

RIGEL PHARMACEUTICALS INC

Form 10-Q

November 01, 2016

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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

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FORM 10-Q

(Mark One)

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

FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2016

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 0-29889

Rigel Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	94-3248524
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)

1180 Veterans Blvd.	
South San Francisco, CA	94080
(Address of principal executive offices)	(Zip Code)

(650) 624-1100

(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 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 No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer

Accelerated filer

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Non-accelerated filer

Smaller reporting company

(Do not check if a smaller reporting company)

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act).

Yes No

As of October 26, 2016, there were 98,937,197 shares of the registrant's Common Stock outstanding.

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RIGEL PHARMACEUTICALS, INC.

QUARTERLY REPORT ON FORM 10-Q

FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2016

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## PART I. FINANCIAL INFORMATION

## Item 1. Financial Statements

## RIGEL PHARMACEUTICALS, INC.

## CONDENSED BALANCE SHEETS

(In thousands)

	September 30, 2016 (unaudited)	December 31, 2015(1)
Assets		
Current assets:		
Cash and cash equivalents	\$ 25,950	\$ 43,456
Short-term investments	59,305	82,820
Accounts receivable	—	203
Prepaid and other current assets	1,292	2,545
Total current assets	86,547	129,024
Property and equipment, net	1,294	1,613
Other assets	800	1,110
	\$ 88,641	\$ 131,747
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 1,518	\$ 2,763
Accrued compensation	4,552	6,251
Accrued research and development	7,686	4,953
Other accrued liabilities	1,041	1,133
Deferred revenue	—	13,427
Deferred liability – sublease, current portion	3,167	3,005
Deferred rent, current portion	2,668	2,264
Total current liabilities	20,632	33,796
Long-term portion of deferred liability – sublease	1,064	3,460
Long-term portion of deferred rent	994	3,083
Other long-term liabilities	8	27

Commitments

Stockholders' equity:

Preferred stock	—	—
Common stock	99	91
Additional paid-in capital	1,111,107	1,082,980
Accumulated other comprehensive gain (loss)	9	(44)
Accumulated deficit	(1,045,272)	(991,646)
Total stockholders' equity	65,943	91,381
	\$ 88,641	\$ 131,747

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(1) The balance sheet at December 31, 2015 has been derived from the audited financial statements included in Rigel's Annual Report on Form 10-K for the year ended December 31, 2015.

See Accompanying Notes.

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RIGEL PHARMACEUTICALS, INC.

## CONDENSED STATEMENTS OF OPERATIONS

(In thousands, except per share amounts)

(unaudited)

	Three Months Ended September 30, 2016		Nine Months Ended September 30, 2015	
	2016	2015	2016	2015
Contract revenues from collaborations	\$ 3,760	\$ 12,996	\$ 17,383	\$ 20,358
Costs and expenses:				
Research and development	16,171	15,501	51,812	46,262
General and administrative	4,558	4,276	13,755	13,092
Restructuring charges	5,770	—	5,770	—
Total costs and expenses	26,499	19,777	71,337	59,354
Loss from operations	(22,739)	(6,781)	(53,954)	(38,996)
Interest income	110	54	328	162
Gain on disposal of assets	—	55	—	57
Net loss	\$ (22,629)	\$ (6,672)	\$ (53,626)	\$ (38,777)
Net loss per share, basic and diluted	\$ (0.24)	\$ (0.08)	\$ (0.58)	\$ (0.44)
Weighted average shares used in computing net loss per share, basic and diluted	95,454	88,506	92,844	88,231

See Accompanying Notes.

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RIGEL PHARMACEUTICALS, INC.

## CONDENSED STATEMENTS OF COMPREHENSIVE LOSS

(In thousands)

(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Net loss	\$ (22,629)	\$ (6,672)	\$ (53,626)	\$ (38,777)
Other comprehensive income (loss):				
Net unrealized gain (loss) on short-term investments	(44)	14	53	32
Comprehensive loss	\$ (22,673)	\$ (6,658)	\$ (53,573)	\$ (38,745)

See Accompanying Notes.

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RIGEL PHARMACEUTICALS, INC.

## CONDENSED STATEMENTS OF CASH FLOWS

(In thousands)

(unaudited)

	Nine Months Ended September 30,	
	2016	2015
Operating activities		
Net loss	\$ (53,626)	\$ (38,777)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization	798	1,101
Stock-based compensation expense	4,667	5,778
Non-cash restructuring charges	818	—
Gain on disposal of assets	—	(57)
Changes in assets and liabilities:		
Accounts receivable	203	5,587
Prepaid and other current assets	1,253	(39)
Other assets	130	52
Accounts payable	(1,245)	(924)
Accrued compensation	(1,699)	1,529
Accrued research and development	2,733	1,206
Other accrued liabilities	(92)	571
Deferred revenue	(13,427)	18,261
Deferred rent and other long term liabilities	(3,938)	(3,904)
Net cash used in operating activities	(63,425)	(9,616)
Investing activities		
Purchases of short-term investments	(82,523)	(109,882)
Maturities of short-term investments	106,091	142,097
Proceeds from disposal of assets	—	60
Capital expenditures	(798)	(412)
Net cash provided by investing activities	22,770	31,863
Financing activities		
Net proceeds from issuances of common stock upon exercise of options and participation in employee stock purchase plan	932	1,127
Proceeds from sale and issuance of common stock, net of offering costs	22,217	—
Net cash provided by financing activities	23,149	1,127
Net increase (decrease) in cash and cash equivalents	(17,506)	23,374
Cash and cash equivalents at beginning of period	43,456	15,203
Cash and cash equivalents at end of period	\$ 25,950	\$ 38,577

See Accompanying Notes.

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Rigel Pharmaceuticals, Inc.

Notes to Condensed Financial Statements

(unaudited)

In this report, “Rigel,” “we,” “us” and “our” refer to Rigel Pharmaceuticals, Inc.

1.Nature of Operations

We were incorporated in the state of Delaware on June 14, 1996. We are engaged in the discovery and development of novel, targeted drug candidates in the therapeutic areas of immunology, oncology and immuno-oncology.

2.Basis of Presentation

Our accompanying unaudited condensed financial statements have been prepared in accordance with U.S. generally accepted accounting principles (U.S. GAAP), for interim financial information and pursuant to the instructions to Form 10-Q and Article 10 of Regulation S-X of the Securities Act of 1933, as amended (Securities Act). Accordingly, they do not include all of the information and notes required by U.S. GAAP for complete financial statements. These unaudited condensed financial statements include only normal and recurring adjustments that we believe are necessary to fairly state our financial position and the results of our operations and cash flows. Interim-period results are not necessarily indicative of results of operations or cash flows for a full-year or any subsequent interim period. The balance sheet at December 31, 2015 has been derived from audited financial statements at that date, but does not include all disclosures required by U.S. GAAP for complete financial statements. Because all of the disclosures required by U.S. GAAP for complete financial statements are not included herein, these interim unaudited condensed financial statements and the notes accompanying them should be read in conjunction with our audited financial statements and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2015.

The preparation of financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. Actual results could differ from these estimates.

3.Recent Accounting Pronouncements

In August 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standard Update (ASU) No. 2014-15—Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern, under Accounting Standards Codification (ASC) Subtopic 205-40, Presentation of Financial Statements—Going Concern. ASU No. 2014-15 provides guidance about management’s responsibility to evaluate whether there is substantial doubt about an entity’s ability to continue as a going concern and to provide related footnote disclosures. Management’s evaluation should be based on relevant conditions and events that are known and reasonably knowable at the date that the financial statements are issued (or at the date that the financial statements are available to be issued when applicable). Substantial doubt about an entity’s ability to continue as a going concern exists when relevant conditions and events, considered in the aggregate, indicate that it is probable that the entity will be unable to meet its obligations as they become due within one year after the date that the financial statements are issued (or available to be issued). ASU No. 2014-15 is effective for the annual period ending after December 15, 2016 and early adoption is permitted. We plan to adopt this new standard in our annual financial statements for the year ending December 31, 2016. We will continue to evaluate the guidance under ASU No. 2014-15 and present the required disclosures within our financial statements at the time of adoption. The actual impact will be dependent upon our liquidity and the nature or significance of future events or conditions that exist upon the adoption of this new standard.

In May 2014, the FASB issued ASU No. 2014-09—Revenue from Contracts with Customers, which supersedes the revenue recognition requirements under ASC Topic 605, Revenue Recognition, and most industry-specific guidance under the ASC. The core principle of ASU No. 2014-09 is that an entity should recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be

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entitled in exchange for those goods or services. ASU No. 2014-09 defines a five step process to achieve this core principle and, in doing so, it is possible more judgment and estimates may be required within the revenue recognition process than required under existing U.S. GAAP including identifying performance obligations in the contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. ASU No. 2014-09 also requires additional disclosures to enable users of financial statements to understand the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts. ASU No. 2014-09 allows for either full retrospective or modified retrospective adoption, and we have not yet determined which approach we will apply. In July 2015, the FASB deferred by one year the effective date of ASU No. 2014-09 with the new effective date beginning after December 15, 2017, and the interim periods within that year and will allow early adoption for all entities as of the original effective date for public business entities, which was annual reporting periods beginning after December 15, 2016. We plan to adopt this new standard on January 1, 2018 using the modified retrospective approach. We are currently evaluating the potential impact of the adoption of ASU No. 2014-09 on our financial statements and cannot estimate the impact of adoption at this time.

In February 2016, the FASB issued ASU No. 2016-02—Leases, which is aimed at making leasing activities more transparent, and requires substantially all leases be recognized by lessees on their balance sheet as a right-of-use asset and corresponding lease liability, including leases currently accounted for as operating leases. The guidance is effective for all interim and annual reporting periods beginning after December 15, 2018. Early adoption is permitted. We plan to adopt this new standard on January 1, 2019. We are currently evaluating the potential impact of the adoption of ASU No. 2016-02 on our financial statements and cannot estimate the impact of adoption at this time.

In March 2016, the FASB issued ASU No. 2016-09—Improvements to Employee Share-Based Payment Accounting, which is intended to simplify several aspects of the accounting for share-based payment award transactions, including the income tax consequences, an option to recognize gross stock compensation expense with actual forfeitures recognized as they occur, as well as certain classifications on the statement of cash flows. The guidance will be effective for the fiscal year beginning after December 15, 2016, including interim periods within that year. We plan to adopt this new standard on January 1, 2017 and do not expect a material impact on our financial statements.

#### 4. Stock Award Plans

We have three stock option plans, our 2011 Equity Incentive Plan (2011 Plan), 2000 Equity Incentive Plan (2000 Plan) and 2000 Non-Employee Directors' Stock Option Plan (Directors' Plan), that provide for granting to our officers, directors and all other employees and consultants options to purchase shares of our common stock. We also have our Employee Stock Purchase Plan (Purchase Plan), wherein eligible employees can purchase shares of our common stock at a price per share equal to the lesser of 85% of the fair market value on the first day of the offering period or 85% of the fair market value on the purchase date. The fair value of each option award is estimated on the date of grant using the Black-Scholes option pricing model which considered our stock price, as well as assumptions regarding a number of complex and subjective variables. These variables include, but are not limited to, volatility, expected term, risk-free interest rate and dividends. We estimate volatility over the expected term of the option using historical share price

performance. For expected term, we take into consideration our historical data of options exercised, cancelled and expired. The risk-free rate is based on the U.S. Treasury constant maturity rate. We have not paid and do not expect to pay dividends in the foreseeable future. In order to calculate stock-based compensation expense, we also estimate the forfeiture rate using our historical experience with options that cancel before they vest. We review our forfeiture rates each quarter and make any necessary changes to our estimates. We use the straight-line attribution method over the requisite employee service period for the entire award in recognizing stock-based compensation expense.

We granted performance-based stock options to purchase shares of our common stock which will vest upon the achievement of certain corporate performance-based milestones. We determined the fair values of these performance-based stock options using the Black-Scholes option pricing model at the date of grant. For the portion of the performance-based stock options of which the performance condition is considered probable of achievement, we recognize stock-based compensation expense on the related estimated fair value of such options on a straight-line basis from the date of grant up to the date when we expect the performance condition will be achieved. For the performance

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conditions that are not considered probable of achievement at the grant date or upon quarterly re-evaluation, prior to the event actually occurring, we recognize the related stock-based compensation expense when the event occurs or when we can determine that the performance condition is probable of achievement. In those cases, we recognize the change in estimate at the time we determine the condition is probable of achievement (by recognizing stock-based compensation expense as cumulative catch-up adjustment as if we had estimated at the grant date that the performance condition would have been achieved) and recognize the remaining compensation cost up to the date when we expect the performance condition will be achieved, if any.

## 5. Net Loss Per Share

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding during the period and the number of additional shares of common stock that would have been outstanding if potentially dilutive securities had been issued. Potentially dilutive securities include a warrant to purchase our common shares and stock options and shares issuable under our stock award plans. The dilutive effect of these potentially dilutive securities is reflected in diluted earnings per share by application of the treasury stock method. Under the treasury stock method, an increase in the fair market value of our common stock can result in a greater dilutive effect from potentially dilutive securities.

We had securities which could potentially dilute basic loss per share, but were excluded from the computation of diluted net loss per share, as their effect would have been antidilutive. These securities consist of the following (in thousands):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Outstanding stock options	20,897	19,711	20,897	19,711
Warrant to purchase common stock	200	200	200	200
Purchase Plan	63	78	63	78
	21,160	19,989	21,160	19,989

## 6. Stock-based Compensation

Total stock-based compensation expense related to all of our share-based payments that we recognized for the three and nine months ended September 30, 2016 and 2015 were as follows (in thousands):

	Three Months Ended		Nine Months	
	September 30,		September 30,	
	2016	2015	2016	2015
Research and development	\$ 643	\$ 966	\$ 2,746	\$ 3,182
General and administrative	572	849	1,921	2,596
Restructuring charges	499	—	499	—
Total stock-based compensation expense	\$ 1,714	\$ 1,815	\$ 5,166	\$ 5,778

During the second quarter of 2016, we entered into severance agreements with certain employees. As part of the severance arrangements, we extended the date through which such former employees had the right to exercise their vested options. In addition, we also accelerated the vesting of certain unvested stock options granted to a former employee. As a result of these modifications, we recorded incremental stock-based compensation expense of \$641,000 during the nine months ended September 30, 2016. The incremental compensation expense was computed based on the fair values of the modified awards on the respective modification dates. This amount is included as part of “Research and development expense” in the accompanying Condensed Statements of Operations.

In September 2016, we entered into a severance agreement with a former executive (see Note 13). As part of the severance arrangement we offered, we extended the date through which such former executive had the right to exercise



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his vested options. In addition, we also accelerated the vesting of certain of his unvested stock options. As a result of these modifications, we recorded incremental stock-based compensation expense of approximately \$499,000 during the three and nine months ended September 30, 2016. This amount is included as part of “Restructuring charges” in the accompanying Condensed Statement of Operations.

The fair value of each option award is estimated on the date of grant using the Black-Scholes option pricing model. We have segregated option awards into the following three homogenous groups for the purposes of determining fair values of options: officers and directors, all other employees, and consultants.

We determined weighted-average valuation assumptions separately for each of these groups as follows:

- Volatility—We estimated volatility using our historical share price performance over the expected life of the option. We also considered other factors, such as implied volatility, our current clinical trials and other company activities that may affect the volatility of our stock in the future. We determined that at this time historical volatility is more indicative of our expected future stock performance than implied volatility.
- Expected term—For options granted to consultants, we use the contractual term of the option, which is generally ten years, for the initial valuation of the option and the remaining contractual term of the option for the succeeding periods. We analyzed various historical data to determine the applicable expected term for each of the other option groups. This data included: (1) for exercised options, the term of the options from option grant date to exercise date; (2) for cancelled options, the term of the options from option grant date to cancellation date, excluding non-vested option forfeitures; and (3) for options that remained outstanding at the balance sheet date, the term of the options from option grant date to the end of the reporting period and the estimated remaining term of the options. The consideration and calculation of the above data gave us reasonable estimates of the expected term for each employee group. We also considered the vesting schedules of the options granted and factors surrounding exercise behavior of the option groups, our current market price and company activity that may affect our market price. In addition, we considered the optionee type (i.e., officers and directors or all other employees) and other factors that may affect the expected term of the option.
  - Risk-free interest rate—The risk-free interest rate is based on U.S. Treasury constant maturity rates with similar terms to the expected term of the options for each option group.
- Dividend yield—The expected dividend yield is 0% as we have not paid and do not expect to pay dividends in the future.

Pursuant to FASB ASC 718, we are required to estimate the amount of expected forfeitures when calculating compensation costs. We estimated the forfeiture rate using our historical experience with non-vested options. We adjust our stock-based compensation expense as actual forfeitures occur, review our estimated forfeiture rates each quarter and make changes to our estimate as appropriate.

The following table summarizes the weighted-average assumptions relating to options granted pursuant to our equity incentive plans, including the performance-based stock option awards which will vest upon the achievement of a corporate performance-based milestone, for the three and nine months ended September 30, 2016 and 2015:

	Three Months Ended			Nine Months Ended			
	September 30,			September 30,			
	2016	2015		2016	2015	2016	2015
Risk-free interest rate	—	1.9	%	1.7	%	1.8	%
Expected term (in years)	—	6.0		6.4		6.5	
Dividend yield	—	0.0	%	0.0	%	0.0	%
Expected volatility	—	59.6	%	63.3	%	65.0	%

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The exercise price of stock options is at the market price of our common stock on the date immediately preceding the date of grant. Options become exercisable at varying dates and generally expire 10 years from the date of grant.

During the three months ended September 30, 2016, we did not issue any stock options. During the nine months ended September 30, 2016, we granted options to purchase 3,833,435 shares of common stock, with a grant-date weighted-average fair value of \$1.59 per share. Of the 3,833,435 common stock options granted, 700,000 shares were related to outstanding performance-based stock option awards and has a grant date fair value of \$1.1 million. In addition, as of September 30, 2016, we have 200,000 shares of outstanding performance-based stock option awards granted in 2014 with a grant date fair value of \$520,000. These performance-based stock option awards will vest upon the achievement of a corporate performance-based milestone. We did not consider the achievement of the corporate-based milestone as probable as of September 30, 2016. Accordingly, no stock-based compensation cost was recognized during the three and nine months ended September 30, 2016 for these performance-based stock option awards.

We granted options to purchase 3,811,920 shares of common stock during the nine months ended September 30, 2015, with a grant-date weighted-average fair value of \$1.40 per share. Of the 3,811,920 common stock options granted, 1,175,000 shares were related to performance-based stock option awards which vested upon the achievement of a corporate performance-based milestone in the first quarter of 2016.

As of September 30, 2016, there was approximately \$5.0 million of total unrecognized stock-based compensation cost, net of estimated forfeitures, related to all unvested options granted under our equity incentive plans. Of this amount, approximately \$1.6 million of unrecognized stock compensation expense relate to the performance-based stock option awards, of which the underlying corporate performance-based milestone was not probable of achievement as of September 30, 2016.

At September 30, 2016, there were 5,460,280 shares of common stock available for future grant under our equity incentive plans and 145,493 options to purchase shares were exercised during the nine months ended September 30, 2016.

## Employee Stock Purchase Plan

Our Employee Stock Purchase Plan (Purchase Plan) permits eligible employees to purchase common stock at a discount through payroll deductions during defined offering periods. The price at which the stock is purchased is equal to the lesser of 85% of the fair market value of the common stock on the first day of the offering or 85% of the fair market value of our common stock on the purchase date. The initial offering period commenced on the effective date of our initial public offering.

The fair value of awards granted under our Purchase Plan is estimated on the date of grant using the Black-Scholes option pricing model, which uses weighted-average assumptions. Our Purchase Plan provides for a twenty-four month offering period comprised of four six-month purchase periods with a look-back option. A look-back option is a provision in our Purchase Plan under which eligible employees can purchase shares of our common stock at a price per share equal to the lesser of 85% of the fair market value on the first day of the offering period or 85% of the fair market value on the purchase date. Our Purchase Plan also includes a feature that provides for a new offering period to begin when the fair market value of our common stock on any purchase date during an offering period falls below the fair market value of our common stock on the first day of such offering period. This feature is called a “reset.” Participants are automatically enrolled in the new offering period. We had a “reset” on July 1, 2016 because the fair market value of our stock on June 30, 2016 was lower than the fair market value of our stock on January 5, 2015, the first day of the offering period. We applied modification accounting in accordance with ASC Topic No. 718, Stock Compensation, to determine the incremental fair value associated with this Purchase Plan “reset” and will recognize the related stock-based compensation expense according to FASB ASC Subtopic No. 718-50, Employee Share Purchase Plans. The total incremental fair value for this Purchase Plan “reset” was approximately \$1.0 million and will be recognized from July 1, 2016 to June 30, 2018.

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As of September 30, 2016, there were approximately 2,689,635 shares reserved for future issuance under the Purchase Plan. The following table summarizes the weighted-average assumptions related to our Purchase Plan for the nine months ended September 30, 2016 and 2015. Expected volatilities for our Purchase Plan are based on the historical volatility of our stock. Expected term represents the weighted-average of the purchase periods within the offering period. The risk-free interest rate for periods within the expected term is based on U.S. Treasury constant maturity rates.

	Nine Months Ended			
	September 30,		2015	
	2016	%	0.6	%
Risk-free interest rate	0.5	%	0.6	%
Expected term (in years)	1.5		1.5	
Dividend yield	0.0	%	0.0	%
Expected volatility	63.0	%	61.2	%

## 7. Research and Development Accruals

We have various contracts with third parties related to our research and development activities. Costs that are incurred but not billed to us as of the end of the period are accrued. We make estimates of the amounts incurred in each period based on the information available to us and our knowledge of the nature of the contractual activities generating such costs. Clinical trial contract expenses are accrued based on units of activity. Expenses related to other research and development contracts, such as research contracts, toxicology study contracts and manufacturing contracts are estimated to be incurred generally on a straight-line basis over the duration of the contracts. Raw materials and study materials purchased for us by third parties are expensed at the time of purchase.

## 8. Sponsored Research and License Agreements

We conduct research and development programs independently and in connection with our corporate collaborators. We are a party to a collaboration agreement with Bristol-Myers Squibb Company (BMS) for the discovery, development and commercialization of cancer immunotherapies based on our small molecule TGF beta receptor kinase inhibitors, as discussed below. Our participation in the collaboration is limited to the Joint Research Committee and the performance of research activities based on billable full-time equivalent fees as specified in the collaboration agreement. We do not have ongoing participation obligations under our agreements with Aclaris Therapeutics International Limited (Aclaris) for the development and commercialization of certain janus kinase (JAK) inhibitors for the treatment of alopecia areata and other dermatological conditions, AstraZeneca (AZ) for the development and commercialization of R256, an inhaled JAK inhibitor, BerGenBio AS (BerGenBio) for the development and commercialization of an oncology program, and Daiichi Sankyo (Daiichi) to pursue research related to a specific

target from a novel class of drug targets called ligases. Under these agreements, which we entered into in the ordinary course of business, we received or may be entitled to receive upfront cash payments, progress dependent contingent payments on events achieved by such partners and royalties on any net sales of products sold by such partners under the agreements. Total future contingent payments to us under all of these current agreements could exceed \$531.9 million if all potential product candidates achieved all of the payment triggering events under all of our current agreements (based on a single product candidate under each agreement). Of this amount, up to \$148.8 million relates to the achievement of development events, up to \$345.6 million relates to the achievement of regulatory events and up to \$37.5 million relates to the achievement of certain commercial or launch events. This estimated future contingent amount does not include any estimated royalties that could be due to us if the partners successfully commercialize any of the licensed products. Future events that may trigger payments to us under the agreements are based solely on our partners' future efforts and achievements of specified development, regulatory and/or commercial events.

In October 2015, we entered into a non-exclusive license agreement with a third party, pursuant to which we received a payment in the single-digit millions in exchange for granting a non-exclusive license to certain limited intellectual property rights. We concluded that the granting of the license, which was fully delivered to such third party in the fourth quarter of 2015, represents the sole deliverable under this agreement. Accordingly, we recognized the payment as revenue during the fourth quarter of 2015.

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In August 2015, we entered into a license agreement with Aclaris, pursuant to which Aclaris will have exclusive rights and will assume responsibility for the continued development of certain JAK inhibitor compounds for the treatment of alopecia areata and other dermatological conditions. Under the license agreement, we received a noncreditable and non-refundable upfront payment of \$8.0 million in September 2015. We are also entitled to receive development and regulatory contingent fees that could exceed \$80.0 million for a successful compound approved in certain indications. In addition, we are also eligible to receive tiered royalties on the net sales of any products under the agreement. We concluded that the granting of the license, which has been fully delivered to Aclaris in the third quarter of 2015, represents the sole deliverable under this agreement. Accordingly, we recognized the \$8.0 million payment as revenue during the third quarter of 2015.

In February 2015, we entered into a collaboration agreement with BMS for the discovery, development and commercialization of cancer immunotherapies based on our extensive portfolio of small molecule TGF beta receptor kinase inhibitors. Under the collaboration agreement, BMS will have exclusive rights and will be solely responsible for the clinical development and commercialization of any products. Pursuant to the collaboration agreement with BMS, we received a noncreditable and non-refundable upfront payment of \$30.0 million in March 2015. We are also entitled to receive development and regulatory contingent fees that could exceed \$309.0 million for a successful compound approved in certain indications. In addition, we are also eligible to receive tiered royalties on the net sales of any products from the collaboration. BMS shall also reimburse us for agreed upon costs based on a contractual cost per full-time equivalent employee in connection with the performance of research activities during the research term. Under the collaboration agreement, we were obligated to provide the following deliverables: (i) granting of license rights to our program, (ii) participation in the Joint Research Committee, and (iii) performance of research activities. We concluded that these deliverables are a single unit of accounting as the license does not have stand-alone value apart from the other deliverables. Accordingly, the \$30.0 million upfront payment was recognized ratably as revenue from the effective date of the agreement through September 2016, the end of the research term. We believe that straight-line recognition of this revenue was appropriate as the research was performed ratably over the research period. At the end of the initial research term, we were not notified by BMS of its intention to extend the initial research term under which we would perform research activities. However, BMS does continue to evaluate compounds from the extensive portfolio under the agreement, on its own, for designation of a compound as an early candidate nomination. During the three and nine months ended September 30, 2016, we recognized revenue of \$3.8 million and \$13.4 million, respectively, relating to the upfront payment. During the nine months ended September 30, 2016, we recognized revenue of \$290,000 relating to the research activities we performed. During the three and nine months ended September 30, 2015, we recognized revenue of \$4.8 million and \$11.7 million, respectively, relating to the upfront payment and \$163,000 and \$619,000, respectively, relating to the research activities we performed. We do not have any deferred revenue as of September 30, 2016.

In June 2011, we entered into an exclusive license agreement with BerGenBio for the development and commercialization of an oncology program. BerGenBio is responsible for all activities it wishes to perform under the license we granted to it. In June 2016, we received contingent payments of \$1.7 million relating to a time-based non-refundable fee and \$2.0 million relating to BerGenBio's exercise of certain option rights before the prescription period to exercise the rights expired. All deliverables under the agreement had been previously delivered, as such, the above payments of \$3.7 million triggered by the above time-based and contingent events were recognized as revenue in the second quarter of 2016.





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## 9. Cash, Cash Equivalents and Short-Term Investments

Cash, cash equivalents and short-term investments consisted of the following (in thousands):

	September 30, 2016	December 31, 2015
Cash	\$ 3,714	\$ 2,118
Money market funds	11,182	26,291
U. S. treasury bills	8,307	9,048
Government-sponsored enterprise securities	16,825	48,613
Corporate bonds and commercial paper	45,227	40,206
	\$ 85,255	\$ 126,276
Reported as:		
Cash and cash equivalents	\$ 25,950	\$ 43,456
Short-term investments	59,305	82,820
	\$ 85,255	\$ 126,276

Cash equivalents and short-term investments include the following securities with gross unrealized gains and losses (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
September 30, 2016				
U. S. treasury bills	\$ 8,301	\$ 6	\$ —	\$ 8,307
Government-sponsored enterprise securities	16,814	11	—	16,825
Corporate bonds and commercial paper	45,235	2	(10)	45,227
Total	\$ 70,350	\$ 19	\$ (10)	\$ 70,359

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
December 31, 2015				
U. S. treasury bills	\$ 9,061	\$ —	\$ (13)	\$ 9,048
Government-sponsored enterprise securities	48,643	1	(31)	48,613
Corporate bonds and commercial paper	40,207	11	(12)	40,206

Total	\$ 97,911	\$ 12	\$ (56)	\$ 97,867
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As of September 30, 2016, our cash equivalents and short-term investments, which have contractual maturities within one year, had a weighted-average time to maturity of approximately 84 days. We view our short-term investments portfolio as available for use in current operations. Accordingly, we have classified our investments as short-term investments. We have the ability to hold all investments as of September 30, 2016 through their respective maturity dates. At September 30, 2016, we had no investments that had been in a continuous unrealized loss position for more than 12 months. As of September 30, 2016, a total of 16 individual securities had been in an unrealized loss position for 12 months or less, and the losses were determined to be temporary. The gross unrealized losses above were caused by interest rate increases. No significant facts or circumstances have arisen to indicate that there has been any deterioration in the creditworthiness of the issuers of the securities held by us. Based on our review of these securities, including the assessment of the duration and severity of the unrealized losses and our ability and intent to hold the investments until maturity, there were no other-than-temporary impairments for these securities at September 30, 2016.

The following table shows the fair value and gross unrealized losses of our investments in individual securities that are in an unrealized loss position, aggregated by investment category (in thousands):

September 30, 2016	Fair Value	Unrealized Losses
Corporate bonds and commercial paper	\$ 20,723	\$ (10)

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10.Fair Value

Under FASB ASC 820, Fair Value Measurements and Disclosures, fair value is defined as the price at which an asset could be exchanged or a liability transferred in a transaction between knowledgeable, willing parties in the principal or most advantageous market for the asset or liability. Where available, fair value is based on observable market prices or parameters or derived from such prices or parameters. Where observable prices or parameters are not available, valuation models are applied.

Assets and liabilities recorded at fair value in our financial statements are categorized based upon the level of judgment associated with the inputs used to measure their fair value. Hierarchical levels directly related to the amount of subjectivity associated with the inputs to fair valuation of these assets and liabilities, are as follows:

Level 1—Inputs are unadjusted, quoted prices in active markets for identical assets at the reporting date. Active markets are those in which transactions for the asset or liability occur in sufficient frequency and volume to provide pricing information on an ongoing basis.

The fair valued assets we hold that are generally included under this Level 1 are money market securities where fair value is based on publicly quoted prices.

Level 2—Inputs, other than quoted prices included in Level 1, that are either directly or indirectly observable for the asset or liability through correlation with market data at the reporting date and for the duration of the instrument's anticipated life.

The fair valued assets we hold that are generally assessed under Level 2 included government-sponsored enterprise securities, U.S. treasury bills and corporate bonds and commercial paper. We utilize third party pricing services in developing fair value measurements where fair value is based on valuation methodologies such as models using observable market inputs, including benchmark yields, reported trades, broker/dealer quotes, bids, offers and other reference data. We use quotes from external pricing service providers and other on-line quotation systems to verify the fair value of investments provided by our third party pricing service providers. We review independent auditor's reports from our third party pricing service providers particularly regarding the controls over pricing and valuation of financial instruments and ensure that our internal controls address certain control deficiencies, if any, and complementary user entity controls are in place.

Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities and which reflect management's best estimate of what market participants would use in

pricing the asset or liability at the reporting date. Consideration is given to the risk inherent in the valuation technique and the risk inherent in the inputs to the model.

We do not have fair valued assets classified under Level 3.

#### Fair Value on a Recurring Basis

Financial assets measured at fair value on a recurring basis are categorized in the tables below based upon the lowest level of significant input to the valuations (in thousands):

	Assets at Fair Value as of September 30, 2016			
	Level 1	Level 2	Level 3	Total
Money market funds	\$ 11,182	\$ —	\$ —	\$ 11,182
U. S. treasury bills	—	8,307	—	8,307
Government-sponsored enterprise securities	—	16,825	—	16,825
Corporate bonds and commercial paper	—	45,227	—	45,227
Total	\$ 11,182	\$ 70,359	\$ —	\$ 81,541

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	Assets at Fair Value as of December 31, 2015			
	Level 1	Level 2	Level 3	Total
Money market funds	\$ 26,291	\$ —	\$ —	\$ 26,291
U. S. treasury bills	—	9,048	—	9,048
Government-sponsored enterprise securities	—	48,613	—	48,613
Corporate bonds and commercial paper	—	40,206	—	40,206
Total	\$ 26,291	\$ 97,867	\$ —	\$ 124,158

## 11.Sublease Agreement

In December 2014, we entered into a sublease agreement with an unrelated third party to occupy a portion of our research and office space. We expect to receive approximately \$3.7 million in future sublease income (excluding our subtenant's share of facilities operating expenses) over the remaining term of the sublease. In connection with this sublease, we recognized a loss on sublease of \$9.3 million during the fourth quarter of 2014. We record rent expense on a straight-line basis for our lease, net of sublease income, wherein such arrangements contain scheduled rent increases over the term of the lease and sublease, respectively. For our sublease arrangement which we classified as an operating lease, our loss on the sublease was comprised of the present value of our future payments to our landlord less the present value of our future rent payments expected from our subtenant over the term of the sublease. The liability arising from this sublease agreement was determined using a credit-adjusted risk-free rate to discount the estimated future net cash flows. The changes in the liability related to the sublease agreement for the nine months ended September 30, 2016 were as follows (in thousands):

Balance at January 1, 2016	\$ 6,465
Accretion of deferred liability	288
Amortization of deferred liability	(2,522)
Balance at September 30, 2016	\$ 4,231

## 12.Controlled Equity Offering

In August 2015, we entered into a Controlled Equity Offering<sup>SM</sup> Sales Agreement (Sales Agreement) with Cantor Fitzgerald & Co. (Cantor), as sales agent, pursuant to which we may sell, through Cantor, up to an aggregate of \$30.0 million in shares of our common stock. All sales of our common stock will be made pursuant to a shelf registration statement that was declared effective by the Securities and Exchange Commission (SEC) on July 13, 2015. Cantor acted as our sole sales agent for any sales made under the Sales Agreement for a low single-digit commission on gross

proceeds. The common stock was sold at prevailing market prices at the time of the sale. During the nine months ended September 30, 2016, 7,895,563 shares of common stock were sold under the Sales Agreement, with aggregate gross proceeds of \$24.3 million. As of September 30, 2016, 9,617,875 shares of our common stock had been issued under the Sales Agreement with aggregate gross proceeds of \$30.0 million. Of this amount, \$1.3 million was received subsequent to September 30, 2016. Accordingly, this amount was accounted for as a reduction in Additional Paid-in Capital as of September 30, 2016 in the accompanying Condensed Balance Sheet. As of September 30, 2016, there are no amounts remaining for future sales under the Sales Agreement.

### 13. Restructuring Charges

In September 2016, we announced that we had reduced our workforce by 46 positions, mostly in the research area. We also announced that effective September 15, 2016, Donald G. Payan, M.D, has retired from the board of directors and from his position as Executive Vice President and President of Discovery and Research. We recorded restructuring charges during the three months ended September 30, 2016 of approximately \$5.8 million within Restructuring Charges in the accompanying Statement of Operations, which included \$5.0 million of severance costs paid or to be paid in cash, \$319,000 impairment of certain property and equipment, and \$499,000 of non-cash stock-based compensation expense as a result of the modification of our former executive's stock options (see Note 6). At September 30, 2016, the remaining accrued restructuring costs, which will substantially be paid in the fourth quarter of 2016, consisted of \$1.4 million related to severance, COBRA benefits and outplacement costs for certain employees that were affected by the reduction in workforce. This accrued liability is classified under Accrued Compensation on the Condensed Balance Sheet.

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Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

This discussion and analysis should be read in conjunction with our financial statements and the accompanying notes included in this report and the audited financial statements and accompanying notes included in our Annual Report on Form 10-K for the year ended December 31, 2015. Operating results for the three and nine months ended September 30, 2016 are not necessarily indicative of results that may occur in future interim periods or for the full fiscal year.

This Quarterly Report on Form 10-Q contains statements indicating expectations about future performance and other forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act, that involve risks and uncertainties. We usually use words such as "may," "will," "should," "could," "expect," "plan," "anticipate," "believe," "estimate," "predict," "intend," or the negative of these terms or similar expressions to identify these forward-looking statements. These statements appear throughout this Quarterly Report on Form 10-Q and are statements regarding our current expectation, belief or intent, primarily with respect to our operations and related industry developments. Examples of these statements include, but are not limited to, statements regarding the following: our business and scientific strategies; the progress of our and our collaborators' product development programs, including clinical testing, and the timing of results thereof; our corporate collaborations and revenues that may be received from our collaborations and the timing of those potential payments; our expectations with respect to regulatory submissions and approvals; our drug discovery technologies; our research and development expenses; protection of our intellectual property; sufficiency of our cash and capital resources and the need for additional capital; and our operations and legal risks. You should not place undue reliance on these forward-looking statements. Our actual results could differ materially from those anticipated in these forward-looking statements for many reasons, including as a result of the risks and uncertainties discussed under the heading "Risk Factors" in Item 1A of Part II of this Quarterly Report on Form 10-Q. Any forward-looking statement speaks only as of the date on which it is made, and we undertake no obligation to update any forward-looking statement to reflect events or circumstances after the date on which the statement is made or to reflect the occurrence of unanticipated events. New factors emerge from time to time, and it is not possible for us to predict which factors will arise. In addition, we cannot assess the impact of each factor on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements.

Overview

We are a clinical-stage biotechnology company dedicated to the discovery and development of novel, targeted drugs in the therapeutic areas of immunology, oncology and immuno-oncology. Our pioneering research focuses on signaling pathways that are critical to disease mechanisms. Our current clinical programs include clinical trials of fostamatinib, an oral spleen tyrosine kinase (SYK) inhibitor, in a number of indications. We completed and reported results from two Phase 3 clinical studies of fostamatinib in chronic immune thrombocytopenia (ITP) in August and October 2016. We are also conducting a Phase 2 clinical trial with fostamatinib in autoimmune hemolytic anemia (AIHA) and a Phase 2 clinical trial for IgA nephropathy (IgAN). In addition, we have two oncology product

candidates in Phase 1 development with partners BerGenBio AS and Daiichi Sankyo.

#### Recent Developments

#### Changes in Executive Team

On September 15, 2016, Donald G. Payan, M.D, retired from the board of directors and from his position as Executive Vice President and President of Discovery and Research.

On September 16, 2016, Esteban Masuda, Ph.D. was promoted to Senior Vice President for Research.

Effective October 10, 2016, Eldon C. Mayer III joined the Company as Executive Vice President and Chief Commercial Officer. Also, Joseph Lasaga joined the Company as Vice President, Business Development and Alliance Management.



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Clinical Stage Programs

Fostamatinib—Immune Thrombocytopenic Purpura

Disease background. Chronic ITP affects an estimated 60,000 to 125,000 people in the U.S. In patients with ITP, the immune system attacks and destroys the body's own blood platelets, which play an active role in blood clotting and healing. ITP patients can suffer extraordinary bruising, bleeding and fatigue as a result of low platelet counts. Current therapies for ITP include steroids, blood platelet production boosters that imitate thrombopoietin (TPOs) and splenectomy.

Orally-available SYK inhibitor program. Taken in tablet form, fostamatinib blocks the activation of SYK inside immune cells. ITP causes the body to produce antibodies that attach to healthy platelets in the blood stream. Immune cells recognize these antibodies and affix to them, which activates the SYK enzyme inside the immune cell, and triggers the destruction of the antibody and the attached platelet. When SYK is inhibited by fostamatinib, it interrupts this immune cell function and allows the platelets to escape destruction. The results of our Phase 2 clinical trial, in which fostamatinib was orally administered to sixteen adults with chronic ITP, published in *Blood*, showed that fostamatinib significantly increased the platelet counts of certain ITP patients, including those who had failed other currently available agents.

In October 2013, we met with the U.S. Food and Drug Administration (FDA) for an end-of-Phase 2 meeting for fostamatinib in ITP. Based on that meeting, we designed a Phase 3 clinical program, called fostamatinib in thrombocytopenia (FIT), in which a total of 150 ITP patients were randomized into two identical multi-center, double-blind, placebo-controlled clinical trials. The patients will have been diagnosed with persistent or chronic ITP, and have blood platelet counts consistently below 30,000 per microliter of blood. Two-thirds of the subjects will receive fostamatinib orally at 100 mg bid (twice daily) and the other third will receive placebo on the same schedule. Subjects were expected to remain on treatment for 24 weeks. At week four of treatment, subjects who met certain platelet count and tolerability thresholds had their dosage of fostamatinib (or corresponding placebo) increased to 150 mg bid. The primary efficacy endpoint of this program is a stable platelet response by week 24 with platelet counts at or above 50,000 per microliter of blood for at least four of the final six qualifying blood draws. In August 2015, the FDA granted our request for Orphan Drug designation to fostamatinib, our oral SYK inhibitor, for the treatment of ITP. On April 1, 2016, we announced that we have completed enrollment for both studies in the FIT Phase 3 clinical program of fostamatinib in ITP.

On August 30, 2016, we announced the results of the first study, reporting that fostamatinib met the study's primary efficacy endpoint. The study showed that 18% of patients receiving fostamatinib achieved a stable platelet response compared to none receiving a placebo control (p=0.0261). On October 20, 2016, we announced the results of the second study, reporting that the fostamatinib response rate was 18%, consistent with the first study. However, one

patient in the placebo group (4%) achieved a stable platelet response, therefore the difference between those on treatment and those on placebo did not reach statistical significance ( $p=0.152$ ) and the study did not meet its primary endpoint. When the data from both studies are combined, however, this difference is statistically significant ( $p=0.007$ ). In the combined dataset for both Phase 3 studies, patients who met the primary endpoint had their platelet counts increase from a median of 18,500/uL of blood at baseline to more than 100,000/uL at week 24 of treatment. These patients benefited substantially and typically did so within weeks of initiating treatment, providing early feedback as to whether fostamatinib may be a viable option for treating their ITP. In the combined dataset, the frequency of patients who achieved a stable platelet response was statistically superior in the fostamatinib group versus the placebo group in the following subgroups: prior splenectomy or not; prior exposure to TPO agents or not; platelet counts below or above 15,000/uL of blood at baseline, demonstrating that the effect of fostamatinib is consistent across various clinical and treatment backgrounds.

Patients from both Phase 3 studies were given the option to enroll in a long-term open-label extension study and receive treatment with fostamatinib. As of June 2016, 118 patients had been enrolled in this study. All the patients who responded to fostamatinib in the parent studies enrolled in the long-term open-label extension study and had a median

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platelet count of 96,000/uL of blood in this study. These patients have been exposed to fostamatinib for a median of 13 months through the combined parent and the long-term open-label extension study.

In addition, there were 43 placebo non-responders that enrolled in the long-term open-label extension study. 36 of these patients had at least 12 weeks of follow-up. Of those, 6 patients (17%) have achieved a prospectively defined stable platelet response, which is statistically significant and similar to the response rate fostamatinib achieved in the parent studies..

The most frequent adverse events were gastrointestinal-related, and the safety profile of the product was consistent with prior clinical experience, with no new or unusual safety issues uncovered. Data from both Phase 3 studies as well as our ongoing open-label extension study demonstrates the consistent benefit of fostamatinib in ITP. We plan to meet with the FDA shortly to discuss the above results and our potential New Drug Application (NDA) submission.

### Fostamatinib—IgAN

**Disease background.** IgAN is an autoimmune disease that severely affects the functioning of the kidneys. An estimated 12,000 Americans are diagnosed with this type of glomerulonephritis each year, with 25% of its victims eventually requiring dialysis and/or kidney transplantation over time. IgAN is characterized by the deposition of IgA immune complexes in the glomeruli of the kidneys leading to an inflammatory response and subsequent tissue damage that ultimately disrupts the normal filtering function of the kidneys. By inhibiting SYK in kidney cells, fostamatinib may block the signaling of IgA immune complex receptors and arrest or slow destruction of the glomeruli.

**Orally-available SYK inhibitor program.** Our Phase 2 clinical trial in patients with IgAN, called SIGN (SYK Inhibition for Glomerulonephritis) completed enrollment for the first cohort and is currently enrolling patients for the second cohort. We expect to report top line results for the first cohort by the end of 2016.

### Fostamatinib—AIHA

**Disease background.** AIHA is a rare, serious blood disorder where the immune system produces antibodies that result in the destruction of the body's own red blood cells. Symptoms can include fatigue, shortness of breath, rapid heartbeat, jaundice or enlarged spleen. While no medical treatments are currently approved for AIHA, physicians generally treat acute and chronic cases of the disorder with corticosteroids, other immuno-suppressants, or splenectomy. Research has shown that inhibiting SYK with fostamatinib may reduce the destruction of red blood cells. This disorder affects an estimated 40,000 Americans, for whom no approved treatment options currently exist.

Orally available SYK inhibitor program. We initiated a Phase 2 clinical trial in patients with AIHA in February 2016. The trial is an open-label, multi-center, two-stage study that will evaluate the efficacy and safety of fostamatinib in patients with warm antibody AIHA who have previously received treatment for the disorder, but have relapsed. Stage 1 will enroll 17 patients who will receive 150 mg of fostamatinib orally twice a day for a period of 12 weeks. The patients will return to the clinic every two weeks for blood draws and medical assessment. The primary efficacy endpoint of this study is to achieve increased hemoglobin levels by week 12 of greater than 10 g/dL, and greater than or equal to 2 g/dL higher than baseline. Stage 2 will include an additional 20 patients who will receive the same treatment protocol as Stage 1. We expect to have results of the Stage 1 segment of the trial in 2017. With this data, we will evaluate the best way forward and potentially an expedited path for pursuing AIHA.

#### R348—Dry Eye in Patients with Ocular Graft-Versus-Host Disease (GvHD)

Disease background. According to an article published by the American Academy of Ophthalmology, a significant number (22% to 80%) of patients with acute or chronic GvHD develop a secondary incidence of dry eye (keratoconjunctivitis sicca). In general, these patients are severely ill and have a great medical need for a topical therapy that may better manage their symptoms.

Topical Ophthalmic JAK/SYK inhibitor program. We recently completed our Phase 2 study of patients with ocular GvHD to determine if R348, an ophthalmic JAK/SYK inhibitor, reduces inflammation and limits the damage to

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the eye tissue caused by the disease. The study was comprised of 30 patients that were randomized to receive either R348 at 0.2%, 0.5% or a placebo eye drops for 12 weeks. The primary endpoint was a mean change from baseline at 12 weeks in total fluorescein staining score (CFS) of all corneal regions, a measure of corneal epithelial damage. In the “per protocol” population which is the pre-specified efficacy analysis population, the mean change from baseline at 12 weeks in CFS scores was -2.11 for the placebo group, -4.14 in the R348 0.2% group ( $p=0.186$  versus placebo group), and -6.00 in the R348 0.5% group ( $p=0.053$  versus placebo, using nonparametric testing). As such, the primary endpoint was not met based on the results of the study. R348 was well tolerated by patients at both doses. In light of the overall findings, we have decided not to pursue this program.

## Research/Preclinical Programs

We are conducting proprietary research in the broad disease areas of inflammation/immunology, immuno-oncology, cancers and muscle wasting/muscle endurance. Within each disease area, our researchers are investigating mechanisms of action as well as screening compounds against potential novel targets and optimizing those leads that appear to have the greatest potential.

We are conducting preclinical studies to identify a lead molecule for our IRAK program. Inhibitors of IRAK activity represent valuable therapeutic tools to treat cytokine-driven autoimmune and inflammatory diseases. This program may provide opportunities in both the oncology and immunology areas. We expect to file an Investigational New Drug (IND) with our molecule in 2017.

## Sponsored Research and License Agreements

We conduct research and development programs independently and in connection with our corporate collaborators. We are a party to a collaboration agreement with BMS for the discovery, development and commercialization of cancer immunotherapies based on our small molecule TGF beta receptor kinase inhibitors, as discussed below. Our participation in the collaboration is limited to the Joint Research Committee and the performance of research activities based on billable full-time equivalent fees as specified in the collaboration agreement. We do not have ongoing participation obligations under our agreements with Aclaris for the development and commercialization of certain JAK inhibitors for the treatment of alopecia areata and other dermatological conditions, AZ for the development and commercialization of R256, an inhaled JAK inhibitor, BerGenBio for the development and commercialization of an oncology program, and Daiichi to pursue research related to a specific target from a novel class of drug targets called ligases. Under these agreements, which we entered into in the ordinary course of business, we received or may be entitled to receive upfront cash payments, progress dependent contingent payments on events achieved by such partners and royalties on any net sales of products sold by such partners under the agreements. Total future contingent payments to us under all of these current agreements could exceed \$531.9 million if all potential product candidates achieved all of the payment triggering events under all of our current agreements (based on a single product candidate under each agreement). Of this amount, up to \$148.8 million relates to the achievement of development events, up to \$345.6 million relates to the achievement of regulatory events and up to \$37.5 million relates to the achievement of

certain commercial or launch events. This estimated future contingent amount does not include any estimated royalties that could be due to us if the partners successfully commercialize any of the licensed products. Future events that may trigger payments to us under the agreements are based solely on our partners' future efforts and achievements of specified development, regulatory and/or commercial events. Because we do not control the research, development or commercialization of the product candidates generated under these agreements, we are not able to reasonably estimate when, if at all, any contingent payments would become payable to us. As such, the contingent payments we could receive thereunder involve a substantial degree of risk to achieve and may never be received in the next 12 months or thereafter. Accordingly, we do not expect, and investors should not assume, that we will receive all of the potential contingent payments provided for under these agreements and it is possible that we may never receive any additional significant contingent payments or royalties under these agreements.

In October 2015, we entered into a non-exclusive license agreement with a third party, pursuant to which we received a payment in the single-digit millions in exchange for granting a non-exclusive license to certain limited intellectual property rights. We concluded that the granting of the license, which was fully delivered to such third party

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in the fourth quarter of 2015, represents the sole deliverable under this agreement. Accordingly, we recognized the payment as revenue during the fourth quarter of 2015.

In August 2015, we entered into a license agreement with Aclaris, pursuant to which Aclaris will have exclusive rights and will assume responsibility for the continued development of certain JAK inhibitor compounds for the treatment of alopecia areata and other dermatological conditions. Under the license agreement, we received a noncreditable and non-refundable upfront payment of \$8.0 million in September 2015. We are also entitled to receive development and regulatory contingent fees that could exceed \$80.0 million for a successful compound approved in certain indications. In addition, we are also eligible to receive tiered royalties on the net sales of any products under the agreement. We concluded that the granting of the license, which has been fully delivered to Aclaris in the third quarter of 2015, represents the sole deliverable under this agreement. Accordingly, we recognized the \$8.0 million payment as revenue during the third quarter of 2015.

In February 2015, we entered into a collaboration agreement with BMS for the discovery, development and commercialization of cancer immunotherapies based on our extensive portfolio of small molecule TGF beta receptor kinase inhibitors. Under the collaboration agreement, BMS will have exclusive rights and will be solely responsible for the clinical development and commercialization of any products. Pursuant to the collaboration agreement with BMS, we received a noncreditable and non-refundable upfront payment of \$30.0 million in March 2015. We are also entitled to receive development and regulatory contingent fees that could exceed \$309.0 million for a successful compound approved in certain indications. In addition, we are also eligible to receive tiered royalties on the net sales of any products from the collaboration. BMS shall also reimburse us for agreed upon costs based on a contractual cost per full-time equivalent employee in connection with the performance of research activities during the research term. Under the collaboration agreement, we were obligated to provide the following deliverables: (i) granting of license rights to our program, (ii) participation in the Joint Research Committee, and (iii) performance of research activities. We concluded that these deliverables are a single unit of accounting as the license does not have stand-alone value apart from the other deliverables. Accordingly, the \$30.0 million upfront payment was recognized ratably as revenue from the effective date of the agreement through September 2016, the end of the research term. We believe that straight-line recognition of this revenue was appropriate as the research was performed ratably over the research period. At the end of the initial research term, we were not notified by BMS of its intention to extend the initial research term under which we would perform research activities. However, BMS does continue to evaluate compounds from the extensive portfolio under the agreement, on its own, for designation of a compound as an early candidate nomination. During the three and nine months ended September 30, 2016, we recognized revenue of \$3.8 million and \$13.4 million, respectively, relating to the upfront payment. During the nine months ended September 30, 2016, we recognized revenue of \$290,000 relating to the research activities we performed. During the three and nine months ended September 30, 2015, we recognized revenue of \$4.8 million and \$11.7 million, respectively, relating to the upfront payment and \$163,000 and \$619,000, respectively, relating to the research activities we performed. We do not have any deferred revenue as of September 30, 2016.

In June 2011, we entered into an exclusive license agreement with BerGenBio for the development and commercialization of an oncology program. BerGenBio is responsible for all activities it wishes to perform under the license we granted to it. In June 2016, we received contingent payments of \$1.7 million relating to a time-based non-refundable fee and \$2.0 million relating to BerGenBio's exercise of certain option rights before the prescription period to exercise the rights expired. All deliverables under the agreement had been previously delivered, as such, the

above payments of \$3.7 million triggered by the above time-based and contingent events were recognized as revenue in the second quarter of 2016.

#### Research and Development Expenses

Our research and development expenditures include costs related to preclinical and clinical trials, scientific personnel, supplies, equipment, consultants, sponsored research, stock based compensation, and allocated facility costs.

We do not track fully burdened research and development costs separately for each of our drug candidates. We review our research and development expenses by focusing on three categories: research, development, and other. Our research team is focused on creating a portfolio of product candidates that can be developed into small molecule therapeutics in our own proprietary programs or with potential collaborative partners and utilizes our robust discovery



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engine to rapidly discover and validate new product candidates in our focused range of therapeutic indications. “Research” expenses relate primarily to personnel expenses, lab supplies, fees to third party research consultants and compounds. Our development group leads the implementation of our clinical and regulatory strategies and prioritizes disease indications in which our compounds may be studied in clinical trials. “Development” expenses relate primarily to clinical trials, personnel expenses, lab supplies and fees to third party research consultants. “Other” expenses primarily consist of allocated facilities costs and allocated stock based compensation expense relating to personnel in research and development groups.

In addition to reviewing the three categories of research and development expenses described in the preceding paragraph, we principally consider qualitative factors in making decisions regarding our research and development programs, which include enrollment in clinical trials and the results thereof, the clinical and commercial potential for our drug candidates and competitive dynamics. We also make our research and development decisions in the context of our overall business strategy, which includes the evaluation of potential collaborations for the development of our drug candidates.

The following table presents our total research and development expense by category (in thousands).

	Three Months Ended September 30,		Nine Months Ended September 30,		From January 1, 2007*
	2016	2015	2016	2015	to September 30, 2016
Categories:					
Research	\$ 4,977	\$ 5,050	\$ 17,391	\$ 15,853	\$ 213,889
Development	8,253	6,865	24,323	19,022	307,606
Other	2,941	3,586	10,098	11,387	219,463
	\$ 16,171	\$ 15,501	\$ 51,812	\$ 46,262	\$ 740,958

\*We started tracking research and development expense by category on January 1, 2007.

“Other” expenses mainly represent allocated facilities costs of approximately \$2.3 million and \$2.6 million for the three months ended September 30, 2016 and 2015, respectively, and allocated stock-based compensation expenses of approximately \$643,000 and \$966,000 for the three months ended September 30, 2016 and 2015, respectively. For the nine months ended September 30, 2016 and 2015, allocated facilities costs were approximately 7.4 million and \$8.2 million, respectively, and allocated stock-based compensation expenses were approximately \$2.7 million and \$3.2 million, respectively.

For the three and nine months ended September 30, 2016 and 2015, a major portion of our total research and development expense was associated with salaries of our research and development personnel, our ITP, AIHA and

IgAN programs, and allocated facilities costs. For the three and nine months ended September 30, 2015, a major portion of our total research and development expense was associated with our ITP and IgAN programs, salaries of our research and development personnel, and allocated facilities costs.

We do not have reliable estimates regarding the timing of our clinical trials. Preclinical testing and clinical development are long, expensive and uncertain processes. In general, biopharmaceutical development involves a series of steps, beginning with identification of a potential target and including, among others, proof of concept in animals and Phase 1, 2 and 3 clinical trials in humans. Significant delays in clinical testing could materially impact our product development costs and timing of completion of the clinical trials. We do not know whether planned clinical trials will begin on time, will need to be halted or revamped or will be completed on schedule, or at all. Clinical trials can be delayed for a variety of reasons, including delays in obtaining regulatory approval to commence a trial, delays from scale up, delays in reaching agreement on acceptable clinical trial agreement terms with prospective clinical sites, delays in obtaining institutional review board approval to conduct a clinical trial at a prospective clinical site or delays in recruiting subjects to participate in a clinical trial.

We currently do not have reliable estimates of total costs for a particular drug candidate to reach the market. Our potential products are subject to a lengthy and uncertain regulatory process that may involve unanticipated

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additional clinical trials and may not result in receipt of the necessary regulatory approvals. Failure to receive the necessary regulatory approvals would prevent us from commercializing the product candidates affected. In addition, clinical trials of our potential products may fail to demonstrate safety and efficacy, which could prevent or significantly delay regulatory approval.

For further discussion on research and development activities, see “Research and Development Expense” under “Results of Operations” below.

## Results of Operations

## Three and Nine Months Ended September 30, 2016 and 2015

## Revenues

	Three Months Ended September 30,		Aggregate Change	Nine Months Ended September 30,		Aggregate Change
	2016	2015 (in thousands)		2016	2015 (in thousands)	
Contract revenues from collaborations	\$ 3,760	\$ 12,996	\$ (9,236)	\$ 17,383	\$ 20,358	\$ (2,975)

Contract revenues from collaborations of \$3.8 million during the three months ended September 30, 2016 represent the remaining amortization of the \$30.0 million upfront payment we earned from BMS. Contract revenues from collaborations of \$17.4 million during the nine months ended September 30, 2016 were comprised of the amortization of the upfront payment and FTE fees we earned from BMS of \$13.4 million and \$290,000, respectively, as well as the contingent payments amounting to \$3.7 million we received from BerGenBio. Contract revenues from collaborations of \$13.0 million in the three months ended September 30, 2015 were comprised of the \$8.0 million upfront payment received from Aclaris in September 2015, the amortization of the \$30.0 million upfront payment from BMS of \$4.8 million and the FTE fees we earned of \$163,000 from BMS. Contract revenues from collaborations of \$20.4 million in the nine months ended September 30, 2015 were comprised of the \$8.0 million upfront payment received from Aclaris in September 2015, the amortization of the \$30.0 million upfront payment from BMS of \$11.7 million and the FTE fees we earned from BMS of \$619,000.

We do not have any deferred revenue as of September 30, 2016. Our potential future revenues may include payments from our current partners and from new partners with whom we enter into agreements in the future, if any, the timing and amount of which is unknown at this time.

#### Research and Development Expense

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2016	2015	Aggregate Change	2016	2015	Aggregate Change
	(in thousands)			(in thousands)		
Research and development expense	\$ 16,171	\$ 15,501	\$ 670	\$ 51,812	\$ 46,262	\$ 5,550
Stock-based compensation expense included in research and development expense	\$ 643	\$ 966	\$ (323)	\$ 2,746	\$ 3,182	\$ (436)

The increases in research and development expense for the three and nine months ended September 30, 2016, compared to the same periods in 2015, were primarily due to the increases in research and development costs related to our fostamatinib in ITP, AIHA and IgAN programs, partially offset by the decreases in personnel costs, and stock based compensation due to the reduction in workforce in September 2016.

We expect that our research and development expense in the fourth quarter of 2016 and in 2017 to decrease relative to the prior periods due to our recently announced restructuring and as we complete the randomized portion of our Phase 3 clinical trials in ITP in 2016.

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## General and Administrative Expense

	Three Months Ended			Nine Months Ended		
	September 30, 2016	2015 (in thousands)	Aggregate Change	September 30, 2016	2015 (in thousands)	Aggregate Change
General and administrative expense	\$ 4,558	\$ 4,276	\$ 282	\$ 13,755	\$ 13,092	\$ 663
Stock-based compensation expense included in general and administrative expense	\$ 572	\$ 849	\$ (277)	\$ 1,921	\$ 2,596	\$ (675)

The increases in general and administrative expense for the three and nine months ended September 30, 2016, compared to the same periods in 2015, was primarily due to the commercial launch costs incurred in anticipation of a potential commercial launch of fostamatinib in ITP, partially offset by the decrease in stock-based compensation expense.

## Restructuring Charges

	Three Months Ended			Nine Months Ended		
	September 30, 2016	2015 (in thousands)	Aggregate Change	September 30, 2016	2015 (in thousands)	Aggregate Change
Restructuring charges	\$ 5,770	\$ —	\$ 5,770	\$ 5,770	\$ —	\$ 5,770
Stock-based compensation expense included in restructuring charges	\$ 499	\$ —	\$ 499	\$ 499	\$ —	\$ 499

In September 2016, we announced that we had reduced our workforce by 46 positions, mostly in the research area. We also announced that effective September 15, 2016, Donald G. Payan, M.D, has retired from the board of directors and from his position as Executive Vice President and President of Discovery and Research. We recorded restructuring charges during the three months ended September 30, 2016 of approximately \$5.8 million within Restructuring Charges in the accompanying Statement of Operations, which included \$5.0 million of severance costs paid or to be paid in cash, \$319,000 impairment of certain property and equipment, and \$499,000 of non-cash stock-based compensation expense as a result of the modification of our former executive's stock options. At September 30, 2016, the remaining accrued restructuring costs, which will substantially be paid in the fourth quarter of 2016, consisted of \$1.4 million related to severance, COBRA benefits and outplacement costs for certain employees that were affected by the reduction in workforce. This accrued liability is classified under Accrued Compensation on the Condensed Balance Sheet.

## Interest Income

	Three Months Ended		Aggregate Change	Nine Months Ended		Aggregate Change
	September 30, 2016	2015 (in thousands)		September 30, 2016	2015 (in thousands)	
Interest income	\$ 110	\$ 54	\$ 56	\$ 328	\$ 162	\$ 166

Interest income results from our interest-bearing cash and investment balances. The increase in interest income for the three months and nine months ended September 30, 2016, as compared to the same periods in 2015 was primarily due to the higher yield on our investments.

## Critical Accounting Policies and the Use of Estimates

Our discussion and analysis of our financial condition and results of operations is based upon our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles (U.S. GAAP). The preparation of these financial statements requires us to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. We evaluate our estimates, including those related to our

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stock based compensation and the probability of achievement of corporate performance-based milestone for our performance-based stock option awards, impairment issues, the estimated useful life of assets, and estimated accruals, particularly research and development accruals, on an ongoing basis. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that there have been no significant changes in our critical accounting policies and estimates disclosed in our Annual Report on Form 10-K for the year ended December 31, 2015, as filed with the SEC.

## Recent Accounting Pronouncements

In August 2014, the FASB issued ASU No. 2014-15—Disclosure of Uncertainties about an Entity’s Ability to Continue as a Going Concern under ASC Subtopic 205-40, Presentation of Financial Statements—Going Concern. ASU No. 2014-15 provides guidance about management’s responsibility to evaluate whether there is substantial doubt about an entity’s ability to continue as a going concern and to provide related footnote disclosures. Management’s evaluation should be based on relevant conditions and events that are known and reasonably knowable at the date that the financial statements are issued (or at the date that the financial statements are available to be issued when applicable). Substantial doubt about an entity’s ability to continue as a going concern exists when relevant conditions and events, considered in the aggregate, indicate that it is probable that the entity will be unable to meet its obligations as they become due within one year after the date that the financial statements are issued (or available to be issued). ASU No. 2014-15 is effective for the annual period ending after December 15, 2016 and early adoption is permitted. We plan to adopt this new standard in our annual financial statements for the year ending December 31, 2016. We will continue to evaluate the guidance under ASU No. 2014-15 and present the required disclosures within our financial statements at the time of adoption. The actual impact will be dependent upon our liquidity and the nature or significance of future events or conditions that exist upon the adoption of this new standard.

In May 2014, the FASB issued ASU No. 2014-09—Revenue from Contracts with Customers, which supersedes the revenue recognition requirements under ASC Topic 605, Revenue Recognition, and most industry-specific guidance under the ASC. The core principle of ASU No. 2014-09 is that an entity should recognize revenue when it transfers promised goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. ASU No. 2014-09 defines a five step process to achieve this core principle and, in doing so, it is possible more judgment and estimates may be required within the revenue recognition process than required under existing U.S. GAAP including identifying performance obligations in the contract, estimating the amount of variable consideration to include in the transaction price and allocating the transaction price to each separate performance obligation. ASU No. 2014-09 also requires additional disclosures to enable users of financial statements to understand the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts. ASU No. 2014-09 allows for either full retrospective or modified retrospective adoption, and we have not yet determined which approach we will apply. In July 2015, the FASB deferred by one year the effective date of ASU No. 2014-09 with the new effective date beginning after December 15, 2017, and the interim periods within that year and will allow early adoption for all entities as of the original effective date for public business entities, which was annual reporting periods beginning after December 15, 2016. We plan to adopt this new standard on January 1, 2018 using the modified retrospective approach. We are currently evaluating the potential impact of the adoption of ASU No. 2014-09 on our financial statements and cannot estimate the impact of adoption at this time.

In February 2016, the FASB issued ASU No. 2016-02—Leases, which is aimed at making leasing activities more transparent, and requires substantially all leases be recognized by lessees on their balance sheet as a right-of-use asset and corresponding lease liability, including leases currently accounted for as operating leases. The guidance is effective for all interim and annual reporting periods beginning after December 15, 2018. Early adoption is permitted. We plan to adopt this new standard on January 1, 2019. We are currently evaluating the potential impact of the adoption of ASU No. 2016-02 on our financial statements and cannot estimate the impact of adoption at this time.

In March 2016, the FASB issued ASU No. 2016-09—Improvements to Employee Share-Based Payment Accounting, which is intended to simplify several aspects of the accounting for share-based payment award transactions, including the income tax consequences, an option to recognize gross stock compensation expense with actual forfeitures



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recognized as they occur, as well as certain classifications on the statement of cash flows. The guidance will be effective for the fiscal year beginning after December 15, 2016, including interim periods within that year. We plan to adopt this new standard on January 1, 2017 and do not expect a material impact on our financial statements.

Liquidity and Capital Resources

Cash Requirements

From inception, we have financed our operations primarily through sales of equity securities, contract payments under our collaboration agreements and equipment financing arrangements. We have consumed substantial amounts of capital to date as we continue our research and development activities, including preclinical studies and clinical trials.

As of September 30, 2016, we had approximately \$85.3 million in cash, cash equivalents and short term investments, as compared to approximately \$126.3 million as of December 31, 2015, a decrease of approximately \$41.0 million. The decrease was primarily attributable to the payments associated with funding our operating expenses for the nine months ended September 30, 2016. In June 2016, we received payments from BerGenBio amounting to \$3.7 million pursuant to our exclusive license agreement which we signed in June 2011. In August 2015, we entered into Controlled Equity OfferingSM Sales Agreement with Cantor, as sales agent, pursuant to which we may sell, through Cantor, up to an aggregate of \$30.0 million in shares of our common stock. During the nine months ended September 30, 2016, 7,895,563 shares of common stock were sold under the Sales Agreement, with an aggregate proceeds of \$24.3 million. In December 2014, we entered into a sublease agreement with an unrelated third party to occupy a portion of our research and office space. We expect to receive approximately \$3.7 million in future sublease income (excluding our subtenant's share of facility's operating expenses) over the remaining term of the sublease through January 2018. During the nine months ended September 30, 2016, we received approximately \$3.4 million of sublease income and reimbursements. We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including funds to continue efforts in preparation of the potential commercial launch of fostamatinib in ITP in the U.S., through the end of 2017. We also continue to evaluate ex-U.S. partnerships for fostamatinib and other partnering opportunities across our pipelines. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development of our product candidates and other research and development activities, including risks and uncertainties that could impact the rate of progress of our development activities, we are unable to estimate with certainty the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials and other research and development activities.

Our operations will require significant additional funding for the foreseeable future. Unless and until we are able to generate a sufficient amount of product, royalty or milestone revenue, we expect to finance future cash needs through public and/or private offerings of equity securities, debt financings and/or collaboration and licensing arrangements, and to a much lesser extent through interest income earned on the investment of our excess cash balances and short term investments. With the exception of contingent and royalty payments that we may receive under our existing

collaborations, we do not currently have any committed future funding. To the extent we raise additional capital by issuing equity securities, our stockholders could at that time experience substantial dilution. Any debt financing that we are able to obtain may involve operating covenants that restrict our business. To the extent that we raise additional funds through collaboration and licensing arrangements, we may be required to relinquish some of our rights to our technologies or product candidates, or grant licenses on terms that are not favorable to us.

Our future funding requirements will depend upon many factors, including, but not limited to:

- the outcome of our meeting with the FDA to discuss the results of our Phase 3 studies of fostamatinib in ITP;
- the progress and success of our clinical trials and preclinical activities (including studies and manufacture of materials) of our product candidates conducted by us;
- the progress of research and development programs carried out by us and our collaborative partners;

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- the costs and timing of regulatory filings and approvals by us and our collaborators;
- the costs to build and expand our sales, marketing and distribution capabilities;
- the costs to commercialize fostamatinib or any other future product candidates, if any such candidate receives regulatory approval for commercial sale;
- any changes in the breadth of our research and development programs;
- the ability to achieve the events identified in our collaborative agreements that may trigger payments to us from our collaboration partners;
- our ability to acquire or license other technologies or compounds that we may seek to pursue;
- our ability to manage our growth;
- competing technological and market developments;
- the costs and timing of obtaining, enforcing and defending our patent and other intellectual property rights; and
- expenses associated with any unforeseen litigation, including any securities class action lawsuits.

Insufficient funds may require us to delay, scale back or eliminate some or all of our research or development programs, to lose rights under existing licenses or to relinquish greater or all rights to product candidates at an earlier stage of development or on less favorable terms than we would otherwise choose or may adversely affect our ability to operate as a going concern.

For the nine months ended September 30, 2016 and 2015, we maintained an investment portfolio primarily in money market funds, U. S. treasury bills, government sponsored enterprise securities, and corporate bonds and commercial paper. Cash in excess of immediate requirements is invested with regard to liquidity and capital preservation. Wherever possible, we seek to minimize the potential effects of concentration and degrees of risk. We will continue to monitor the impact of the changes in the conditions of the credit and financial markets to our investment portfolio and assess if future changes in our investment strategy are necessary.

## Cash Flows from Operating, Investing and Financing Activities

	Nine Months Ended September 30,	
	2016	2015
	(in thousands)	
Net cash provided by (used in):		
Operating activities	\$ (63,425)	\$ (9,616)
Investing activities	22,770	31,863
Financing activities	23,149	1,127
Net increase (decrease) in cash and cash equivalents	\$ (17,506)	\$ 23,374

Net cash used in operating activities was approximately \$63.4 million for the nine months ended September 30, 2016, compared to approximately \$9.6 million for the nine months ended September 30, 2015. Net cash used in operating activities for the nine months ended September 30, 2016 was primarily due to the cash payments related to our research and development programs and severance payments as a result of the reduction in workforce in September 2016, partially offset by the \$3.7 million payments we received from BerGenBio. Net cash used in operating activities for the nine months ended September 30, 2015 was primarily due to the cash payments related to our research and development programs, partially offset by the \$8.0 million and \$30.0 million upfront payments we received from Aclaris and BMS, respectively. The timing of cash requirements may vary from period to period depending on our research and

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development activities, including our planned preclinical and clinical trials, and future requirements to establish commercial capabilities for any products that we may develop.

Net cash provided by investing activities was approximately \$22.8 million for the nine months ended September 30, 2016, compared to approximately \$31.9 million for the nine months ended September 30, 2015. Net cash provided by investing activities in each period related to net maturities of short-term investments, partially offset by capital expenditures. Capital expenditures were approximately \$798,000 for the nine months ended September 30, 2016, compared to approximately \$412,000 for the same period in 2015.

Net cash provided by financing activities was approximately \$23.1 million for the nine months ended September 30, 2016, compared to approximately \$1.1 million for the nine months ended September 30, 2015. Net cash provided by financing activities for the nine months ended September 30, 2016 consisted of net proceeds of \$22.2 million from issuance of shares under our Sales Agreement as well as proceeds from exercise of outstanding options and issuance of shares under the Purchase Plan. Net cash provided by financing activities for the nine months ended September 30, 2015 was primarily due to the proceeds from the issuance of shares under our Purchase Plan.

## Off-Balance Sheet Arrangements

As of September 30, 2016, we had no off-balance sheet arrangements (as defined in Item 303(a)(4)(ii) of Regulation S-K under the Exchange Act).

## Contractual Obligations

We conduct our research and development programs internally and through third parties that include, among others, arrangements with universities, consultants and contract research organizations. We have contractual arrangements with these parties, however our contracts with them are cancelable generally on reasonable notice within one year and our obligations under these contracts are primarily based on services performed. We do not have any purchase commitments under any collaboration arrangements.

As of September 30, 2016, we had the following contractual commitments:

	Total	Less than 1 Year	Payment Due By Period 1 - 3 Years	3 - 5 Years	More than 5 Years
Facilities lease(1)	\$ 21,399	\$ 15,997	\$ 5,402	\$ —	\$ —

(1) In December 2014, we entered into a sublease agreement with an unrelated third party to lease up a portion of the research and office space. The facilities lease obligations above do not include the sublease income of \$3.7 million over the remaining term of the sublease.

### Item 3. Quantitative and Qualitative Disclosures About Market Risk

During the nine months ended September 30, 2016, there were no material changes to our market risk disclosures as set forth in Part II, Item 7A, "Quantitative and Qualitative Disclosures About Market Risk," of our Annual Report on Form 10-K for the year ended December 31, 2015.

### Item 4. Controls and Procedures

**Evaluation of Disclosure Controls and Procedures.** Based on the evaluation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act), our chief executive officer and chief financial officer have concluded that, as of the end of the period covered by this report, our disclosure controls and procedures were effective.

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Changes in Internal Controls. There were no changes in our internal control over financial reporting that occurred during the quarter ended September 30, 2016 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls. A control system, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the controls are met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues, if any, within a company have been detected. Accordingly, our disclosure controls and procedures are designed to provide reasonable, not absolute, assurance that the objectives of our disclosure control system are met and, as set forth above, our chief executive officer and chief financial officer have concluded, based on their evaluation as of the end of the period covered by this report, that our disclosure controls and procedures were sufficiently effective to provide reasonable assurance that the objectives of our disclosure control system were met.

PART II. OTHER INFORMATION

Item 1. Legal Proceedings

None.

Item 1A. Risk Factors

In evaluating our business, you should carefully consider the following risks, as well as the other information contained in this Quarterly Report on Form 10-Q. These risk factors could cause our actual results to differ materially from those contained in forward-looking statements we have made in this Quarterly Report on Form 10-Q and those we may make from time to time. If any of the following risks actually occurs, our business, financial condition and operating results could be harmed. The risks and uncertainties described below are not the only ones facing us. Additional risks and uncertainties not presently known to us, or that we currently see as immaterial, may also harm our business.

We have marked with an asterisk (\*) those risk factors below that reflect a substantive change from the risk factors included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 8, 2016.

If we are unable to submit the NDA for fostamatinib in ITP or if we submit the NDA but it is not accepted or not approved for ITP by the FDA, this would have a material adverse effect on our business, financial performance and results of operations.\*

In August 2016, we announced the results of the first Phase 3 study of fostamatinib in ITP, reporting that fostamatinib met its primary efficacy endpoint. The study showed that 18% of patients receiving fostamatinib achieved a stable platelet response compared to none receiving a placebo control. In October 2016, we announced the results of the second study, reporting that the fostamatinib response rate was 18% consistent with the first study. However, one patient in the placebo group achieved a stable platelet response, therefore the difference between groups did not reach statistical significance. Given that one of the two studies did not meet its primary endpoint, there is a risk that the FDA will not agree that we should move forward with submitting the NDA, or, that the FDA will not accept the submission, or that the submission is accepted but not approved, which would have a material adverse effect on our ability to generate revenue from the sales of fostamatinib in ITP. An inability to generate such revenue would have a material adverse effect on our business, financial performance and results of operations. In addition, the FDA may suggest that we need to conduct additional trials at significant costs before we submit applications seeking regulatory approval of our product candidates. Any such requirement for additional trials would most likely result in our inability to commercialize fostamatinib in the United States for a significant period of time, which would have a material adverse effect on our ability to generate revenue from the sales of fostamatinib in ITP.



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We will need additional capital in the future to sufficiently fund our operations and research.\*

We have consumed substantial amounts of capital to date as we continue our research and development activities, including preclinical studies and clinical trials. We may seek another collaborator or licensee in the future for further clinical development and commercialization of fostamatinib, as well as our other clinical programs, which we may not be able to obtain on commercially reasonable terms or at all. We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including funds to continue efforts in preparation of the potential commercial launch of fostamatinib in ITP in the U.S., through the end of 2017. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development of our product candidates and other research and development activities, including risks and uncertainties that could impact the rate of progress of our development activities, we are unable to estimate with certainty the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials and other research and development activities. We will continue to need additional capital and the amount of future capital needed will depend largely on the success of our internally developed programs as they proceed in later and more expensive clinical trials, including any additional clinical trials that we may decide to conduct with respect to fostamatinib. Unless and until we are able to generate a sufficient amount of product, royalty or milestone revenue, which may never occur, we expect to finance future cash needs through public and/or private offerings of equity securities, debt financings or collaboration and licensing arrangements, as well as through interest income earned on the investment of our cash balances and short-term investments. With the exception of contingent and royalty payments that we may receive under our existing collaborations, we do not currently have any commitments for future funding. We do not know whether additional financing will be available when needed, or that, if available, we will obtain financing on reasonable terms.

To the extent we raise additional capital by issuing equity securities in the future, our stockholders could at that time experience substantial dilution. Any debt financing that we are able to obtain may involve operating covenants that restrict our business. To the extent that we raise additional funds through any new collaboration and licensing arrangements, we may be required to relinquish some rights to our technologies or product candidates, or grant licenses on terms that are not favorable to us.

Our future funding requirements will depend on many uncertain factors.\*

Our future funding requirements will depend upon many factors, many of which are beyond our control, including, but not limited to:

- the outcome of our meeting with the FDA to discuss the results of our Phase 3 studies of fostamatinib in ITP;

the progress and success of our clinical trials and preclinical activities (including studies and manufacture of materials) of our product candidates conducted by us;

- the progress of research and development programs carried out by us and our collaborative partners;
- the costs and timing of regulatory filings and approvals by us and our collaborators;
- the costs to build and expand our sales, marketing and distribution capabilities;
- the costs to commercialize fostamatinib or any other future product candidates, if any such candidate receives regulatory approval for commercial sale;
- any changes in the breadth of our research and development programs;
- the ability to achieve the events identified in our collaborative agreements that may trigger payments to us from our collaboration partners;

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- our ability to acquire or license other technologies or compounds that we may seek to pursue;
- our ability to manage our growth;
- competing technological and market developments;
- the costs and timing of obtaining, enforcing and defending our patent and other intellectual property rights; and
- expenses associated with any unforeseen litigation, including any securities class action lawsuits.

Insufficient funds may require us to delay, scale back or eliminate some or all of our research and development programs, to lose rights under existing licenses or to relinquish greater or all rights to product candidates at an earlier stage of development or on less favorable terms than we would otherwise choose or may adversely affect our ability to operate as a going concern.

There is a high risk that drug discovery and development efforts might not generate successful product candidates.\*

At the present time, the majority of our operations are focused on various stages of drug identification and development. We currently have various product candidates in the clinical testing stage. In our industry, it is statistically unlikely that the limited number of compounds that we have identified as potential product candidates will actually lead to successful product development efforts. We have invested a significant portion of our efforts and financial resources into the development of fostamatinib. Our ability to generate product revenue, which will not occur until after regulatory approval, if ever, will depend on the successful development, regulatory approval and eventual commercialization of one of our product candidates.

Our compounds in clinical trials and our future leads for potential drug compounds are subject to the risks and failures inherent in the development of pharmaceutical products. These risks include, but are not limited to, the inherent difficulty in selecting the right drug and drug target and avoiding unwanted side effects, as well as unanticipated problems relating to product development, testing, enrollment, obtaining regulatory approvals, maintaining regulatory compliance, manufacturing, competition and costs and expenses that may exceed current estimates. In future clinical trials, we or our partners may discover additional side effects and/or higher frequency of side effects than those observed in previously completed clinical trials. The results of preliminary and mid-stage clinical trials do not necessarily predict clinical or commercial success, and larger later-stage clinical trials may fail to confirm the results observed in the previous clinical trials. Similarly, a clinical trial may show that a product candidate is safe and effective for certain patient populations in a particular indication, but other clinical trials may fail to confirm those results in a subset of that population or in a different patient population, which may limit the potential market for that product candidate. With respect to our own compounds in development, we have established anticipated timelines with respect to the initiation of clinical trials based on existing knowledge of the compounds. However, we cannot

provide assurance that we will meet any of these timelines for clinical development. Additionally, the initial results of a completed earlier clinical trial of a product candidate do not necessarily predict final results and the results may not be repeated in later clinical trials.

Because of the uncertainty of whether the accumulated preclinical evidence (pharmacokinetic, pharmacodynamic, safety and/or other factors) or early clinical results will be observed in later clinical trials, we can make no assurances regarding the likely results from our future clinical trials or the impact of those results on our business. If our clinical trials fail to meet the primary efficacy endpoints, the commercial prospects of our business may be harmed, our ability to generate product revenues may be delayed or eliminated or we may be forced to undertake other strategic alternatives that are in our shareholders' best interests, including cost reduction measures. If we are unable to obtain adequate financing or engage in a strategic transaction on commercially reasonable terms or at all, we may be required to implement further cost reduction strategies which could significantly impact activities related to our research and development of our future product candidates, and could significantly harm our business, financial condition and results of operations. In addition, these cost reduction strategies could cause us to further curtail our operations or take other actions that would adversely impact our shareholders.

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We might not be able to commercialize our product candidates successfully if problems arise in the clinical testing and approval process.\*

Commercialization of our product candidates depends upon successful completion of extensive preclinical studies and clinical trials to demonstrate their safety and efficacy for humans. Preclinical testing and clinical development are long, expensive and uncertain processes.

In connection with clinical trials of our product candidates, we face the risks that:

- the product candidate may not prove to be effective;
- the product candidate may cause harmful side effects;
- the clinical results may not replicate the results of earlier, smaller trials;
  - we, or the FDA or similar foreign regulatory authorities, may terminate or suspend the trials;
- our results may not be statistically significant;
- patient recruitment and enrollment may be slower than expected;
  - patients may drop out of the trials;  
and
- regulatory and clinical trial requirements, interpretations or guidance may change.

We do not know whether we will be permitted to undertake clinical trials of potential products beyond the trials already concluded and the trials currently in process. It will take us, or our collaborative partners several years to complete any such testing, and failure can occur at any stage of testing. Interim results of trials do not necessarily predict final results, and acceptable results in early trials may not be repeated in later trials. A number of companies in the pharmaceutical industry, including biotechnology companies, have suffered significant setbacks in advanced clinical trials, even after achieving promising results in earlier trials. For example, in October 2016, we announced

that our second Phase 3 study in our FIT Phase 3 clinical program did not meet its primary endpoint. Moreover, we or our collaborative partners or regulators may decide to discontinue development of any or all of these projects at any time for commercial, scientific or other reasons. For example, in August 2014, we have discontinued our indirect AMPK activator program, R118, due to its side-effect profile in Phase 1 clinical trials.

We cannot assure you that we will be able to successfully complete the clinical development of our product candidates or receive regulatory approval to ultimately commercialize any of our other product candidates. For example, if we are unable to ultimately commercialize fostamatinib, our business will be harmed.

If we are unable to obtain regulatory approval to market products in the United States and foreign jurisdictions, we will not be permitted to commercialize products we or our collaborative partners may develop.

We cannot predict whether regulatory clearance will be obtained for any product that we, or our collaborative partners, hope to develop. Satisfaction of regulatory requirements typically takes many years, is dependent upon the type, complexity and novelty of the product and requires the expenditure of substantial resources. Of particular significance to us are the requirements relating to research and development and testing.

Before commencing clinical trials in humans in the United States, we, or our collaborative partners, will need to submit and receive approval from the FDA of an IND. Clinical trials are subject to oversight by institutional review boards and the FDA and:

- must be conducted in conformance with the FDA's good clinical practices and other applicable regulations;
- must meet requirements for institutional review board oversight;

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- must meet requirements for informed consent;
- are subject to continuing FDA and regulatory oversight;
- may require large numbers of test subjects; and
- may be suspended by us, our collaborators or the FDA at any time if it is believed that the subjects participating in these trials are being exposed to unacceptable health risks or if the FDA finds deficiencies in the IND or the conduct of these trials.

While we have stated that we intend to file additional INDs for future product candidates, this is only a statement of intent, and we may not be able to do so because we may not be able to identify potential product candidates. In addition, the FDA may not approve any IND we or our collaborative partners may submit in a timely manner, or at all.

Before receiving FDA approval to market a product, we must demonstrate with substantial clinical evidence that the product is safe and effective in the patient population and the indication that will be treated. Data obtained from preclinical and clinical activities are susceptible to varying interpretations that could delay, limit or prevent regulatory approvals. In addition, delays or rejections may be encountered based upon additional government regulation from future legislation or administrative action or changes in FDA policy during the period of product development, clinical trials and FDA regulatory review. Failure to comply with applicable FDA or other applicable regulatory requirements may result in criminal prosecution, civil penalties, recall or seizure of products, total or partial suspension of production or injunction, adverse publicity, as well as other regulatory action against our potential products or us. Additionally, we have limited experience in conducting and managing the clinical trials necessary to obtain regulatory approval.

If regulatory approval of a product is granted, this approval will be limited to those indications or disease states and conditions for which the product is demonstrated through clinical trials to be safe and efficacious. We cannot assure you that any compound developed by us, alone or with others, will prove to be safe and efficacious in clinical trials and will meet all of the applicable regulatory requirements needed to receive marketing approval.

Outside the United States, our ability, or that of our collaborative partners, to market a product is contingent upon receiving a marketing authorization from the appropriate regulatory authorities. This foreign regulatory approval process typically includes all of the risks and costs associated with FDA approval described above and may also include additional risks and costs, such as the risk that such foreign regulatory authorities, which often have different regulatory and clinical trial requirements, interpretations and guidance from the FDA, may require additional clinical trials or results for approval of a product candidate, any of which could result in delays, significant additional costs or failure to obtain such regulatory approval. For example, there can be no assurance that we or our collaborative

partners will not have to provide additional information or analysis, or conduct additional clinical trials, before receiving approval to market product candidates.

Even if our product candidates receive regulatory approval, they may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.\*

If any of our product candidates receive regulatory approval, they may nonetheless fail to gain sufficient market acceptance by physicians, hospital administrators, patients, healthcare payors and others in the medical community. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including the following:

- relative convenience and ease of administration;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the willingness of physicians to change their current treatment practices;



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- the willingness of hospitals and hospital systems to include our product candidates as treatment options;
- demonstration of efficacy and safety in clinical trials;
- the prevalence and severity of any side effects;
- the ability to offer product candidates for sale at competitive prices;
- the price we charge for our product candidates;
- the strength of marketing and distribution support; and
- the availability of third-party coverage or reimbursement.

If any of our product candidates are approved, if at all, but do not achieve an adequate level of acceptance, we may not generate significant product revenue and we may not become profitable on a sustained basis.

We are in the initial stages of developing our sales, marketing and distribution capabilities. If we are unable to develop effective sales, marketing and distribution capabilities on our own or through collaborations or other marketing partners, we will not be successful in commercializing one or more of our product candidates.\*

We are in the early stages of developing our sales and marketing infrastructure and have never sold, marketed or distributed therapeutic products. To achieve commercial success for any of our product candidates, if at all approved, we must either develop a sales and marketing organization or outsource these functions to third parties. There are risks involved with establishing our own sales and marketing capabilities, as well as entering into arrangements with third parties to perform these services. Developing an internal sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of one or more of our product candidates for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel. On the other hand, if we enter into arrangements with third parties to perform sales, marketing and distribution services, our product revenues will be lower than if we market and sell any products that we develop ourselves.

We also may not be successful entering into arrangements with third parties to sell and market one or more of our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market one or

more of our product candidates effectively, which could damage our reputation. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

Delays in clinical testing could result in increased costs to us.\*

We may not be able to initiate or continue clinical studies or trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these clinical trials as required by the FDA or other regulatory authorities. Even if we are able to enroll a sufficient number of patients in our clinical trials, if the pace of enrollment is slower than we expect, the development costs for our product candidates may increase and the completion of our clinical trials may be delayed or our clinical trials could become too expensive to complete. Significant delays in clinical testing could materially impact our product development costs and timing. Our estimates regarding timing are based on a number of assumptions, including assumptions based on past experience with our other clinical programs. If we are unable to enroll the patients in these trials at the projected rate, the completion of the clinical program could be delayed and the costs of conducting the program could increase, either of which could harm our business.

Clinical trials can be delayed for a variety of reasons, including delays in obtaining regulatory approval to

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commence a study, delays from scaling up of a study, delays in reaching agreement on acceptable clinical trial agreement terms with prospective clinical sites, delays in obtaining institutional review board approval to conduct a study at a prospective clinical site or delays in recruiting subjects to participate in a study. In addition, we typically rely on third-party clinical investigators to conduct our clinical trials and other third-party organizations to oversee the operations of such trials and to perform data collection and analysis. The clinical investigators are not our employees, and we cannot control the amount or timing of resources that they devote to our programs. Failure of the third-party organizations to meet their obligations could adversely affect clinical development of our products. As a result, we may face additional delaying factors outside our control if these parties do not perform their obligations in a timely fashion. For example, any number of those issues could arise with our clinical trials causing a delay. Delays of this sort could occur for the reasons identified above or other reasons. If we have delays in conducting the clinical trials or obtaining regulatory approvals, our product development costs will increase. For example, we may need to make additional payments to third-party investigators and organizations to retain their services or we may need to pay recruitment incentives. If the delays are significant, our financial results and the commercial prospects for our product candidates will be harmed, and our ability to become profitable will be delayed. Moreover, these third-party investigators and organizations may also have relationships with other commercial entities, some of which may compete with us. If these third-party investigators and organizations assist our competitors at our expense, it could harm our competitive position.

We lack the capability to manufacture compounds for clinical development and rely on third parties to manufacture our product candidates, and we may be unable to obtain required material in a timely manner, at an acceptable cost or at a quality level required to receive regulatory approval.\*

We currently do not have the manufacturing capabilities or experience necessary to produce our product candidates for clinical trials, including fostamatinib in ITP, AIHA and IgAN. For each clinical trial of our unpartnered product candidates, we rely on third-party manufacturers for the active pharmaceutical ingredients, as well as various manufacturers to manufacture starting components, excipients and formulated drug products. We rely on manufacturers to produce and deliver all of the materials required for our clinical trials, and many of our preclinical efforts, on a timely basis and to comply with applicable regulatory requirements, including the FDA's current Good Manufacturing Practices (cGMP). In addition, we rely on our suppliers to deliver sufficient quantities of materials produced under cGMP conditions to enable us to conduct planned preclinical studies and clinical trials.

Our current and anticipated future dependence upon these third-party manufacturers may adversely affect our ability to develop and commercialize product candidates on a timely and competitive basis. These manufacturers may not be able to produce material on a timely basis or manufacture material at the quality level or in the quantity required to meet our development timelines and applicable regulatory requirements and may also experience a shortage in qualified personnel. We may not be able to maintain or renew our existing third-party manufacturing arrangements, or enter into new arrangements, on acceptable terms, or at all. Our third party manufacturers could terminate or decline to renew our manufacturing arrangements based on their own business priorities, at a time that is costly or inconvenient for us. If we are unable to contract for the production of materials in sufficient quantity and of sufficient quality on acceptable terms, our planned clinical trials may be significantly delayed. Manufacturing delays could postpone the filing of our IND applications and/or the initiation or completion of clinical trials that we have currently planned or may plan in the future.

Drug manufacturers are subject to ongoing periodic unannounced inspection by the FDA, the Drug Enforcement Administration, and other federal and state agencies to ensure strict compliance with cGMP and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards and they may not be able to comply. Switching manufacturers may be difficult because the number of potential manufacturers is limited. It may be difficult or impossible for us to find a replacement manufacturer quickly on acceptable terms, or at all. Additionally, if we are required to enter into new supply arrangements, we may not be able to obtain approval from the FDA of any alternate supplier in a timely manner, or at all, which could delay or prevent the clinical development and commercialization of any related product candidates. Failure of our third-party manufacturers or us to comply with applicable regulations could result in sanctions being imposed on us, including fines, civil penalties, delays in or failure to grant marketing approval of our product candidates, injunctions, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of products and compounds, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect our business.

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We have obtained orphan drug designation from the FDA for fostamatinib for the treatment of ITP, but we may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug or biologic intended to treat a rare disease or condition, which is defined as one occurring in a patient population of fewer than 200,000 in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug or biologic will be recovered from sales in the United States. In the United States, orphan drug designation entitles a party to financial incentives such as opportunities for grant funding towards clinical trial costs, tax advantages and user-fee waivers. In addition, if a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including a full Biologics License Application, or BLA, to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the manufacturer is unable to assure sufficient product quantity.

Even though we have received orphan drug designation for fostamatinib for the treatment of ITP, we may not be the first to obtain marketing approval for the orphan-designated indication due to the uncertainties associated with developing pharmaceutical products. In addition, exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan-designated indication or may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Further, even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs with different active moieties can be approved for the same condition. Even after an orphan product is approved, the FDA can subsequently approve the same drug with the same active moiety for the same condition if the FDA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

Our research and development efforts will be seriously jeopardized if we are unable to attract and retain key employees and relationships.

As a small company, our success depends on the continued contributions of our principal management and scientific personnel and on our ability to develop and maintain important relationships with leading academic institutions, scientists and companies in the face of intense competition for such personnel. In particular, our research programs depend on our ability to attract and retain highly skilled chemists, other scientists, and development, regulatory and clinical personnel. If we lose the services of any of our key personnel, our research and development efforts could be seriously and adversely affected. Our employees can terminate their employment with us at any time.

Our success as a company is uncertain due to our history of operating losses and the uncertainty of any future profitability.\*

We incurred a loss from operations of approximately \$54.0 million for the nine months ended September 30, 2016. Other than for 2010, we have historically incurred losses from operations each year since we were incorporated in June 1996, due in large part to the significant research and development expenditures required to identify and validate new product candidates and pursue our development efforts. We expect to continue to incur losses from operations and there can be no assurance that we will generate operating income in the foreseeable future. Currently, our only potential sources of revenues are upfront payments, research and development contingent payments and royalty payments pursuant to our collaboration arrangements, which may never materialize if our collaborators do not achieve certain events or generate net sales to which these contingent payments are dependent on. If our drug candidates fail or do not gain regulatory approval, or if our drugs do not achieve market acceptance, we may not be profitable. As of September 30, 2016, we had an accumulated deficit of approximately \$1.0 billion. The extent of our future losses or profitability, if any, is highly uncertain.

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If our corporate collaborations or license agreements are unsuccessful, or if we fail to form new corporate collaborations or license agreements, our research and development efforts could be delayed.

Our strategy depends upon the formation and sustainability of multiple collaborative arrangements and license agreements with third parties now and in the future. We rely on these arrangements for not only financial resources, but also for expertise we need now and in the future relating to clinical trials, manufacturing, sales and marketing, and for licenses to technology rights. To date, we have entered into several such arrangements with corporate collaborators; however, we do not know if these collaborations or additional collaborations with third parties, if any, will dedicate sufficient resources or if any development or commercialization efforts by third parties will be successful. In addition, our corporate collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a drug candidate or development program. Should a collaborative partner fail to develop or commercialize a compound or product to which it has rights from us for any reason, including corporate restructuring, such failure might delay our ongoing research and development efforts, because we might not receive any future payments, and we would not receive any royalties associated with such compound or product. We conducted a Phase 3 clinical program to study fostamatinib in ITP on our own. We may seek another collaborator or licensee in the future for clinical development and commercialization of fostamatinib, as well as our other clinical programs, which we may not be able to obtain on commercially reasonable terms or at all. If we are unable to form new collaborations or enter into new license agreements, our research and development efforts could be delayed. In addition, the continuation of some of our partnered drug discovery and development programs may be dependent on the periodic renewal of our corporate collaborations.

Each of our collaborations could be terminated by the other party at any time, and we may not be able to renew these collaborations on acceptable terms, if at all, or negotiate additional corporate collaborations on acceptable terms, if at all. If these collaborations terminate or are not renewed, any resultant loss of revenues from these collaborations or loss of the resources and expertise of our collaborative partners could adversely affect our business.

Conflicts also might arise with collaborative partners concerning proprietary rights to particular compounds. While our existing collaborative agreements typically provide that we retain milestone payments and royalty rights with respect to drugs developed from certain derivative compounds, any such payments or royalty rights may be at reduced rates, and disputes may arise over the application of derivative payment provisions to such drugs, and we may not be successful in such disputes. Additionally, the management teams of our collaborators may change for various reasons including due to being acquired. Different management teams or an acquiring company of our collaborators may have different priorities which may have adverse results on the collaboration with us.

We are also a party to various license agreements that give us rights to use specified technologies in our research and development processes. The agreements pursuant to which we have in-licensed technology permit our licensors to terminate the agreements under certain circumstances. If we are not able to continue to license these and future technologies on commercially reasonable terms, our product development and research may be delayed or otherwise adversely affected.

If conflicts arise between our collaborators or advisors and us, any of them may act in their self-interest, which may be adverse to our stockholders' interests.

If conflicts arise between us and our corporate collaborators or scientific advisors, the other party may act in its self-interest and not in the interest of our stockholders. Some of our corporate collaborators are conducting multiple product development efforts within each disease area that is the subject of the collaboration with us or may be acquired or merged with a company having a competing program. In some of our collaborations, we have agreed not to conduct, independently or with any third party, any research that is competitive with the research conducted under our collaborations. Our collaborators, however, may develop, either alone or with others, products in related fields that are competitive with the products or potential products that are the subject of these collaborations. Competing products, either developed by our collaborators or to which our collaborators have rights, may result in their withdrawal of support for our product candidates.



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If any of our corporate collaborators were to breach or terminate its agreement with us or otherwise fail to conduct the collaborative activities successfully and in a timely manner, the preclinical or clinical development or commercialization of the affected product candidates or research programs could be delayed or terminated. We generally do not control the amount and timing of resources that our corporate collaborators devote to our programs or potential products. We do not know whether current or future collaborative partners, if any, might pursue alternative technologies or develop alternative products either on their own or in collaboration with others, including our competitors, as a means for developing treatments for the diseases targeted by collaborative arrangements with us.

Our success is dependent on intellectual property rights held by us and third parties, and our interest in such rights is complex and uncertain.\*

Our success will depend to a large part on our own, our licensees' and our licensors' ability to obtain and defend patents for each party's respective technologies and the compounds and other products, if any, resulting from the application of such technologies. We have about 70 pending patent applications and about 342 issued and active patents in the United States, as well as corresponding pending foreign patent applications and issued foreign patents. In the future, our patent position might be highly uncertain and involve complex legal and factual questions. For example, we may be involved in post-grant proceedings before the United States Patent and Trademark Office. Post-grant proceedings are complex and expensive legal proceedings and there is no assurance we will be successful in any such proceedings. A post-grant proceeding could result in our losing our patent rights and/or our freedom to operate and/or require us to pay significant royalties. Additional uncertainty may result because no consistent policy regarding the breadth of legal claims allowed in biotechnology patents has emerged to date. Accordingly, we cannot predict the breadth of claims allowed in our or other companies' patents.

Because the degree of future protection for our proprietary rights is uncertain, we cannot assure you that:

- we were the first to make the inventions covered by each of our pending patent applications;
- we were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any of our pending patent applications will result in issued patents;
- any patents issued to us or our collaborators will provide a basis for commercially-viable products or will provide us with any competitive advantages or will not be challenged by third parties;

- we will develop additional proprietary technologies that are patentable; or
- the patents of others will not have a negative effect on our ability to do business.

We rely on trade secrets to protect technology where we believe patent protection is not appropriate or obtainable; however, trade secrets are difficult to protect. While we require employees, collaborators and consultants to enter into confidentiality agreements, we may not be able to adequately protect our trade secrets or other proprietary information in the event of any unauthorized use or disclosure or the lawful development by others of such information.

We are a party to certain in-license agreements that are important to our business, and we generally do not control the prosecution of in-licensed technology. Accordingly, we are unable to exercise the same degree of control over this intellectual property as we exercise over our internally-developed technology. Moreover, some of our academic institution licensors, research collaborators and scientific advisors have rights to publish data and information in which we have rights. If we cannot maintain the confidentiality of our technology and other confidential information in connection with our collaborations, our ability to receive patent protection or protect our proprietary information may otherwise be impaired. In addition, some of the technology we have licensed relies on patented inventions developed using U.S. government resources.

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The U.S. government retains certain rights, as defined by law, in such patents, and may choose to exercise such rights. Certain of our in-licenses may be terminated if we fail to meet specified obligations. If we fail to meet such obligations and any of our licensors exercise their termination rights, we could lose our rights under those agreements. If we lose any of our rights, it may adversely affect the way we conduct our business. In addition, because certain of our licenses are sublicenses, the actions of our licensors may affect our rights under those licenses.

If a dispute arises regarding the infringement or misappropriation of the proprietary rights of others, such dispute could be costly and result in delays in our research and development activities and partnering.

Our success will depend, in part, on our ability to operate without infringing or misappropriating the proprietary rights of others. There are many issued patents and patent applications filed by third parties relating to products or processes that are similar or identical to our licensors or ours, and others may be filed in the future. There may also be copyrights or trademarks that third parties hold. There can be no assurance that our activities, or those of our licensors, will not violate intellectual property rights of others. We believe that there may be significant litigation in the industry regarding patent and other intellectual property rights, and we do not know if our collaborators or we would be successful in any such litigation. Any legal action against our collaborators or us claiming damages or seeking to enjoin commercial activities relating to the affected products, our methods or processes could:

- require our collaborators or us to obtain a license to continue to use, manufacture or market the affected products, methods or processes, which may not be available on commercially reasonable terms, if at all;
- prevent us from using the subject matter claimed in the patents held by others;
- subject us to potential liability for damages;
- consume a substantial portion of our managerial and financial resources; and
- result in litigation or administrative proceedings that may be costly, whether we win or lose.

Our ability to use net operating losses and certain other tax attributes is uncertain and may be limited.

Our ability to use our federal and state net operating losses to offset potential future taxable income and related income taxes that would otherwise be due is dependent upon our generation of future taxable income before the

expiration dates of the net operating losses, and we cannot predict with certainty when, or whether, we will generate sufficient taxable income to use all of our net operating losses. In addition, utilization of net operating losses to offset potential future taxable income and related income taxes that would otherwise be due is subject to annual limitations under the “ownership change” provisions of Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (Internal Revenue Code) and similar state provisions, which may result in the expiration of net operating losses before future utilization. In general, under the Code, if a corporation undergoes an “ownership change,” generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation’s ability to use its pre-change net operating losses and other pre-change tax attributes (such as research and development credit carryforwards) to offset its post-change taxable income or taxes may be limited. Our equity offerings and other changes in our stock ownership, some of which are outside of our control, may have resulted or could in the future result in an ownership change. Although we have completed studies to provide reasonable assurance that an ownership change limitation would not apply, we cannot be certain that a taxing authority would reach the same conclusion. If, after a review or audit, an ownership change limitation were to apply, utilization of our domestic net operating losses and tax credit carryforwards could be limited in future periods and a portion of the carryforwards could expire before being available to reduce future income tax liabilities.

Because we expect to be dependent upon collaborative and license agreements, we might not meet our strategic objectives.

Our ability to generate revenue in the near term depends on the timing of recognition of certain upfront payments, achievement of certain payment triggering events with our existing collaboration agreements and our ability to

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enter into additional collaborative agreements with third parties. Our ability to enter into new collaborations and the revenue, if any, that may be recognized under these collaborations is highly uncertain. If we are unable to enter into one or more new collaborations, our business prospects could be harmed, which could have an immediate adverse effect on our ability to continue to develop our compounds and on the trading price of our stock. Our ability to enter into a collaboration may be dependent on many factors, such as the results of our clinical trials, competitive factors and the fit of one of our programs with another company's risk tolerance, including toward regulatory issues, patent portfolio, clinical pipeline, the stage of the available data, particularly if it is early, overall corporate goals and financial position.

To date, a portion of our revenues have been related to the research or transition phase of each of our collaborative agreements. Such revenues are for specified periods, and the impact of such revenues on our results of operations is at least partially offset by corresponding research costs. Following the completion of the research or transition phase of each collaborative agreement, additional revenues may come only from payments triggered by milestones and/or the achievement of other contingent events, and royalties, which may not be paid, if at all, until certain conditions are met. This risk is heightened due to the fact that unsuccessful research efforts may preclude us from receiving any contingent payments under these agreements. Our receipt of revenues from collaborative arrangements is also significantly affected by the timing of efforts expended by us and our collaborators and the timing of lead compound identification. We have received payments from our collaborations with Aclaris, BMS, AZ, BerGenBio, Janssen Pharmaceutica N.V., a division of Johnson & Johnson, Novartis Pharma A.G., Daiichi, Merck & Co., Inc., Merck Serono and Pfizer. Under many agreements, future payments may not be earned until the collaborator has advanced product candidates into clinical testing, which may never occur or may not occur until some time well into the future. If we are not able to generate revenue under our collaborations when and in accordance with our expectations or the expectations of industry analysts, this failure could harm our business and have an immediate adverse effect on the trading price of our common stock.

Our business requires us to generate meaningful revenue from royalties and licensing agreements. To date, we have not received any revenue from royalties for the commercial sale of drugs, and we do not know when we will receive any such revenue, if at all.

Securities class action lawsuits or other litigation could result in substantial damages and may divert management's time and attention from our business.

We have been subject to class action lawsuits in the past, including a securities class action lawsuit commenced in the United States District Court for the Northern District of California in February 2009, that was ultimately dismissed in November 2012. However, we may be subject to similar or completely unrelated claims in the future, such as those that might occur if there was to be a change in our corporate strategy. These and other lawsuits are subject to inherent uncertainties, and the actual costs to be incurred relating to the lawsuit will depend upon many unknown factors. The outcome of litigation is necessarily uncertain, and we could be forced to expend significant resources in the defense of such suits, and we may not prevail. Monitoring and defending against legal actions is time-consuming for our management and detracts from our ability to fully focus our internal resources on our business activities. In addition, we may incur substantial legal fees and costs in connection with any such litigation. We have not established any

reserves for any potential liability relating to any such potential lawsuits. It is possible that we could, in the future, incur judgments or enter into settlements of claims for monetary damages. A decision adverse to our interests on any such actions could result in the payment of substantial damages, or possibly fines, and could have a material adverse effect on our cash flow, results of operations and financial position.

If our competitors develop technologies that are more effective than ours, our commercial opportunity will be reduced or eliminated.

The biotechnology and pharmaceutical industries are intensely competitive and subject to rapid and significant technological change. Many of the drugs that we are attempting to discover will be competing with existing therapies. In addition, a number of companies are pursuing the development of pharmaceuticals that target the same diseases and conditions that we are targeting. For example, there are existing therapies and drug candidates in development for the treatment of ITP that may be alternative therapies to fostamatinib, if it is ultimately approved for commercialization. We face, and will continue to face, intense competition from pharmaceutical and biotechnology companies, as well as from academic and research institutions and government agencies, both in the United States and abroad. Some of these

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competitors are pursuing the development of pharmaceuticals that target the same diseases and conditions as our research programs. Our major competitors include fully integrated pharmaceutical companies that have extensive drug discovery efforts and are developing novel small-molecule pharmaceuticals. We also face significant competition from organizations that are pursuing the same or similar technologies, including the discovery of targets that are useful in compound screening, as the technologies used by us in our drug discovery efforts.

Competition may also arise from:

- new or better methods of target identification or validation;
- other drug development technologies and methods of preventing or reducing the incidence of disease;
- new small molecules; or
- other classes of therapeutic agents.

Our competitors or their collaborative partners may utilize discovery technologies and techniques or partner with collaborators in order to develop products more rapidly or successfully than we or our collaborators are able to do. Many of our competitors, particularly large pharmaceutical companies, have substantially greater financial, technical and human resources and larger research and development staffs than we do. In addition, academic institutions, government agencies and other public and private organizations conducting research may seek patent protection with respect to potentially competitive products or technologies and may establish exclusive collaborative or licensing relationships with our competitors.

We believe that our ability to compete is dependent, in part, upon our ability to create, maintain and license scientifically-advanced technology and upon our and our collaborators' ability to develop and commercialize pharmaceutical products based on this technology, as well as our ability to attract and retain qualified personnel, obtain patent protection or otherwise develop proprietary technology or processes and secure sufficient capital resources for the expected substantial time period between technological conception and commercial sales of products based upon our technology. The failure by any of our collaborators or us in any of those areas may prevent the successful commercialization of our potential drug targets.

Many of our competitors, either alone or together with their collaborative partners, have significantly greater experience than we do in:

- identifying and validating targets;
- screening compounds against targets; and
- undertaking preclinical testing and clinical trials.

Accordingly, our competitors may succeed in obtaining patent protection, identifying or validating new targets or discovering new drug compounds before we do.

Our competitors might develop technologies and drugs that are more effective or less costly than any that are being developed by us or that would render our technology and product candidates obsolete and noncompetitive. In addition, our competitors may succeed in obtaining the approval of the FDA or other regulatory agencies for product candidates more rapidly. Companies that complete clinical trials, obtain required regulatory agency approvals and commence commercial sale of their drugs before us may achieve a significant competitive advantage, including certain patent and FDA marketing exclusivity rights that would delay or prevent our ability to market certain products. Any drugs resulting from our research and development efforts, or from our joint efforts with our existing or future collaborative partners, might not be able to compete successfully with competitors' existing or future products or obtain regulatory approval in the United States or elsewhere.



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We face and will continue to face intense competition from other companies for collaborative arrangements with pharmaceutical and biotechnology companies, for establishing relationships with academic and research institutions and for licenses to additional technologies. These competitors, either alone or with their collaborative partners, may succeed in developing technologies or products that are more effective than ours.

Our stock price may be volatile, and our stockholders' investment in our stock could decline in value.

The market prices for our common stock and the securities of other biotechnology companies have been highly volatile and may continue to be highly volatile in the future. The following factors, in addition to other risk factors described in this section, may have a significant impact on the market price of our common stock:

- the progress and success of our clinical trials and preclinical activities (including studies and manufacture of materials) of our product candidates conducted by us;
- the receipt or failure to receive the additional funding necessary to conduct our business;
- selling by large stockholders;
- presentations of detailed clinical trial data at medical and scientific conferences and investor perception thereof;
- announcements of technological innovations or new commercial products by our competitors or us;
- developments concerning proprietary rights, including patents;
- developments concerning our collaborations;
- publicity regarding actual or potential medical results relating to products under development by our competitors or us;
- regulatory developments in the United States and foreign countries;
- litigation or arbitration;

- economic and other external factors or other disaster or crisis; and
- period-to-period fluctuations in financial results.

If we fail to continue to meet the listing standards of NASDAQ, our common stock may be delisted, which could have a material adverse effect on the liquidity of our common stock.

Our common stock is currently listed on the Nasdaq Global Market. The NASDAQ Stock Market LLC has requirements that a company must meet in order to remain listed on NASDAQ. In particular, NASDAQ rules require us to maintain a minimum bid price of \$1.00 per share of our common stock. If the closing bid price of our common stock were to fall below \$1.00 per share for 30 consecutive trading days or we do not meet other listing requirements, we would fail to be in compliance with NASDAQ's listing standards. There can be no assurance that we will continue to meet the minimum bid price requirement, or any other requirement in the future. If we fail to meet the minimum bid price requirement, The NASDAQ Stock Market LLC may initiate the delisting process with a notification letter. If we were to receive such a notification, we would be afforded a grace period of 180 calendar days to regain compliance with the minimum bid price requirement. In order to regain compliance, shares of our common stock would need to maintain a minimum closing bid price of at least \$1.00 per share for a minimum of 10 consecutive trading days. In addition, we may be unable to meet other applicable NASDAQ listing requirements, including maintaining minimum levels of stockholders' equity or market values of our common stock in which case, our common stock could be delisted. If our common stock were to be delisted, the liquidity of our common stock would be adversely affected and the market price of our common stock could decrease.

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The vote by the United Kingdom (U.K.) electorate in favor of the U.K.'s exit from the European Union (E.U.) could adversely impact our business, results of operations and financial condition.\*

The passage of the referendum on the U.K.'s membership in the E.U., referred to as "Brexit," in June 2016 resulted in a determination that the U.K. should exit the E.U. Such an exit from the E.U. could cause uncertainty in the credit markets and financial services industry which could result to lower interest paid on certain of our investments and the value of certain securities we hold may decline in the future, which could negatively affect our financial condition, results of operations and cash flow, as well as limit our future access to the capital markets. The Brexit could also cause disruptions to and create uncertainty surrounding the business environment in which we operate. For example, we conduct clinical trials in the U.K. and other E.U. member states. Although the terms of U.K.'s exit from and its future relationship with E.U. are unknown, it is possible that there will be increased regulatory complexities which can disrupt the timing of our clinical trials and regulatory approvals, if any, of our current and future product candidates.

Our ability to generate revenues will be diminished if we or our collaborative partners fail to obtain acceptable prices or an adequate level of reimbursement for products from third-party payers or government agencies.

The drugs we hope to develop may be rejected by the marketplace due to many factors, including cost. Our ability to commercially exploit a drug may be limited due to the continuing efforts of government and third-party payers to contain or reduce the costs of health care through various means. For example, in some foreign markets, pricing and profitability of prescription pharmaceuticals are subject to government control. In the United States, we expect that there will continue to be a number of federal and state proposals to implement similar government control. In addition, increasing emphasis on managed care in the United States will likely continue to put pressure on the pricing of pharmaceutical products. Cost control initiatives could decrease the price that we or any of our collaborators would receive for any products in the future. Further, cost control initiatives could adversely affect our and our collaborators' ability to commercialize our products and our ability to realize royalties from this commercialization.

Our ability to commercialize pharmaceutical products with collaborators may depend, in part, on the extent to which reimbursement for the products will be available from:

- government and health administration authorities;
  
- private health insurers; and
  
- other third-party payers.

Significant uncertainty exists as to the reimbursement status of newly-approved healthcare products. Third-party payers, including Medicare, are challenging the prices charged for medical products and services. Government and other third-party payers increasingly are attempting to contain healthcare costs by limiting both coverage and the level of reimbursement for new drugs and by refusing, in some cases, to provide coverage for uses of approved products for disease indications for which the FDA has not granted labeling approval. Third-party insurance coverage may not be available to patients for any products we discover and develop, alone or with collaborators. If government and other third-party payers do not provide adequate coverage and reimbursement levels for our products, the market acceptance of these products may be reduced.

If product liability lawsuits are successfully brought against us, we may incur substantial liabilities and may be required to limit commercialization of our products.

The testing and marketing of medical products entail an inherent risk of product liability. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our products. We carry product liability insurance that is limited in scope and amount and may not be adequate to fully protect us against product liability claims. Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of pharmaceutical products we develop, alone or with corporate collaborators. We, or our corporate collaborators, might not be able to obtain insurance at a reasonable cost, if at all. While under various circumstances we are entitled to be indemnified against losses by our corporate collaborators, indemnification may not be available or

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adequate should any claim arise.

We depend on various scientific consultants and advisors for the success and continuation of our research and development efforts.

We work extensively with various scientific consultants and advisors. The potential success of our drug discovery and development programs depends, in part, on continued collaborations with certain of these consultants and advisors. We, and various members of our management and research staff, rely on certain of these consultants and advisors for expertise in our research, regulatory and clinical efforts. Our scientific advisors are not our employees and may have commitments to, or consulting or advisory contracts with, other entities that may limit their availability to us. We do not know if we will be able to maintain such consulting agreements or that such scientific advisors will not enter into consulting arrangements, exclusive or otherwise, with competing pharmaceutical or biotechnology companies, any of which would have a detrimental impact on our research objectives and could have a material adverse effect on our business, financial condition and results of operations.

If we use biological and hazardous materials in a manner that causes injury or violates laws, we may be liable for damages, penalties or fines.

Our research and development activities involve the controlled use of potentially harmful biological materials as well as hazardous materials, chemicals and various radioactive compounds. We cannot completely eliminate the risk of accidental contamination or injury from the use, storage, handling or disposal of these materials. In the event of contamination or injury, we could be held liable for damages that result or for penalties or fines that may be imposed, and such liability could exceed our resources. We are also subject to federal, state and local laws and regulations governing the use, storage, handling and disposal of these materials and specified waste products. The cost of compliance with, or any potential violation of, these laws and regulations could be significant.

Our internal computer systems, or those used by our contract research organizations or other contractors or consultants, may fail or suffer security breaches.

Despite the implementation of security measures, our internal computer systems and those of our contract research organizations and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a disruption of our drug development programs. For example, the loss of clinical trial data from completed or ongoing clinical trials for a product candidate could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of or damage to our data or applications, or inappropriate disclosure of

confidential or proprietary information, we could incur liability and the further development of any product candidates could be delayed.

Our facilities are located near known earthquake fault zones, and the occurrence of an earthquake or other catastrophic disaster could cause damage to our facilities and equipment, which could require us to cease or curtail operations.

Our facilities are located in the San Francisco Bay Area near known earthquake fault zones and are vulnerable to significant damage from earthquakes. We are also vulnerable to damage from other types of disasters, including fires, floods, power loss, communications failures and similar events. If any disaster were to occur, our ability to operate our business at our facilities would be seriously, or potentially completely, impaired, and our research could be lost or destroyed. In addition, the unique nature of our research activities and of much of our equipment could make it difficult for us to recover from a disaster. The insurance we maintain may not be adequate to cover our losses resulting from disasters or other business interruptions.

Future equity issuances or a sale of a substantial number of shares of our common stock may cause the price of our common stock to decline.\*

Because we will continue to need additional capital in the future to continue to expand our business and our

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research and development activities, among other things, we may conduct additional equity offerings. For example, under the universal shelf registration statement filed by us in May 2015 and declared effective by the SEC in July 2015, we may offer and sell any combination of common stock, preferred stock, debt securities and warrants in one or more offerings, up to a cumulative value of \$150 million. If we or our stockholders sell, or if it is perceived that we or they will sell, substantial amounts of our common stock (including shares issued upon the exercise of options and warrants) in the public market, the market price of our common stock could fall. A decline in the market price of our common stock could make it more difficult for us to sell equity or equity-related securities in the future at a time and price that we deem appropriate. In addition, future sales by us of our common stock may be dilutive to existing stockholders. Furthermore, if we obtain funds through a credit facility or through the issuance of debt or preferred securities, these securities would likely have rights senior to the rights of our common stockholders, which could impair the value of our common stock.

Anti-takeover provisions in our charter documents and under Delaware law may make an acquisition of us, which may be beneficial to our stockholders, more difficult.

Provisions of our amended and restated certificate of incorporation and bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us, even if doing so would benefit our stockholders. These provisions:

- establish that members of the board of directors may be removed only for cause upon the affirmative vote of stockholders owning a majority of our capital stock;
- authorize the issuance of “blank check” preferred stock that could be issued by our board of directors to increase the number of outstanding shares and thwart a takeover attempt;
- limit who may call a special meeting of stockholders;
- prohibit stockholder action by written consent, thereby requiring all stockholder actions to be taken at a meeting of our stockholders;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted upon at stockholder meetings;
- provide for a board of directors with staggered terms; and
- provide that the authorized number of directors may be changed only by a resolution of our board of directors.

In addition, Section 203 of the Delaware General Corporation Law, which imposes certain restrictions relating to transactions with major stockholders, may discourage, delay or prevent a third party from acquiring us.

Item 2.Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3.Defaults Upon Senior Securities

None.

Item 4.Mine Safety Disclosures

Not applicable.

Item 5.Other Information

None.

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## Item 6.Exhibits

The exhibits listed on the accompanying index to exhibits are filed or incorporated by reference (as stated therein) as part of this Quarterly Report on Form 10-Q.

Exhibit Number	Description of Document
3.1	Amended and Restated Certificate of Incorporation. (1)
3.2	Amended and Restated Bylaws. (2)
4.1	Form of warrant to purchase shares of common stock. (3)
4.2	Specimen Common Stock Certificate. (4)
4.3	Warrant issued to HCP BTC, LLC for the purchase of shares of common stock. (5)
10.1	Separation Agreement by and between Rigel Pharmaceuticals, Inc. and Donald G. Payan, M.D., dated September 15, 2016.
10.2	Offer Letter from Rigel Pharmaceuticals, Inc. to Eldon C. Mayer III, dated September 12, 2016 (6)
10.3	Offer Letter from Rigel Pharmaceuticals, Inc. to Joseph Lasaga, dated September 26, 2016 (6)
31.1	Certification required by Rule 13a-14(a) or Rule 15d-14(a) of the Exchange Act.
31.2	Certification required by Rule 13a-14(a) or Rule 15d-14(a) of the Exchange Act.
32.1	Certification required by Rule 13a-14(b) or Rule 15d-14(b) of the Exchange Act and Section 1350 of Chapter 63 of Title 18 of the United States Code (18 U.S.C. 1350).
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document

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- (1) Filed as an exhibit to Rigel's Current Report on Form 8-K (No. 000-29889) filed on May 29, 2012 and incorporated herein by reference.
- (2) Filed as an exhibit to Rigel's Current Report on Form 8-K (No. 000-29889) filed on February 2, 2007 and incorporated herein by reference.
- (3) Filed as an exhibit to Rigel's Registration Statement on Form S-1 (No. 333-45864), as amended, and incorporated herein by reference.
- (4) Filed as an exhibit to Rigel's Current Report on Form 8-K (No. 000-29889) filed on June 24, 2003, and incorporated herein by reference.
- (5) Filed as an exhibit to Rigel's Quarterly Report on Form 10-Q (No. 000-29889) for the quarter ended March 31, 2009, and incorporated herein by reference.
- (6) Confidential treatment requested as to specific portions, which portions are omitted and filed separately with the Securities and Exchange Commission.

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SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

RIGEL PHARMACEUTICALS, INC.

By: /s/ RAUL R. RODRIGUEZ  
Raul R. Rodriguez  
Chief Executive Officer  
(Principal Executive Officer)

Date: November 1, 2016

By: /s/ RYAN D. MAYNARD  
Ryan D. Maynard  
Executive Vice President and Chief Financial Officer  
(Principal Financial and Accounting Officer)

Date: November 1, 2016

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