in connection with our existing strategic alliance with Sanofi or future strategic alliances for one or more of our programs we establish, if any. 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 any strategic alliance partner. If Sanofi does not elect or otherwise agree to fund development costs for our RG-012 program for the treatment of Alport syndrome pursuant to our strategic alliance agreement, or if we or any potential future strategic alliance partner 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 and the development of our therapeutic programs. 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. Certain of the raw materials used in the process of manufacturing drug product are capitalized upon their acquisition and expensed upon usage, as we have determined these materials have alternative future use.

To date, we have conducted research on many different microRNAs 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 known 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 clinical development program, RG-012 for the treatment of Alport syndrome, and preclinical pipeline.

Since our conversion to a corporation in January 2009, we have grown from 15 research and development personnel to 49 and have spent a total of approximately $288.6 million in research and development expenses through June 30, 2017.

Table of Contents

The process of conducting clinical trials and preclinical studies 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. 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.

Successful development of future product candidates is highly uncertain and may not result in approved products. Completion dates and completion costs can vary significantly for each future product candidate and are difficult to predict. We 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 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 costs will likely include legal fees, Sarbanes-Oxley compliance and other accounting fees and directors' and officers' liability insurance premiums.

Other income (expense), net

Other income (expense) consists primarily of interest income and expense and various income or expense items of a non-recurring nature. 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 is primarily attributable to interest charges associated with borrowings under our secured term loan from Oxford Finance, LLC, or Oxford.

CRITICAL ACCOUNTING POLICIES AND ESTIMATES

There have been no significant changes to our critical accounting policies since December 31, 2016. For a description of critical accounting policies that affect our significant judgments and estimates used in the preparation of our consolidated financial statements, refer to Item 7 in Management's Discussion and Analysis of Financial Condition and Results of Operations and Note 1 to our financial statements contained in our Annual Report and Note 1 to our condensed financial statements contained in this quarterly report on Form 10-Q.

RESULTS OF OPERATIONS

Comparison of the three and six months ended June 30, 2017 and 2016