Regulus Therapeutics Inc.
Form 10-Q
May 10, 2018

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, 2018

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 ¨

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

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

As of May 4, 2018, the registrant had 104,319,552 shares of Common Stock ($0.001 par value) outstanding.

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, 2018 (Unaudited)	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 10,057	$ 13,519
Short-term investments	35,077	46,555
Contract and other receivables	188	373
Prepaid materials, net	4,655	4,783
Prepaid expenses and other current assets	1,451	1,506
Total current assets	51,428	66,736
Property and equipment, net	9,305	9,708
Intangibles, net	726	775
Other assets	620	590
Total assets	$ 62,079	$ 77,809
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,396	$ 5,743
Accrued liabilities	3,371	2,995
Accrued compensation	1,116	1,985
Current portion of term loan, less debt issuance costs	19,874	19,859
Other current liabilities	2,176	2,018
Total current liabilities	30,933	32,600
Contract liabilities, less current portion	60	1,921
Deferred rent, less current portion	7,772	8,072
Other long-term liabilities	438	—
Total liabilities	39,203	42,593
Commitments and Contingencies		
Stockholders' equity:		
Common stock, $0.001 par value; 200,000,000 shares authorized, 104,319,552 and 103,955,147 shares issued and outstanding at March 31, 2018 (unaudited) and December 31, 2017, respectively	104	104
Additional paid-in capital	382,939	381,104
Accumulated other comprehensive loss	(127)	(134)
Accumulated deficit	(360,040)	(345,858)
Total stockholders' equity	22,876	35,216
Total liabilities and stockholders' equity	$ 62,079	$ 77,809

See accompanying notes to these condensed financial statements.

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

	Three months ended March 31,	
	2018	2017
	(Unaudited)	
Revenues:		
Revenue under strategic alliances and collaborations	$18	$18
Total revenues	18	18
Operating expenses:		
Research and development	11,828	15,752
General and administrative	3,773	3,959
Total operating expenses	15,601	19,711
Loss from operations	(15,583)	(19,693)
Other income (expense):		
Interest and other income	164	214
Interest and other expense	(605)	(546)
Loss before income taxes	(16,024)	(20,025)
Income tax (expense) benefit	(1)	4
Net loss	$(16,025)	$(20,021)
Other comprehensive loss:		
Unrealized gain on short-term investments, net	7	26
Comprehensive loss	$(16,018)	$(19,995)
Net loss per share, basic and diluted	$(0.15)	$(0.38)
Weighted average shares used to compute basic and diluted net loss per share	104,018,275	52,990,383

See accompanying notes to these condensed financial statements.

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

	Three months ended March 31,	
	2018	2017
	(Unaudited)	
Operating activities		
Net loss	$(16,025)	$(20,021)
Adjustments to reconcile net loss to net cash used in operating activities		
Depreciation and amortization expense	563	699
Stock-based compensation	1,627	2,381
Amortization of premium on investments, net	47	107
Other	93	135
Change in operating assets and liabilities:		
Contracts and other receivables	185	1,218
Prepaid materials	128	(660)
Prepaid expenses and other assets	26	627
Accounts payable	(1,347)	(1,331)
Accrued liabilities	377	(818)
Accrued compensation	(868)	(1,012)
Deferred rent and other liabilities	(408)	(5)
Net cash used in operating activities	(15,602)	(18,680)
Investing activities		
Purchases of short-term investments	—	(2,265)
Sales and maturities of short-term investments	11,439	20,780
Purchases of property and equipment	—	(139)
Acquisition of intangibles	—	(16)
Net cash provided by investing activities	11,439	18,360
Financing activities		
Proceeds from issuance of common stock, net	208	265
Proceeds from exercise of common stock options	1	3
Proceeds from capital lease financing	492	—
Net cash provided by financing activities	701	268
Net decrease in cash and cash equivalents	(3,462)	(52)
Cash and cash equivalents at beginning of period	13,519	14,941
Cash and cash equivalents at end of period	$10,057	$14,889
Supplemental disclosure of cash flow information		
Interest paid	$(506)	$(465)
Income taxes paid	$(1)	$(1)
Supplemental disclosure of non-cash investing and financing activities		
Non-cash acquisition of property and equipment	$191	$—
Amounts accrued for property and equipment	$—	$9

See accompanying notes to these condensed financial statements.

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, 2017, from which the balance sheet information herein was derived.

Liquidity

The accompanying financial statements have been prepared on a basis which assumes we are a going concern, and does not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classifications of liabilities that may result from any uncertainty related to our ability to continue as a going concern. Through the date of the issuance of these financial statements, we have principally been financed through proceeds received from the sale of our common stock and other equity securities, debt financings, up-front payments and milestones received from collaboration agreements. As of March 31, 2018, we had approximately $45.1 million of cash, cash equivalents and short-term investments. Based on our operating plans, we believe our cash, cash equivalents and short-term investments may not be sufficient to fund our operations for the period one year following the issuance of these financial statements. As a result, there is substantial doubt about the Company's ability to continue as a going concern. All amounts due under the Term Loan (see note 5) have been classified as a current liability as of March 31, 2018 and December 31, 2017 due to the considerations discussed above and the assessment that the material adverse change clause under the Term Loan is not within the Company's control. The Company has not been notified of an event of default by the Lender as of the date of the filing of this Form 10-Q.

We intend to seek additional capital through equity and/or debt financings, collaborative or other funding arrangements with partners or through other sources of financing. Should we seek additional financing from outside sources, we may not be able to raise such financing on terms acceptable to us, or at all. If we are unable to raise additional capital when required or on acceptable terms, we may be required to scale back or discontinue the advancement of product candidates, reduce headcount, file for bankruptcy, reorganize, merge with another entity, or cease operations.

If the Company becomes unable to continue as a going concern, we may have to liquidate our assets, and might realize significantly less than the values at which they are carried on our financial statements, and stockholders may lose all or part of their investment in our common stock.

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.

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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.

Effective January 1, 2018, we adopted Accounting Standards Update ("ASU") 2014-09, Revenue from Contracts with Customers (Topic 606) ("Topic 606") using the modified retrospective method which consisted of applying and recognizing the cumulative effect of Topic 606 at the date of initial application. Topic 606 supersedes the revenue recognition requirements in Accounting Standards Codification ("ASC") Topic 605, Revenue Recognition ("Topic 605"). All periods prior to the adoption date of Topic 606 have not been restated to reflect the impact of the adoption of Topic 606, but are accounted for and presented under Topic 605.

The following paragraphs in this section describe our revenue recognition accounting polices under Topic 606 upon adoption on January 1, 2018. Refer to Note 1 to the financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2017 for revenue recognition accounting policies under Topic 605.

We recognize revenue when we transfer promised goods or services to customers in an amount that reflects the consideration to which we expect to be entitled in exchange for those goods or services. To determine revenue recognition for contracts with customers we perform the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligation(s) in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligation(s) in the contract; and (v) recognize revenue when (or as) we satisfy the performance obligation(s). At contract inception, we assess the goods or services promised within each contract, assess whether each promised good or service is distinct and identify those that are performance obligations. We recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Collaborative Arrangements

We enter into collaborative arrangements with partners that typically include payment to us of one of more of the following: (i) license fees; (ii) payments related to the achievement of developmental, regulatory, or commercial milestones; and (iii) royalties on net sales of licensed products. Where a portion of non-refundable up-front fees or other payments received are allocated to continuing performance obligations under the terms of a collaborative arrangement, they are recorded as contract liabilities and recognized as revenue when (or as) the underlying performance obligation is satisfied.

As part of the accounting for these arrangements, we must develop estimates and assumptions that require judgment to determine the underlying stand-alone selling price for each performance obligation which determines how the transaction price is allocated among the performance obligation(s). The stand-alone selling price may include items such as forecasted revenues, development timelines, discount rates, and probabilities of technical and regulatory success. We evaluate each performance obligation to determine if it can be satisfied at a point in time, or over time. In addition, variable consideration must be evaluated to determine if it is constrained and, therefore, excluded from the transaction price.

License Fees

If a license to our intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, we recognize revenues from non-refundable, up-front fees allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other performance obligations, we use judgment to assess the nature of the combined performance obligation to determine whether it is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition.

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Milestone Payments

At the inception of each arrangement that includes milestone payments (variable consideration), we evaluate whether the milestones are considered probable of being reached and estimate the amount to be included in the transaction price. If it is probable that a milestone event would occur at the inception of an arrangement, the associated milestone value is included in the transaction price. Milestone payments that are contingent upon the achievement of events that are uncertain or not controllable, such as regulatory approvals, are generally not considered probable of being achieved until those approvals are received, and therefore not included in the transaction price. The transaction price is then allocated to each performance obligation on a relative stand-alone selling price basis, for which we recognize revenue as or when the performance obligations under the contract are satisfied. At the end of each reporting period, we evaluate the probability of achievement of such milestones and any related constraint(s), and if necessary, may adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect license, collaboration or other revenues and earnings in the period of adjustment.

Royalties

For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and for which the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any of our collaborative arrangements.

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 restricted stock units by determining the fair value of each restricted stock unit based on the closing market price of our common stock on the date of grant. We recognize stock-based compensation expense using the accelerated multiple-option approach over the requisite service periods of the awards.

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.

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 expense the cost of materials when used. We periodically review these capitalized materials for continued alternative future use and write down the

asset to its net realizable value in the period in which it is identified. As of March 31, 2018 and December 31, 2017, our net prepaid materials balance was $4.7 million and $4.8 million, respectively.

Recent Accounting Pronouncements

As disclosed above, effective January 1, 2018, we adopted Topic 606. Since ASU 2014-09 was issued, several additional ASUs have been issued and incorporated within Topic 606 to clarify various elements of the guidance. As part of our adoption efforts, we have completed the assessment of our collaboration and license agreements under Topic 606. We adopted Topic 606 in the first quarter of 2018 using the modified retrospective method which consists of applying and recognizing the cumulative effect of Topic 606 at the date of initial application and providing certain additional disclosures as defined per Topic 606. On January 1, 2018, we recorded a cumulative adjustment to decrease deferred revenue and accumulated deficit by approximately $1.8 million to reflect the impact of the adoption of Topic 606. The cumulative adjustment relates primarily to our agreement with Sanofi which is described further in Note 7.

Below is a summary of the affected line items of the condensed balance sheets upon adoption of Topic 606 (in thousands):

	Balance at December 31, 2017	Adjustments due to Topic 606	Balance at January 1, 2018
Balance Sheet			
Deferred revenue, non-current	1,921	(1,844)	77
Accumulated deficit	(345,858)	1,844	(344,014)

There is no difference between what our revenue would have been in the three months ended March 31, 2018, reported under Topic 606 or Topic 605.

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. The adoption of this guidance had 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 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 had 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. 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 in our Annual Report on Form 10-K. There is no 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. We applied this guidance in the

first quarter of 2018. The adoption of this guidance had no impact on our financial statements and will continue to have no impact on our financial statements unless we have modification accounting in accordance with Topic 718.

In December 2017, The Tax Cuts and Jobs Act (the "Act") was signed into law and amended the Internal Revenue Code, or IRC, to reduce tax rates and modify policies, credits, and deductions for individuals and businesses. Due to uncertainties which currently exist in the interpretation of the provisions of the Act regarding IRC Section 162(m), as of March 31, 2018, we have not completed our evaluation of the potential impacts of IRC Section 162(m) as amended by the Act on our financial statements. We have provisionally determined that there is no deferred tax benefit or expense with respect to the re-measurement of certain deferred tax assets and liabilities due to the full valuation allowance against net deferred tax assets. Additional analysis of the law and the impact to the company will be performed and any impact will be recorded in the respective quarter.

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 8,108,448 shares attributable to common stock options and restricted stock units for the three months ended March 31, 2018, compared to 1,824,296 shares attributable to common stock options for the three months ended March 31, 2017.

3. Investments

We invest our excess cash primarily in debt instruments of financial institutions, corporations, U.S. government-sponsored agencies and the U.S. Treasury. We generally hold our investments to maturity and do not sell our investments before we have recovered our amortized cost basis.

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

	Maturity (in years)	Amortized cost	Unrealized Gains	Unrealized Losses	Estimated fair value
As of March 31, 2018					
Corporate debt securities	1 or less	$ 23,373	$ —	$ (46)	$ 23,327
Certificates of deposit	1 or less	6,278	—	—	6,278
U.S. Treasury securities	1 or less	3,997	—	(20)	3,977
Debt securities of U.S. government-sponsored agencies	1 or less	1,499	—	(4)	1,495
Total		$ 35,147	$ —	$ (70)	$ 35,077

	Maturity (in years)	Amortized cost	Unrealized Gains	Unrealized Losses	Estimated fair value
As of December 31, 2017					
Corporate debt securities	1 or less	$ 32,922	$ —	$ (55)	$ 32,867
Certificates of deposit	1 or less	8,216	—	—	8,216
U.S. Treasury securities	1 or less	3,996	—	(18)	3,978
Debt securities of U.S. government-sponsored agencies	1 or less	1,498	—	(4)	1,494
Total		$ 46,632	$ —	$ (77)	$ 46,555

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 March 31, 2018 and December 31, 2017 (in thousands):

	Fair value as of March 31, 2018			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$8,540	$8,540	$—	$ —
Corporate debt securities	23,327	—	23,327	—
Certificates of deposit	6,278	—	6,278	—
U.S. treasury securities	3,977	—	3,977	—
Debt securities of U.S. government-sponsored agencies	1,495	—	1,495	—
	$43,617	$8,540	$35,077	$ —

	Fair value as of December 31, 2017			
	Total	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$10,847	$10,847	$—	$ —
Corporate debt securities	32,867	—	32,867	—
Certificates of deposit	8,216	—	8,216	—
U.S. treasury securities	3,978	—	3,978	—
Debt securities of U.S. government-sponsored agencies	1,494	—	1,494	—
	$57,402	$10,847	$46,555	$ —

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" or sometimes referred to as the "Lender"), 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, and no amounts were borrowed under the Term B Loan. 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%. In March 2018, we entered into an amendment to our Loan Agreement, providing for the modification of the loan amortization period from a 24-month period commencing on July 1, 2018 to a 15-month period commencing on April 1, 2019, subject to our receipt, following the date of the amendment, of unrestricted net cash proceeds of not less than $30.0 million on or prior to June 30, 2018.

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, 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 and certain assets under capital

lease obligations. We have also agreed not to encumber our intellectual property assets, except as permitted by the Loan Agreement. The Loan Agreement includes customary events of default, including instances of a material adverse change in our operations, that may require prepayment of the outstanding Term Loan. All amounts due under the Term Loan have been classified as a current liability as of March 31, 2018 due to the considerations discussed in Note 1 and the assessment that the material adverse change clause under the Term Loan is not within the Company's control. The Company has not been notified of an event of default by the Lender as of the date of the filing of this Form 10-Q.

As of March 31, 2018, we had $20.0 million outstanding under the Term Loan. The Term Loan is recorded at its carrying value of $20.0 million, less debt issuance costs of approximately $0.1 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 March 31, 2018, we were in compliance with all covenants under the Loan Agreement.

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

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 March 31, 2018:

Common stock options outstanding	14,030,591
Restricted stock units outstanding	311,799
Common stock available for future grant under 2012 Equity Incentive Plan	1,421,771
Common stock available for future grant under 2015 Inducement Plan	41,948
Employee Stock Purchase Plan	1,918,590
Total common shares reserved for future issuance	17,724,699

The following table summarizes our stock option activity under all equity incentive plans for the three months ended March 31, 2018 (shares in thousands):

	Number of options	Weighted average exercise price
Options outstanding at December 31, 2017	10,649	$ 4.44
Granted	3,655	$ 1.24
Exercised	(4)	$ 0.38
Canceled/forfeited/expired	(269)	$ 4.65
Options outstanding at March 31, 2018	14,031	$ 3.60

We granted 415,728 restricted stock units during the year ended December 31, 2017 with a weighted average grant date fair value per share of $0.89. All such restricted stock units remained outstanding at December 31, 2017. During the three months ended March 31, 2018, 103,929 restricted stock units vested. No restricted stock units were granted or canceled during the three months ended March 31, 2018. At March 31, 2018, 311,799 restricted stock units remained outstanding with a weighted average grant date fair value per share of $0.89.

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:

	Three months ended March 31,	
	2018	2017
Stock options		
Risk-free interest rate	2.6 %	2.1 %
Volatility	87.9%	89.3 %
Dividend yield	—	—
Expected term (years)	6.1	6.1
Performance stock options		
Risk-free interest rate	2.7 %	2.1 %
Volatility	87.4%	89.9 %
Dividend yield	—	—
Expected term (years)	5.7	5.6
Employee stock purchase plan shares		
Risk-free interest rate	1.4 %	0.7 %
Volatility	89.0%	116.0%
Dividend yield	—	—
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:

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	Three months ended March 31,	
	2018	2017
Research and development	$666	$1,108
General and administrative	961	1,273
Total	$1,627	$2,381

7. Strategic Alliances and Collaborations

Revenue recognized from our strategic alliances and collaborations was less than $0.1 million for each the three months ended March 31, 2018 and 2017.

Sanofi

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 was deferred as of December 31, 2017 and recorded as an adjustment to accumulated deficit upon our adoption of ASC 606 on January 1, 2018. 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 has 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 ("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 March 31, 2018, deferred revenue associated with the Purchase Agreement and the 2014 Sanofi Amendment was $0.1 million, which we are expecting to recognize over the remaining estimated period of performance of approximately two 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.

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 each the three months ended March 31, 2018 and 2017.

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, 2017 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, 2017, or Annual Report, filed with the Securities and Exchange Commission on March 8, 2018. 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 contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. For these forward-looking statements, we claim the protection 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;
- the potential election of any strategic alliance or collaboration partner to pursue development and commercialization of any programs or product candidates that are subject to a collaboration with such partner;
- 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;
- our ability to satisfy our debt obligations;
- 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.

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In addition, statements that "we believe" and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this report, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and investors are cautioned not to unduly rely upon these statements.

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 two lead product candidates, RG-012 and RGLS4326, are currently in clinical development. RG-012 is an anti-miR targeting miR-21 for the treatment of Alport syndrome, a life-threatening kidney disease with no approved therapy available. RGLS4326 is an anti-miR targeting miR-17 for the treatment of autosomal dominant polycystic kidney disease, or ADPKD. In addition to these programs, we continue to develop a pipeline of preclinical drug product candidates.

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 deoxyribonucleic acid, or 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.

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.

Development Stage Pipeline

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

RG-012: In the third quarter of 2017, we initiated 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 was also initiated in the third quarter of 2017 to evaluate RG-012 renal tissue pharmacokinetics, or PK, target engagement and
downstream effects on genomic disease biomarkers. In December 2017, we concluded our global ATHENA natural history of disease 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 Phase I clinical studies to date, RG-012 was well-tolerated, and there were no serious adverse events, or SAEs, reported. RG-012 has received orphan designation in both the United States and Europe.

RGLS4326: In April 2018, we initiated a Phase I randomized, double-blind, placebo-controlled, MAD study in healthy volunteers designed to characterize the safety, tolerability, pharmacokinetics and pharmacodynamics of multiple doses of RGLS4326. In March 2018, we completed dose escalation of a Phase I single ascending dose, or SAD, study in healthy volunteers and found RGLS4326 was well tolerated and no SAEs were reported. RGLS4326 is a novel oligonucleotide designed to inhibit miR-17 using a unique chemistry designed to preferentially deliver to the kidney. Preclinical studies with RGLS4326 have demonstrated a reduction in kidney cyst formation, improved kidney weight/body weight ratio, decreased cyst cell proliferation, and preserved kidney function in mouse models of ADPKD.
RG-125(AZD4076): In June 2017, AstraZeneca AB, or 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 May 2018, AstraZeneca requested to extend the Collaboration and License Agreement termination effective date by an additional 12 months to allow AstraZeneca to complete all activities involving AZD4076. By the end of this 12-month extension, the parties will complete the transfer activities contemplated by the agreement. The new termination effective date pursuant to the extension will be June 2019.
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. For example, 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, immunology and indications for which there is microRNA dysregulation or in disease settings where the host microRNAs are essential for the replication and/or survival of the pathogen.

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 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 current or future strategic alliance partners do not elect or otherwise agree to fund our development costs pursuant to our current or future strategic alliance agreement, or we or our strategic alliance partner fails 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 development programs.

Since our conversion to a corporation in January 2009, we have grown from 15 research and development personnel to 50 and, since our inception, have spent a total of approximately $323.5 million in research and development expenses through March 31, 2018.

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 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

and professional fees for auditing, tax and legal services, some of which are incurred as a result of being a publicly-traded company. 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 Act 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.

CRITICAL ACCOUNTING POLICIES AND ESTIMATES

There have been no significant changes to our critical accounting policies since December 31, 2017, with the exception of changes made upon adoption of ASU No. 2014-09 and the related supplemental ASUs. 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. For a description of accounting policy changes resulting from the adoption of ASU No. 2014-09 and the related supplemental ASUs, refer to Note 1 to our condensed financial statements contained in this quarterly report on From 10-Q.

RESULTS OF OPERATIONS

Comparison of the three months ended March 31, 2018 and 2017

The following table summarizes our results of operations for the three months ended March 31, 2018 and 2017 (in thousands):

	Three months ended March 31,	
	2018	2017
Revenue under strategic alliances and collaborations	$18	$ 18
Research and development expenses	11,828	15,752
General and administrative expenses	3,773	3,959
Interest and other expenses, net	(441)	(332)

Revenue under strategic alliances and collaborations

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. Revenue under our strategic alliance was less than $0.1 million for the three months ended March 31, 2018 and 2017. As of March 31, 2018, we had $0.1 million of deferred revenue, which consisted of payments received through our strategic alliance that have not yet been recognized in accordance with our revenue recognition policies. Upon adoption of ASU No 2014-09 and the related supplemental ASUs, we reclassified $1.8 million of contract liabilities into accumulated deficit through the modified retrospective method of adoption.

Research and development expenses

The following tables summarize the components of our research and development expenses for the periods indicated, together with year-over-year changes (dollars in thousands):

	Three months ended March 31, 2018	% of total	Three months ended March 31, 2017	% of total	Increase (decrease) $	%
Research and development						
Personnel and internal expenses	$4,643	39 %	$6,557	42 %	$(1,914)	(29)%
Third-party and outsourced expenses	6,291	53 %	7,493	47 %	(1,202)	(16)%
Non-cash stock-based compensation	666	6 %	1,108	7 %	(442)	(40)%
Depreciation	228	2 %	594	4 %	(366)	(62)%
Total research and development expenses	$11,828	100%	$15,752	100%	$(3,924)	

Research and development expenses were $11.8 million for the three months ended March 31, 2018, compared to $15.8 million for the three months ended March 31, 2017. The decrease in research and development expenses for the three months ended March 31, 2018 compared to the three months ended March 31, 2017 was driven by a $1.9 million reduction in personnel and internal costs, primarily attributable to a reduction in costs subsequent to our May 2017 corporate restructuring, and a $1.2 million decrease in external development expenses, primarily attributable to the discontinuation of the RG-101 and RGLS5040 programs in 2017.

General and administrative expenses

General and administrative expenses were $3.8 million for the three months ended March 31, 2018, compared to $4.0 million for the three months ended March 31, 2017. These amounts reflect personnel-related and ongoing general business operating costs.

Interest and other expenses, net

Net interest and other expenses were $0.4 million for the three months ended March 31, 2018, compared to $0.3 million for the three months ended March 31, 2017, primarily related to interest charges associated with our outstanding $20.0 million Term Loan.

LIQUIDITY AND CAPITAL RESOURCES

Since our inception through March 31, 2018, we have received $85.1 million from our strategic alliances and collaborations, principally from upfront payments, research funding and preclinical and clinical milestones, $300.1 million from the sale of our equity and convertible debt securities and $19.8 million in net proceeds from our Term Loan. As of March 31, 2018, we had cash, cash equivalents and marketable securities of $45.1 million.

The accompanying financial statements have been prepared on a basis which assumes we are a going concern, and does not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classifications of liabilities that may result from any uncertainty related to our ability to continue as a going concern.

If we are unable to maintain sufficient financial resources, our business, financial condition and results of operations will be materially and adversely affected. There can be no assurance that we will be able to obtain the needed financing on acceptable terms or at all. Additionally, equity or debt financings may have a dilutive effect on the holdings of the Company’s existing stockholders. These factors raise substantial doubt about our ability to continue as a going concern.

Our future capital requirements are difficult to forecast and will depend on many factors, including:

- whether and when we achieve any milestones under our strategic alliance agreement with Sanofi;
- the terms and timing of any other strategic alliance, licensing and other arrangements that we may establish;
- the initiation, progress, timing and completion of preclinical studies and clinical trials for our development programs and product candidates, and associated costs;
- the number and characteristics of product candidates that we pursue;

- the outcome, timing and cost of regulatory approvals;
- delays that may be caused by changing regulatory requirements;
- the cost and timing of hiring new employees to support our continued growth;
- the costs involved in filing and prosecuting patent applications and enforcing and defending patent claims;

- the costs and timing of procuring clinical and commercial supplies of our product candidates;
- the costs and timing of establishing sales, marketing and distribution capabilities;
- the extent to which we acquire or invest in businesses, products or technologies; and
- payments under our Term Loan.

The following table shows a summary of our cash flows for the three months ended March 31, 2018 and 2017 (in thousands):

	Three months ended March 31,	
	2018	2017
	(unaudited)	
Net cash (used in) provided by:		
Operating activities	$(15,602)	$(18,680)
Investing activities	11,439	18,360
Financing activities	701	268
Total	$(3,462)	$(52)

Operating activities

Net cash used in operating activities was $15.6 million for the three months ended March 31, 2018, compared to $18.7 million for the three months ended March 31, 2017. The decrease in net cash used in operating activities was primarily attributable to a net loss of $16.0 million for the three months ended March 31, 2018, compared to a net loss of $20.0 million for the three months ended March 31, 2017. This decrease was partially offset by adjustments for non-cash charges, including stock-based compensation, resulting in net cash used in operating activities of $2.3 million for the three months ended March 31, 2018, compared to net cash used in operating activities of $3.3 million for the three months ended March 31, 2017.

Investing activities

Net cash provided by investing activities for the periods presented primarily related to the net of purchases, sales and maturities of investments used to fund our operations. We invest cash in excess of our immediate operating requirements in a way that maturity is staggered and designed to optimize our return on investment, while satisfying our liquidity needs. Net cash provided by the net sales and maturities of short-term investments was $11.4 million for the three months ended March 31, 2018, compared to $18.5 million for the three months ended March 31, 2017.

Financing activities

Net cash provided by financing activities was $0.7 million for the three months ended March 31, 2018, compared to $0.3 million for the three months ended March 31, 2017.

CONTRACTUAL OBLIGATIONS AND COMMITMENTS

As of March 31, 2018, there have been no material changes, outside of the ordinary course of business, in our outstanding contractual obligations from those disclosed within “Management's Discussion and Analysis of Financial Condition and Results of Operations”, as contained in our Annual Report.

Off-Balance Sheet Arrangements

As of March 31, 2018, we did not have any off-balance sheet arrangements.

ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Some of the securities that we invest in have market risk where a change in prevailing interest rates may cause the principal amount of the marketable securities to fluctuate. Financial instruments that potentially subject us to significant concentrations of credit risk consist primarily of cash, cash equivalents and short-term investments. We invest our excess cash primarily in debt instruments of financial institutions, corporations, U.S. government-sponsored agencies and the U.S. Treasury. The primary objectives of our investment activities are to ensure liquidity and to preserve principal while at the same time maximizing the income we receive from our marketable securities without significantly increasing risk. Additionally, we

established guidelines regarding approved investments and maturities of investments, which are designed to maintain safety and liquidity.

Because of the short-term maturities of our cash equivalents and marketable securities, we do not believe that an increase in market rates would have any significant impact on the realized value of our marketable securities. If a 10% change in interest rates were to have occurred on March 31, 2018, this change would not have had a material effect on the fair value of our investment portfolio as of that date.

We also have interest rate exposure as a result of our outstanding $20.0 million Term Loan. As of March 31, 2018, the outstanding principal amount of the Term Loan was $20.0 million. The Term Loan bears 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%. Changes in the U.S. Dollar LIBOR rate may therefore affect our interest expense associated with the Term Loan.

If a 10% change in interest rates were to have occurred on March 31, 2018, this change would not have had a material effect on our interest expense as of that date.

ITEM 4. CONTROLS AND PROCEDURES

Disclosure Controls and Procedures

We maintain disclosure controls and procedures that are designed to provide reasonable assurance that information required to be disclosed in our periodic and current reports that we file with the SEC is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our principal executive officer and our principal financial and accounting officer, as appropriate, to allow timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable and not absolute assurance of achieving the desired control objectives. In reaching a reasonable level of assurance, management is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures. In addition, the design of any system of controls also is based, in part, upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a cost-effective control system, misstatements due to error or fraud may occur and not be detected.

As of March 31, 2018, we carried out an evaluation, under the supervision and with the participation of our management, including our principal executive officer and our principal financial and accounting officer, of the effectiveness of the design and operation of our disclosure controls and procedures, as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act. Based on this evaluation, our principal executive officer and our principal financial and accounting officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of March 31, 2018.

Changes in Internal Control Over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as such term is defined in Rule 13a-15(f) of the Exchange Act. An evaluation was also performed under the supervision and with the participation of our management, including our principal executive officer and our principal financial and accounting officer, of any change in our internal control over financial reporting that occurred during our last fiscal quarter and that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. That evaluation did not identify any change in our internal control over financial reporting that occurred during our latest fiscal quarter that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. OTHER INFORMATION

ITEM 1. LEGAL PROCEEDINGS

On January 31, 2017, a putative class action complaint was filed by Baran Polat in the United States District Court for the Southern District of California, or District Court, against us, Paul C. Grint (our former Chief Executive Officer),

and Joseph P. Hagan (then our Chief Operating Officer and currently our President and Chief Executive Officer). The complaint includes claims asserted, on behalf of certain purchasers of our securities, under Sections 10(b) and 20(a) of the Securities Exchange Act of 1934, as amended. In general, the complaint alleges that, between January 21, 2016, and June 27, 2016, the defendants violated the federal securities laws by making materially false and misleading statements regarding our business and the prospects for RG-101, thereby artificially inflating the price of our securities. The plaintiff seeks unspecified monetary damages and other relief. On February 10, 2017, a second putative class action complaint was filed by Li Jin in the District Court against the Company, Mr. Hagan, Dr. Grint, and Timothy Wright, the Company's Chief Research and Development Officer. The Complaint alleges claims similar to those asserted by Mr. Polat. The actions have been related. On February17, 2017, the District Court entered an order stating that defendants need not answer, or otherwise respond, until the District Court enters an order appointing, pursuant to the Private Securities Litigation Reform Act of 1995, lead plaintiff and lead counsel, and the parties then submit a schedule to the District Court for the filing of an amended or consolidated complaint and the timing of

defendants' answer or response. On April 3, 2017, two motions for consolidation of the two actions, appointment of lead plaintiff and approval of counsel were filed in the actions, or the Consolidation and Lead Plaintiff Motions. On October 26, 2017, the District Court entered an order consolidating the cases, appointing lead plaintiffs, and appointing lead counsel for lead plaintiffs. On December 22, 2017, lead plaintiffs filed a consolidated complaint against the Company, Dr. Grint, Mr. Hagan, and Michael Huang (our former Vice President of Clinical Development). The consolidated complaint alleges that between February 17, 2016 and June 12, 2017, the Defendants violated Sections 10(b) and 20(a) of the Securities Exchange Act of 1934, as amended, by making materially false and misleading statements regarding RG-101. The consolidated complaint seeks unspecified monetary damages and an award of attorneys' fees and costs. On February 6, 2018, defendants filed a Motion to Dismiss the Consolidated Complaint. On March 23, 2018, plaintiff filed their opposition to the motion and on April 24, 2018, defendants filed their response. No hearing date has been set. We intend to vigorously defend this matter.

ITEM 1A. RISK FACTORS

You should carefully consider the following risk factors, as well as the other information in this report, before deciding whether to purchase, hold or sell shares of our common stock. The occurrence of any of the following risks could harm our business, financi