

RIGEL PHARMACEUTICALS INC

Form 10-Q

August 01, 2017

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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 JUNE 30, 2017

OR

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

FOR THE TRANSITION PERIOD FROM      TO

Commission File Number 0-29889

Rigel Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

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

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

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

Emerging Growth Company

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

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

As of July 26, 2017, there were 124,392,998 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 JUNE 30, 2017

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## PART I. FINANCIAL INFORMATION

## Item 1. Financial Statements

## RIGEL PHARMACEUTICALS, INC.

## CONDENSED BALANCE SHEETS

(In thousands)

	June 30, 2017 (unaudited)	December 31, 2016(1)
Assets		
Current assets:		
Cash and cash equivalents	\$ 32,591	\$ 17,632
Short-term investments	49,711	57,134
Prepaid and other current assets	1,490	1,448
Total current assets	83,792	76,214
Property and equipment, net	982	1,156
Other assets	704	764
	\$ 85,478	\$ 78,134
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 2,066	\$ 5,563
Accrued compensation	3,659	4,085
Accrued research and development	5,171	5,881
Other accrued liabilities	996	1,033
Deferred liability – sublease, current portion	2,196	3,222
Deferred rent, current portion	1,785	2,804
Total current liabilities	15,873	22,588
Long-term portion of deferred liability – sublease	—	238
Long-term portion of deferred rent	—	279
Other long-term liabilities	—	2
Commitments		

Stockholders' equity:		
Preferred stock	—	—
Common stock	124	100
Additional paid-in capital	1,164,823	1,115,807
Accumulated other comprehensive loss	(19)	(18)
Accumulated deficit	(1,095,323)	(1,060,862)
Total stockholders' equity	69,605	55,027
	\$ 85,478	\$ 78,134

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(1) The balance sheet at December 31, 2016 has been derived from the audited financial statements included in Rigel's Annual Report on Form 10-K for the year ended December 31, 2016.

See Accompanying Notes.

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RIGEL PHARMACEUTICALS, INC.

## CONDENSED STATEMENTS OF OPERATIONS

(In thousands, except per share amounts)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Contract revenues from collaborations	\$ —	\$ 8,594	\$ 3,584	\$ 13,623
Costs and expenses:				
Research and development	11,524	17,468	23,900	35,641
General and administrative	7,820	4,774	15,230	9,197
Total costs and expenses	19,344	22,242	39,130	44,838
Loss from operations	(19,344)	(13,648)	(35,546)	(31,215)
Gain on disposal of assets	—	—	732	—
Interest income	197	115	353	218
Net loss	\$ (19,147)	\$ (13,533)	\$ (34,461)	\$ (30,997)
Net loss per share, basic and diluted	\$ (0.16)	\$ (0.15)	\$ (0.29)	\$ (0.34)
Weighted average shares used in computing net loss per share, basic and diluted	122,500	92,495	118,074	91,525

See Accompanying Notes.

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RIGEL PHARMACEUTICALS, INC.

CONDENSED STATEMENTS OF COMPREHENSIVE LOSS

(In thousands)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Net loss	\$ (19,147)	\$ (13,533)	\$ (34,461)	\$ (30,997)
Other comprehensive income (loss):				
Net unrealized gain (loss) on short-term investments	10	4	(1)	97
Comprehensive loss	\$ (19,137)	\$ (13,529)	\$ (34,462)	\$ (30,900)

See Accompanying Notes.

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RIGEL PHARMACEUTICALS, INC.

## CONDENSED STATEMENTS OF CASH FLOWS

(In thousands)

(unaudited)

	Six Months Ended June 30,	
	2017	2016
Operating activities		
Net loss	\$ (34,461)	\$ (30,997)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	2,055	3,452
Gain on disposal of assets	(732)	—
Loss on sublease	495	—
Depreciation and amortization	240	584
Net amortization of premium on short-term investment	(107)	—
Changes in assets and liabilities:		
Accounts receivable	—	108
Prepaid and other current assets	(42)	683
Other assets	60	88
Accounts payable	(3,508)	(1,263)
Accrued compensation	(426)	(3,132)
Accrued research and development	(710)	1,749
Other accrued liabilities	(37)	136
Deferred revenue	—	(9,667)
Deferred rent and other long term liabilities	(3,059)	(2,595)
Net cash used in operating activities	(40,232)	(40,854)
Investing activities		
Purchases of short-term investments	(44,920)	(67,895)
Maturities of short-term investments	52,449	80,818
Proceeds from disposal of assets	732	—
Capital expenditures	(55)	(546)
Net cash provided by investing activities	8,206	12,377
Financing activities		
Net proceeds from issuances of common stock upon exercise of options and participation in employee stock purchase plan	810	618
Proceeds from sale and issuance of common stock, net of offering costs	46,175	9,349
Net cash provided by financing activities	46,985	9,967
Net increase (decrease) in cash and cash equivalents	14,959	(18,510)
Cash and cash equivalents at beginning of period	17,632	43,456

Cash and cash equivalents at end of period	\$ 32,591	\$ 24,946
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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 small molecule drugs that significantly improve the lives of patients with immune and hematological disorders, cancer and rare diseases.

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, 2016 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, 2016.

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 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. 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. The adoption of ASU No. 2014-09 may have a material effect on our financial statements. To date, our revenues have been derived from license and collaboration agreements. The consideration we are eligible to receive under these agreements includes upfront payments, progress dependent contingent payments on events achieved by our collaboration partners, and royalties on net sales of products sold by such partners under the agreements. Each license and collaboration agreement is unique and will need to be assessed separately under the five-step process of the new standard. ASU No. 2014-09

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differs from the current accounting standard in many respects, such as in the accounting for variable consideration, including milestone payments or contingent payments. Under our current accounting policy, we recognize contingent payments as revenue in the period that the payment-triggering event occurred or is achieved. However, under the new accounting standard, it is possible to start to recognize contingent payments before the payment-triggering event is completely achieved, subject to management's assessment of whether it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is subsequently resolved. We have performed a preliminary assessment of the impact of the new standard on our active license and collaboration agreements. Based on this preliminary assessment, we expect that the timing of recognition of certain future milestone payments may be impacted depending on the assessed probability of achievement for these milestones as of the date of adoption.

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. We adopted ASU No. 2016-09 on January 1, 2017. Under this guidance, on a prospective basis, companies will no longer record excess tax benefits and certain tax deficiencies in additional paid-in capital. Instead, they will record all excess tax benefits and tax deficiencies as income tax expense or benefit in the income statement. In addition, the guidance eliminates the requirement that excess tax benefits be realized before companies can recognize them. The ASU requires a cumulative-effect adjustment for previously unrecognized excess tax benefits in opening retained earnings in the annual period of adoption. Upon adoption, we recognized additional excess tax benefit as a deferred tax asset with a corresponding increase to our deferred tax asset valuation allowance, which did not result in a net impact to retained earnings. Additionally, as provided for under this new guidance, we elected to account for forfeitures as they occur. The adoption of this aspect of the guidance did not have a material impact on our financial statements.

#### 4. Stock Award Plans

We have four stock option plans, our 2011 Equity Incentive Plan (2011 Plan), 2000 Equity Incentive Plan (2000 Plan), 2000 Non-Employee Directors' Stock Option Plan (Directors' Plan) and the Inducement 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. We use the straight-line attribution method over the requisite employee service period for the entire award in recognizing stock-based compensation expense. In connection with the adoption of ASU No. 2016-09—Improvements to Employee Share-Based Payment Accounting, on January 1, 2017, we have elected to account for forfeitures as they occur.

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

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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 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		Six Months Ended	
	June 30, 2017	2016	June 30, 2017	2016
Outstanding stock options	21,958	22,232	21,958	22,232
Warrant to purchase common stock	—	200	—	200
Purchase Plan	187	247	124	156
	22,145	22,679	22,082	22,588

## 6. Stock-based Compensation

Total stock-based compensation expense related to all of our share-based payments that we recognized for the three and six months ended June 30, 2017 and 2016 were as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
General and administrative	\$ 764	\$ 604	\$ 1,359	\$ 1,349
Research and development	336	1,410	696	2,103
Total stock-based compensation expense	\$ 1,100	\$ 2,014	\$ 2,055	\$ 3,452

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.



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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.

In connection with the adoption of ASU No. 2016-09 on January 1, 2017, we have elected to account for forfeitures as they occur and its adoption did not have a material impact on our financial statements.

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 certain corporate performance-based milestones or corporate sales target, for the three and six months ended June 30, 2017 and 2016:

	Three Months Ended				Six Months Ended			
	June 30, 2017		2016		June 30, 2017		2016	
Risk-free interest rate	2.1	%	1.5	%	2.2	%	1.7	%

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Expected term (in years)	6.6		7.0		6.8		6.4	
Dividend yield	0.0	%	0.0	%	0.0	%	0.0	%
Expected volatility	63.7	%	76.9	%	63.0	%	63.3	%

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.

We granted options to purchase 2,992,675 shares of common stock during the six months ended June 30, 2017 with a grant-date weighted-average fair value of \$1.35 per share. Of the 2,992,675 common stock options granted, 1,025,000 shares related to outstanding performance-based stock option awards with a grant date fair value of \$1.3 million will vest upon the achievement of a corporate performance-based milestone and 75,000 shares related to performance-based stock option awards with a grant date fair value of \$111,000 will vest upon achievement of certain corporate sales targets. We did not consider the corporate-based milestone nor the corporate sales targets as probable of

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achievement as of June 30, 2017. Accordingly, no stock-based compensation cost was recognized during the three and six months ended June 30, 2017 for these performance-based stock option awards.

We granted options to purchase 3,833,435 shares of common stock during the six months ended June 30, 2016, 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 related to outstanding performance-based stock option awards with a grant date fair value of \$1.1 million which vested upon the achievement of a corporate performance-based milestone as of December 31, 2016. Accordingly, we recognized the \$1.1 million as stock-based compensation expense during the fourth quarter of 2016. In addition, as of June 30, 2017, we have 200,000 shares of outstanding performance-based stock option awards granted in the fourth quarter of 2016, wherein 100,000 shares with a grant date fair value of \$232,000 will vest upon achievement of a corporate performance-based milestone and 100,000 shares with a grant date fair value of \$240,000 will vest upon achievement of certain corporate sales targets. We did not consider the corporate-based milestone nor the corporate sales targets as probable of achievement as of June 30, 2017. Accordingly, for these performance-based option awards, no stock-based compensation expense was recognized during the three and six months ended June 30, 2017.

As of June 30, 2017, there was approximately \$7.5 million of total unrecognized stock-based compensation cost related to all unvested options granted under our equity incentive plans.

At June 30, 2017, there were 10,244,931 shares of common stock available for future grant under our equity incentive plans and 166,796 options to purchase shares were exercised during the six months ended June 30, 2017.

Employee Stock Purchase Plan

Our 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.

As of June 30, 2017, there were approximately 2,324,942 shares reserved for future issuance under the Purchase Plan. The following table summarizes the weighted-average assumptions related to our Purchase Plan for the six months ended June 30, 2017 and 2016. 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.

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	Six Months Ended			
	June 30,		2016	
	2017		2016	
Risk-free interest rate	0.5	%	0.7	%
Expected term (in years)	1.5		1.8	
Dividend yield	0.0	%	0.0	%
Expected volatility	63.1	%	61.5	%

## 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.

In the first quarter of 2017, we entered into a consulting agreement with a third party, pursuant to which we may be required to pay amounts ranging from \$1.5 million to \$4.0 million if certain future regulatory milestone events occur. As of June 30, 2017, we do not consider any of the future regulatory milestone events as probable of occurring. As such, no expense was recognized for the three and six months ended June 30, 2017.

## 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 during the research term was 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 \$533.3 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 \$146.4 million relates to the achievement of development events, up to \$345.6 million relates to the achievement of regulatory events and up to \$41.3 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 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

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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 were a single unit of accounting as the license did 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 and was fully amortized in September 2016, the end of the research term. We believed that straight-line recognition of this revenue was appropriate as the research was performed ratably over the research period. During the three and six months ended June 30, 2016, we recognized revenue of \$4.8 million and \$9.7 million, respectively, relating to the upfront payment and \$95,000 and \$290,000, respectively, relating to the research activities we performed. 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. As of September 30, 2016, all deliverables under the agreement have been delivered.

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 February 2017, we received \$3.3 million from BerGenBio as a result of BerGenBio advancing BGB324, an AXL kinase inhibitor licensed under the agreement, to a Phase 2 clinical study. 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 \$3.3 million in 2017 and \$3.7 million in 2016, triggered by the above time-based and contingent events were recognized as revenue in the first quarter of 2017 and second quarter of 2016, respectively.

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

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

	June 30, 2017	December 31, 2016
Cash	\$ 352	\$ 240
Money market funds	6,750	9,496
U.S. treasury bills	—	4,300
Government-sponsored enterprise securities	9,062	16,459
Corporate bonds and commercial paper	66,138	44,271
	\$ 82,302	\$ 74,766
Reported as:		
Cash and cash equivalents	\$ 32,591	\$ 17,632

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Short-term investments	49,711	57,134
	\$ 82,302	\$ 74,766

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

June 30, 2017	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
Government-sponsored enterprise securities	\$ 9,067	\$ —	\$ (5)	\$ 9,062
Corporate bonds and commercial paper	66,152	—	(14)	66,138
Total	\$ 75,219	\$ —	\$ (19)	\$ 75,200



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December 31, 2016	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury bills	\$ 4,300	\$ —	\$ —	\$ 4,300
Government-sponsored enterprise securities	16,457	3	(1)	16,459
Corporate bonds and commercial paper	44,291	2	(22)	44,271
Total	\$ 65,048	\$ 5	\$ (23)	\$ 65,030

As of June 30, 2017, our cash equivalents and short-term investments, which have contractual maturities within one year, had a weighted-average time to maturity of approximately 83 days. We view our short-term investments portfolio as available for use in current operations. We have the ability to hold all investments as of June 30, 2017 through their respective maturity dates. At June 30, 2017, we had no investments that had been in a continuous unrealized loss position for more than 12 months. As of June 30, 2017, a total of 30 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 significant 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 June 30, 2017.

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):

June 30, 2017	Fair Value	Unrealized Losses
Government-sponsored enterprise securities	\$ 6,663	\$ (5)
Corporate bonds and commercial paper	32,716	(14)
Total	\$ 39,379	\$ (19)

## 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

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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 June 30, 2017			
	Level 1	Level 2	Level 3	Total
Money market funds	\$ 6,750	\$ —	\$ —	\$ 6,750
Government-sponsored enterprise securities	—	9,062	—	9,062
Corporate bonds and commercial paper	—	66,138	—	66,138
Total	\$ 6,750	\$ 75,200	\$ —	\$ 81,950

	Assets at Fair Value as of December 31, 2016			
	Level 1	Level 2	Level 3	Total
Money market funds	\$ 9,496	\$ —	\$ —	\$ 9,496
U.S. treasury bills	—	4,300	—	4,300
Government-sponsored enterprise securities	—	16,459	—	16,459
Corporate bonds and commercial paper	—	44,271	—	44,271
Total	\$ 9,496	\$ 65,030	\$ —	\$ 74,526

## 11. Lease Agreements

We currently lease our research and office space under a noncancelable lease agreement with our landlord, HCP BTC, LLC (formerly known as Slough BTC, LLC) which expires in 2018. The lease term provides for renewal option for up to two additional periods of five years each. In April 2017, we notified our landlord that we exercised our option to extend the term of our lease for another five years through 2023 and is in the process of negotiating certain terms of our lease agreement.

In December 2014, we entered into a sublease agreement with an unrelated third party to occupy approximately 57,000 square feet of our research and office space. In connection with this sublease, we recognized a loss on sublease of \$9.3 million during the fourth quarter of 2014. In February 2017, we entered into an amendment to the sublease agreement to increase the subleased research and office space for an additional 9,328 square feet under the same term of the sublease, and recognized an additional loss on sublease of \$495,000. We expect to receive approximately \$1.8 million in future sublease income (excluding our subtenant's share of facilities operating expenses) through January 2018, the end of the initial term of the sublease. During the second quarter of 2017 and pursuant to the terms of the sublease agreement, our sublessee exercised its right to extend the sublease term for another five years through 2023 at a monthly rate equal to the amount we will pay our landlord.

We record rent expense on a straight-line basis for our lease, net of sublease income. For our sublease arrangement which we classified as an operating lease, our loss on the sublease was comprised of the present value of

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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 six months ended June 30, 2017 were as follows (in thousands):

Balance at January 1, 2017	\$ 3,460
Increase in deferred liability	495
Accretion of deferred liability	108
Amortization of deferred liability	(1,867)
Balance at June 30, 2017	\$ 2,196

## 12. Equity Offerings

In February 2017, we completed an underwritten public offering in which we sold 23,000,000 shares of our common stock pursuant to an effective registration statement at a price to the public of \$2.00 per share. We received net proceeds of approximately \$43.0 million, net of underwriting discounts and commissions and offering expenses.

In August 2015, we entered into a Controlled Equity Offering<sup>SM</sup> Sales Agreement (Original 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. As of September 30, 2016, all the shares under the Original Sales Agreement have been fully sold. In May 2017, we entered into an Amendment No. 1 (Amended Sales Agreement) to the Controlled Equity Offering<sup>SM</sup> Sales Agreement pursuant to which we may offer and sell, through Cantor, additional shares of our common stock, up to an aggregate offering price of \$40.0 million. These additional shares are in addition to the shares of common stock sold under the Original Sales Agreement. All sales of our common stock will be made pursuant to a shelf registration statement filed by us in May 2015 and declared effective by the Securities and Exchange Commission (SEC) in July 2015. Cantor is acting as our sole sales agent for any sales made under the Amended Sales Agreement for a low single-digit commission on gross proceeds. The common stock is sold at prevailing market prices at the time of the sale. Unless otherwise terminated earlier, the Amended Sales Agreement continues until all shares available under the agreement have been sold. During the six months ended June 30, 2017, 1,209,027 shares of common stock were sold under the Amended Sales Agreement, with an aggregate net proceeds of \$3.1 million. As of June 30, 2017, we had approximately \$36.6 million of remaining common stock registered for sale under the Amended 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, 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 third quarter of 2016 of approximately \$5.8 million, which included \$5.0 million of severance costs 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 June 30, 2017, the remaining accrued restructuring cost of \$218,000 related to COBRA benefits and outplacement costs and is classified under Accrued Compensation in 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, 2016. Operating results for the three and six months ended June 30, 2017 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 biotechnology company dedicated to discovering, developing and commercializing novel small molecule drugs that significantly improve the lives of patients with immune and hematological disorders, cancer and rare diseases. 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 have submitted and the FDA has accepted for review, an NDA for fostamatinib in patients with chronic or persistent immune thrombocytopenia (ITP). In addition, Rigel has product candidates in development with partners BerGenBio AS, Daiichi Sankyo and Aclaris Therapeutics.

Since inception, we have financed our operations primarily through the sale of equity securities, and contract payments under our collaboration agreements. Our research and development activities, including preclinical studies and clinical trials, consume substantial amounts of capital. As of June 30, 2017, we had approximately \$82.3 million in cash, cash equivalents and short term investments. During the six months ended June 30, 2017, we received an aggregate of \$3.6 million payments pursuant to our agreements with our collaborative partners, \$3.1 million under the Amended Sales Agreement, and approximately \$2.5 million of sublease income and reimbursements under a sublease agreement with an unrelated third party. In February 2017, we completed an underwritten public offering in which we sold 23,000,000 shares of our common stock pursuant to an effective registration statement at a price to the public of \$2.00 per share. We received net proceeds of approximately \$43.0 million, net of underwriting discounts and commissions and offering expenses. We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including the preparation for potential commercial launch of TAVALISSETM (fostamatinib disodium) in ITP in the U.S., through at least the next 12 months. We also continue to evaluate ex-U.S. partnerships for fostamatinib and other partnering opportunities across our pipelines.



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Our revenues have consisted primarily of revenues from sponsored research and license agreements with our corporate collaborators. We earned contract revenues from collaborations of \$3.6 million during the six months ended June 30, 2017 which is comprised primarily of the payment amounting to \$3.3 million we received from BerGenBio pursuant to advancing a licensed AXL kinase inhibitor to Phase 2 clinical study.

Within our product development portfolio, our most advanced program is fostamatinib in ITP. We submitted an NDA for fostamatinib in ITP in April 2017, which was accepted by the FDA in June 2017, with an action date for the FDA to complete its review by April 17, 2018, under the Prescription Drug User Fee Act (PDUFA). On April 27, 2017, we announced that we received the conditional acceptance by the U.S Food & Drug Administration (FDA) of the proprietary name TAVALISSETM for fostamatinib disodium, our lead investigational product candidate.

### TAVALISSETM (fostamatinib disodium)—Immune Thrombocytopenic Purpura

**Disease background.** Chronic ITP affects an estimated 65,000 adult patients 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 fostamatinib program.** Taken in tablet form, fostamatinib blocks the activation of SYK inside immune cells. ITP is typically characterized by the body producing 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 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 were diagnosed with persistent or chronic ITP, and had blood platelet counts consistently below 30,000 per microliter of blood. Two-thirds of the subjects received fostamatinib orally at 100 mg bid (twice daily) and the other third received placebo on the same schedule. Subjects were expected to remain on treatment for up to 24 weeks. At week four of treatment, subjects who failed to meet certain platelet count and met certain tolerability thresholds could have their dosage of fostamatinib (or corresponding placebo) increased to 150 mg bid. The primary efficacy endpoint of this program was 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 for fostamatinib for the treatment of ITP. On April 1, 2016, we announced that we completed enrollment

in the FIT Phase 3 clinical program.

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 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 datasets for the FIT 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 datasets, 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

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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 the FIT studies were given the option to enroll in a long-term open-label extension study and receive treatment with fostamatinib, also a Phase 3 trial. A total of 123 patients had enrolled in this study. All the patients who responded to fostamatinib in the FIT studies and enrolled in the long-term open-label extension study maintained a median platelet count of 106,500/uL at a median of 16 months. In addition, there were 44 placebo non-responders that enrolled in the long-term open-label extension study. 41 of these patients had at least 12 weeks of follow-up. Of those, 9 patients (22%) have achieved a prospectively defined stable platelet response, which is statistically significant ( $p=0.0078$ ) and similar to the response rate fostamatinib achieved in the parent studies.

In the combined dataset of both stable and intermediate responders for the FIT studies, the response rate was 29% (29/101), compared to 2% (1/49) for placebo ( $p<0.0001$ ). A stable response was defined as a patient achieving platelet counts of greater than 50,000/uL on more than 4 of the 6 visits between weeks 14 and 24. In the post-study analysis performed by the Company, an intermediate response was defined to include patients achieving at least two consecutive median platelet counts over 50,000/uL during the trial without rescue, but who did not otherwise meet the stable response criteria.

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. We submitted an NDA for TAVALISSE™ in ITP in April 2017, which was accepted by the FDA in June 2017, with an action date for the FDA to complete its review by April 17, 2018, under the PDUFA.

## Commercial activities, including sales and marketing

We intend to commercialize fostamatinib disodium in ITP in the U.S., in 2018, subject to FDA approval, on our own. We plan to enter into partnership with third parties to commercial fostamatinib in Europe and Asia. A significant portion of our operating expenses in the second half of 2017 and 2018 will be related to our commercialization activities. Specifically, our marketing and sales efforts will be focused on targeting approximately 3,000 hematologists and hematologist-oncologists, who treat chronically managed ITP patients. We will continue to hire and recruit experienced commercial professionals, including sales management, marketing, and market access professionals to support these efforts.

## Competitive Landscape

Our industry is 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.

Currently, corticosteroids remain the most common first line therapy for ITP, occasionally in conjunction with intravenous immunoglobulin (IVIg) or anti-Rh(D) as added agents to help further augment platelet count recovery, particularly in emergency situations. However, it has been estimated that frontline agents lead to durable remissions in only a small percentage of newly-diagnosed adults with ITP. Moreover, concerns with steroid-related side effects often restrict therapy to approximately 4 weeks. As such, many patients progress to persistent or chronic ITP, requiring other forms of therapeutic intervention.

Other approaches to treat ITP are varied in their mechanism of action, and there is no consensus about their consequential use, according to the most recent ITP guideline from the American Society of Hematology. Options include splenectomy, thrombopoietin receptor agonists (TPO-Rs) and various immunosuppressants (such as rituximab). The response rate criteria of the abovementioned options vary, precluding a comparison of response rates for individual therapies.

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Even with the above treatment options, a significant number of patients remain severely thrombocytopenic for long durations and are subject to risk of spontaneous or trauma-induced hemorrhage. The addition of fostamatinib to the treatment options makes sense since it has a different mechanism of action than the thrombopoietin (TPO) agonists. Fostamatinib is a potent and relatively selective SYK inhibitor, and its inhibition of Fc receptors and B-cell receptors signaling pathways make it a potentially broad immunomodulatory agent.

Other products that are commercially-approved to increase platelet production through binding and TPO receptors on megakaryocyte precursors include PROMACTA® (Novartis), Nplate® (Amgen, Inc.), TPIAO (3SBio Inc.) and MULPETA® (Shionogi, Inc.).

## Clinical Stage Programs

### 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 fostamatinib 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. In January 2017, we announced that the first cohort in the Phase 2 study of fostamatinib in IgAN was completed in various centers throughout Asia, the U.S. and Europe. This cohort evaluated the efficacy, safety, and tolerability of the lower dose of fostamatinib (100mg BID, n=26; placebo n=12) as measured by change in proteinuria, renal function, and histology (comparing the pre- and post-study renal biopsies). The primary efficacy endpoint was the mean change in proteinuria from baseline at 24 weeks. The study found that at 24 weeks, fostamatinib was well tolerated with a good safety profile. The initial data suggest a trend towards a greater reduction in proteinuria in fostamatinib treated patients relative to placebo. The Phase 2 study for the second cohort is currently enrolling patients. We expect that the second cohort, evaluating a higher dose of fostamatinib (150 mg BID) for IgAN, will finish enrollment in 2017, with results in 2018.

### 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 fostamatinib program. Our Phase 2 clinical trial is currently enrolling patients with AIHA. 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 by the end of 2017. With this data, we will evaluate the best way forward and potentially an expedited path for pursuing AIHA.

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### 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 potentially treat cytokine-driven autoimmune and inflammatory diseases. We have selected a molecule from our IRAK program for preclinical development. The molecule is differentiated in that it inhibits both the IRAK 1 and IRAK 4 signaling pathways, with potential to treat autoimmune and inflammatory diseases such as lupus, gout, psoriatic arthritis and multiple sclerosis. We expect to initiate clinical trials in the first half of 2018.

### 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 during the research term was 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 \$533.3 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 \$146.4 million relates to the achievement of development events, up to \$345.6 million relates to the achievement of regulatory events and up to \$41.3 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 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



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that these deliverables were a single unit of accounting as the license did 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 and was fully amortized in September 2016, the end of the research term. We believed that straight-line recognition of this revenue was appropriate as the research was performed ratably over the research period. During the three and six months ended June 30, 2016, we recognized revenue of \$4.8 million and \$9.7 million, respectively, relating to the upfront payment and \$95,000 and \$290,000, respectively, relating to the research activities we performed. 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. As of September 30, 2016, all deliverables under the agreement have been delivered.

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 February 2017, we received \$3.3 million from BerGenBio as a result of BerGenBio advancing BGB324, an AXL kinase inhibitor licensed under the agreement, to a Phase 2 clinical study. 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 \$3.3 million in 2017 and \$3.7 million in 2016, triggered by the above time-based and contingent events were recognized as revenue in the first quarter of 2017 and second quarter of 2016, respectively.

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 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, costs related to the submission and management of our NDA, 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.

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

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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 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.

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

	Three Months Ended		Six Months Ended		From January 1, 2007*
	2017	2016	2017	2016	to June 30, 2017
Categories:					
Research	\$ 2,500	\$ 5,814	\$ 5,127	\$ 12,414	\$ 221,535
Development	6,939	7,642	14,471	16,070	328,704
Other	2,085	4,012	4,302	7,157	226,253
	\$ 11,524	\$ 17,468	\$ 23,900	\$ 35,641	\$ 776,492

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

“Other” expenses mainly represent allocated facilities costs of approximately \$1.7 million and \$2.6 million for the three months ended June 30, 2017 and 2016, respectively, and allocated stock-based compensation expenses of approximately \$336,000 and \$1.4 million for the three months ended June 30, 2017 and 2016, respectively. For the six months ended June 30, 2017 and 2016, allocated facilities costs were approximately \$3.6 million and \$5.1 million, respectively, and allocated stock-based compensation expenses were approximately \$696,000 and \$2.1 million, respectively.

For the three and six months ended June 30, 2017, a major portion of our total research and development expense was associated with salaries of our research and development personnel, our ITP, IRAK and IgAN programs, and allocated facilities costs. For the three and six months ended June 30, 2016, 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 further discussion on research and development activities, see “Research and Development Expense” under “Results of Operations” below.

## Results of Operations

Three and Six Months Ended June 30, 2017 and 2016

### Revenues

	Three Months Ended June 30,		Aggregate Change	Six Months Ended June 30,		Aggregate Change
	2017	2016 (in thousands)		2017	2016 (in thousands)	
Contract revenues from collaborations	\$ —	\$ 8,594	\$ (8,594)	\$ 3,584	\$ 13,623	\$ (10,039)

There were no contract revenues from collaborations during the three months ended June 30, 2017. Contract revenues from collaborations of \$8.6 million during the three months ended June 30, 2016 were comprised of the amortization of the \$30.0 million upfront payment and FTE fees we earned from BMS of \$4.8 million and \$95,000, respectively, as well as the contingent payment amounting to \$3.7 million we received pursuant to our license agreement with BerGenBio.

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Contract revenues from collaborations of \$3.6 million during the six months ended June 30, 2017 is comprised primarily of the payment amounting to \$3.3 million we received from BerGenBio pursuant to advancing a licensed AXL kinase inhibitor to Phase 2 clinical study which we recognized as revenue in the first quarter of 2017. Contract revenues from collaborations of \$13.6 million during the six months ended June 30, 2016 were comprised of the amortization of the upfront payment and FTE fees we earned from BMS of \$9.7 million and \$290,000, respectively, as well as the contingent payment amounting to \$3.7 million we received from BerGenBio.

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		Aggregate Change	Six Months Ended		Aggregate Change
	June 30, 2017	2016 (in thousands)		June 30, 2017	2016 (in thousands)	
Research and development expense	\$ 11,524	\$ 17,468	\$ (5,944)	\$ 23,900	\$ 35,641	\$ (11,741)
Stock-based compensation expense included in research and development expense	\$ 336	\$ 1,410	\$ (1,074)	\$ 696	\$ 2,103	\$ (1,407)

The decreases in research and development expense for the three and six months ended June 30, 2017, compared to the same periods in 2016, were primarily due to the decreases in personnel costs, stock based compensation expense and facility costs as a result of the reduction in workforce in September 2016, and clinical trial costs primarily due to the completion of the pivotal Phase 3 clinical trials in ITP, partially offset by the increase in costs related to the submission of our NDA for fostamatinib in ITP.

We expect that our research and development expense will remain relatively consistent quarter over quarter during the remainder of 2017.

## General and Administrative Expense

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	Three Months Ended			Six Months Ended		
	June 30, 2017	2016 (in thousands)	Aggregate Change	June 30, 2017	2016 (in thousands)	Aggregate Change
General and administrative expense	\$ 7,820	\$ 4,774	\$ 3,046	\$ 15,230	\$ 9,197	\$ 6,033
Stock-based compensation expense included in general and administrative expense	\$ 764	\$ 604	\$ 160	\$ 1,359	\$ 1,349	\$ 10

The increases in general and administrative expense for the three and six months ended June 30, 2017, compared to the same periods in 2016, were primarily due to the commercial launch preparation costs, including personnel costs, incurred for a potential commercial launch of fostamatinib in ITP. We expect general and administrative expenses to continue to increase during the remainder of 2017 as we continue to prepare for a potential commercial launch of fostamatinib in ITP.

Gain on Disposal of Assets

	Three Months Ended			Six Months Ended		
	June 30, 2017	2016 (in thousands)	Aggregate Change	June 30, 2017	2016 (in thousands)	Aggregate Change
Gain on disposal of assets	\$ —	\$ —	\$ —	\$ 732	\$ —	\$ 732

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Gain on disposal of assets during the six months ended June 30, 2017 related to the proceeds from sale of our fully depreciated property and equipment in the first quarter of 2017.

## Interest Income

	Three Months Ended		Aggregate Change	Six Months Ended		Aggregate Change
	June 30, 2017	2016 (in thousands)		June 30, 2017	2016 (in thousands)	
Interest income	\$ 197	\$ 115	\$ 82	\$ 353	\$ 218	\$ 135

Interest income results from our interest-bearing cash and investment balances. The increases in interest income for the three and six months ended June 30, 2017, as compared to the same periods in 2016 were 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. 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 sublease agreement (including the determination of discount rate used), 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, 2016, as filed with the SEC.

## Recent Accounting Pronouncements

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. 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. The adoption of ASU No. 2014-09 may have a material effect on our financial statements. To date, our revenues have been derived from license and collaboration agreements. The consideration we are eligible to receive under these agreements includes upfront payments, progress dependent contingent payments on events achieved by our collaboration partners, and royalties on net sales of products sold by such partners under the agreements. Each license and collaboration agreement is unique and will need to be assessed separately under the five-step process of the new standard. ASU No. 2014-09 differs from the current accounting standard in many respects, such as in the accounting for variable consideration, including milestone payments or contingent payments. Under our current accounting policy, we recognize contingent



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payments as revenue in the period that the payment-triggering event occurred or is achieved. However, under the new accounting standard, it is possible to start to recognize contingent payments before the payment-triggering event is completely achieved, subject to management's assessment of whether it is probable that a significant reversal in the amount of cumulative revenue recognized will not occur when the uncertainty associated with the variable consideration is subsequently resolved. We have performed a preliminary assessment of the impact of the new standard on our active license and collaboration agreements. Based on this preliminary assessment, we expect that the timing of recognition of certain future milestone payments may be impacted depending on the assessed probability of achievement for these milestones as of the date of adoption.

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. We adopted ASU No. 2016-09 on January 1, 2017. Under this guidance, on a prospective basis, companies will no longer record excess tax benefits and certain tax deficiencies in additional paid-in capital. Instead, they will record all excess tax benefits and tax deficiencies as income tax expense or benefit in the income statement. In addition, the guidance eliminates the requirement that excess tax benefits be realized before companies can recognize them. The ASU requires a cumulative-effect adjustment for previously unrecognized excess tax benefits in opening retained earnings in the annual period of adoption. Upon adoption, we recognized additional excess tax benefit as a deferred tax asset with a corresponding increase to our deferred tax asset valuation allowance, which did not result in a net impact to retained earnings. Additionally, as provided for under this new guidance, we elected to account for forfeitures as they occur. The adoption of this aspect of the guidance did not have 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 and contract payments under our collaboration agreements. 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 June 30, 2017, we had approximately \$82.3 million in cash, cash equivalents and short term investments, as compared to approximately \$74.8 million as of December 31, 2016, an increase of approximately \$7.5 million. The increase was primarily attributable to the completed underwritten public offering whereby we received approximately \$43.0 million, net of underwriting discounts and commissions and offering expenses, partially offset by the payments associated with funding our operating expenses during the six months ended June 30, 2017. In February 2017, we received a payment from BerGenBio of \$3.3 million pursuant to our exclusive license agreement which we signed in June 2011. In May 2017, we entered into an Amended Sales Agreement with Cantor, pursuant to which we may offer and sell, through Cantor additional shares of our common stock, up to an aggregate offering price of \$40.0 million. During the six months ended June 30, 2017, 1,209,027 shares of common stock were sold under the Amended Sales Agreement, with aggregate net proceeds of \$3.1 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. This sublease agreement was amended in February 2017 to sublease additional research and office space. During the six months ended June 30, 2017, we received approximately \$2.5 million of sublease income and reimbursements. We expect to receive approximately \$1.8 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. We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including the preparation for potential commercial launch of fostamatinib in ITP in

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the U.S., through at least the next 12 months. 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 successful regulatory approval of our recently submitted NDA;
- the outcome of any potential FDA advisory committee meetings held for any of our product candidates;
- 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 as we prepare for a potential commercial launch of fostamatinib in ITP;
- 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.

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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 six months ended June 30, 2017 and 2016, 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

	Six Months Ended June 30,	
	2017	2016
	(in thousands)	
Net cash provided by (used in):		
Operating activities	\$ (40,232)	\$ (40,854)
Investing activities	8,206	12,377
Financing activities	46,985	9,967
Net increase (decrease) in cash and cash equivalents	\$ 14,959	\$ (18,510)

Net cash used in operating activities was approximately \$40.2 million for the six months ended June 30, 2017, compared to approximately \$40.9 million for the six months ended June 30, 2016. Net cash used in operating activities for the six months ended June 30, 2017 was primarily due to the cash payments related to our research and development programs, partially offset by the \$3.3 million payment we received from BerGenBio. Net cash used in operating activities for the six months ended June 30, 2016 was primarily due to the cash payments related to our research and development programs, partially offset by the \$3.7 million payment we received from BerGenBio. The timing of cash requirements may vary from period to period depending on our research and 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 \$8.2 million for the six months ended June 30, 2017, compared to approximately \$12.4 million for the six months ended June 30, 2016. 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 \$55,000 for the six months ended June 30, 2017, compared to approximately

\$546,000 for the same period in 2016.

Net cash provided by financing activities was approximately \$47.0 million for the six months ended June 30, 2017, compared to approximately \$10.0 million for the six months ended June 30, 2016. Net cash provided by financing activities for the six months ended June 30, 2017 consisted of net proceeds of \$43.0 million from issuance of common stock pursuant to the underwritten public offering, \$3.6 million from issuance of shares under our Amended Sales Agreement with Cantor and proceeds from exercise of stock options and participation in the Purchase Plan. Net cash provided by financing activities for the six months ended June 30, 2016 consisted of net proceeds of \$9.3 million from issuance of shares under our Original Sales Agreement, as well as proceeds from exercise of outstanding options and issuance of shares under the Purchase Plan.

#### Off-Balance Sheet Arrangements

As of June 30, 2017, we had no off-balance sheet arrangements (as defined in Item 303(a)(4)(ii) of Regulation S-K under the Exchange Act).

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## 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 (CRO). 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.

We have agreements with certain CROs to conduct our clinical trials. The timing of payments for any amounts owed under the respective agreements will depend on various factors including, but not limited to, patient enrollment and other progress of the clinical trial. We can terminate these agreements at any time, and if terminated, we would not be liable for the full amount of the respective agreements. Instead, we will be liable for services through the termination date plus certain cancellation charges, if any, as defined in each of the respective agreements. In addition, these agreements may, from time to time, be subjected to amendments as a result of any change orders executed by the parties.

As of June 30, 2017, 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
	(in thousands)				
Facilities lease (1)	\$ 9,453	\$ 9,453	\$ —	\$ —	\$ —

(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 \$1.8 million over the remaining term of the sublease through January 2018.

We are also subject to claims related to the patent protection of certain of our technologies, as well as purported securities class action lawsuit, other litigations, and other contractual agreements. We are required to assess the likelihood of any adverse judgments or outcomes to these matters as well as potential ranges of probable losses. A determination of the amount of reserves required, if any, for these contingencies is made after careful analysis of each individual matter.

In the first quarter of 2017, we entered into a consulting agreement with a third party, pursuant to which we may be required to pay amounts ranging from \$1.5 million to \$4.0 million if certain future regulatory milestone events occur. As of June 30, 2017, we do not consider any of the future regulatory milestone events as probable of occurring.

As such, no expense was recognized for the three and six months ended June 30, 2017.

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

During the six months ended June 30, 2017, 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, 2016.

### 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.

**Changes in Internal Controls.** There were no changes in our internal control over financial reporting that occurred during the quarter ended June 30, 2017 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.



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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 7, 2017.

If our recently submitted NDA is not approved 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 FIT 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 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. Additionally, we have announced updates on the results of the ongoing open label long term extension study of fostamatinib in ITP. We submitted an NDA for fostamatinib in ITP in April 2017. In June 2017, we announced that the FDA accepted our NDA submission for the use of fostamatinib in patients with chronic or persistent ITP. Our NDA submission included, among others, data on both FIT trials as well as the ongoing open label long-term extension study, a number of post-study analyses including an overall response rate for the FIT studies, which combined stable and intermediate responders, and a large safety database from previous trials of fostamatinib. Although the FDA recently accepted our NDA submission, given that our second FIT trial did not meet its primary endpoint, there is a risk that the FDA may not approve the submission for any reason as the FDA has substantial discretion in evaluating the results of our clinical trials. For example, notwithstanding our view to the contrary, the FDA may determine that the efficacy data and/or safety data from our earlier clinical trials do not support approval of our NDA. Clinical data often is susceptible to varying interpretations and many companies that have believed that their products performed satisfactorily in clinical trials have nonetheless failed to obtain FDA approval for their products. The FDA may disagree with our trial design and our interpretation of data from nonclinical studies and clinical trials. Upon the FDA's review of the data in our NDA, it may request that we conduct additional analyses and, if it believes that such data are not satisfactory, could advise us that fostamatinib is not approvable with the filed data package. Any such decision 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. If the FDA does suggest that we need to conduct additional trials, this would add significant costs before we seek regulatory approval of our product

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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.

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 also continue to evaluate ex-U.S. partnerships for fostamatinib and other partnering opportunities across our pipeline. We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including the preparation for potential commercial launch of fostamatinib in ITP in the U.S., through at least the next 12 months. 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. In addition, we have a significant number of stock options outstanding. To the extent that outstanding stock options have been or may be exercised or other shares issued, our stockholders may experience further dilution. Further, we may choose to raise additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans, including through an “at-the-market” equity offering program. 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 successful regulatory approval of our recently submitted NDA;
- the outcome of any potential FDA advisory committee meetings held for any of our product candidates;
- the progress and success of 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;

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- the costs to build and expand our sales, marketing and distribution capabilities as we prepare for a potential commercial launch of fostamatinib in ITP;
- 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 and development programs, to reduce personnel and operating expenses, 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, a significant portion 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.

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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.

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. Further, in August 2014, we 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.



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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;
- 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

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have to provide additional information or analysis, or conduct additional clinical trials, before receiving approval to market product candidates.

Enacted or future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

Third-party payors, whether domestic or foreign, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In both the United States and certain foreign jurisdictions, there have been, and we expect there will continue to be, a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, collectively, the Affordable Care Act, was enacted.

The Affordable Care Act and its implementing regulations, among other things, substantially change the way healthcare is financed by both governmental and private insurers in the U.S. Among the provisions of the Affordable Care Act, those of the greatest impact on the pharmaceutical and biotechnology industry include a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for certain drugs that are inhaled, infused, instilled, implanted or injected; increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program; extended the Medicaid Drug Rebate Program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations; subjected manufacturers to new annual fees and taxes for certain branded prescription drugs; provided incentives to programs that increase the federal government's comparative effectiveness research and established a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D.

Other legislative changes have been proposed and adopted in the U.S. since the Affordable Care Act was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction, was unable to reach its required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year, which went into effect in April 2013, and will remain in effect through 2024 unless additional Congressional action is taken. In January 2013, former President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Legislative changes to the Affordable Care Act remain possible and appear likely in the 115th U.S. Congress and under the Trump Administration. We expect that the Affordable Care Act, as currently enacted or as it may be

amended or repealed in the future, and other healthcare reform measures that may be adopted in the future, could have a material adverse effect on our industry generally and on our ability to successfully commercialize our product candidates, if approved. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we or our collaborators are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or our collaborators are not able to maintain regulatory compliance, our product candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability, which would adversely affect our business.

Our activities and drugs will still be subject to extensive postmarketing regulation if approved. \*

Following regulatory approval of any of our drug candidates, we and our collaborators will be subject to ongoing obligations and continued regulatory review from the FDA and other applicable regulatory agencies, such as continued adverse event reporting requirements. There may also be additional postmarketing obligations imposed by the FDA or other regulatory agencies. These obligations may result in significant expense and limit the ability to commercialize such drugs.

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The FDA or other regulatory agencies may also require that the sponsor of the NDA or foreign equivalent, as applicable, conduct additional clinical trials to further assess approved drugs after approval under a post-approval commitment. Such additional studies may be costly and may impact the commercialization of the drug. Along with being costly and time consuming, a delay or unfavorable results from these trials could negatively impact market acceptance of our product candidates; limit the revenues we generate from sales; result in withdrawal from the market; and result in litigation.

The FDA or other regulatory agencies may also impose significant restrictions on the indicated uses for which a drug may be marketed. Additionally, the FDA may require a Risk Evaluation and Mitigation Strategies, or REMS, study, including in connection with a drug's approval, to help ensure that the benefits of the drug outweigh its risks. A REMS may be required to include various elements, such as a medication guide or patient package insert, a communication plan to educate healthcare providers of the drug's risks, limitations on who may prescribe or dispense the drug, requirements that patients enroll in a registry or undergo certain health evaluations or other measures that the FDA deems necessary to ensure the safe use of the drug.

With regard to any of drug that receives regulatory approval, the labeling, packaging, adverse event reporting, storage, advertising and promotion for the drug will be subject to extensive regulatory requirements. We and the manufacturers of our products are also required to comply with Good Manufacturing Practices (cGMP) regulations, which include requirements relating to quality control and quality assurance, as well as the corresponding maintenance of records and documentation. Further, regulatory agencies must approve these manufacturing facilities before they can be used to manufacture our products, and these facilities are subject to ongoing regulatory inspections. In addition, regulatory agencies subject a drug, its manufacturer and the manufacturer's facilities to continual review and inspections. The subsequent discovery of previously unknown problems with a drug, including adverse events of unanticipated severity or frequency, or problems with the facility where the drug is manufactured, may result in restrictions on the marketing of that drug, up to and including withdrawal of the drug from the market. In the United States, the DEA and comparable state-level agencies also heavily regulate the manufacturing, holding, processing, security, recordkeeping and distribution of drugs that are considered controlled substances, and the DEA periodically inspects facilities for compliance with its rules and regulations.

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;
- 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;

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- 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 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,



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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. We currently use one manufacturer of fostamatinib. 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 cGMP. In addition, we rely on our third-party 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, which could have a material adverse effect on sales, results of operations and financial condition. If we were required to transfer manufacturing processes to other third-party manufacturers and we were able to identify an alternative manufacturer, we would still need to satisfy various regulatory requirements. Satisfaction of these requirements could cause us to experience significant delays in receiving an adequate supply of our products and products in development and could be costly. Moreover, we may not be able to transfer processes that are proprietary to the manufacturer, if any. 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 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 NDA, to market the same drug 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 \$35.5 million for the six months ended June 30, 2017. 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 June 30, 2017, we had an accumulated deficit of approximately \$1.1 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. As of June 30, 2017, we had 56 pending patent applications and 357 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.

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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 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.

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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 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.

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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.

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 ability to compete successfully will depend, in part, on our ability to:

- identify and validate targets;
- discover candidate drug compounds that interact with the targets we identify;
- attract and retain scientific and product development personnel;
- obtain patent or other proprietary protection for our new drug compounds and technologies; and
- enter commercialization agreements for our new drug compounds.

Our stock price may be volatile, and our stockholders' investment in our common 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;



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- 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.

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. In March 2017, the U.K. government initiated the withdrawal process, with the U.K. scheduled to exit the E.U. by April 2019. 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.

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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 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.

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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 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. To date, we have \$70.6 million remaining under such universal shelf registration statement. If we or our stockholders sell, or if it is perceived that we or they will sell, substantial amounts of our common stock (including including pursuant to our Amended Sales Agreement with Cantor or 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, including pursuant to our Amended Sales Agreement with Cantor, 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;

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- 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+	2017 Cash Incentive Plan. (6)
10.2+	Rigel Pharmaceuticals, Inc. Inducement Plan, as amended. (7)
10.3+	Form of Stock Option Grant Notice, Option Agreement and Notice of Exercise under the Rigel Pharmaceuticals, Inc. Inducement Plan. (8)
10.4	Amendment No. 1 to the Controlled Equity Offering <sup>SM</sup> Sales Agreement, by and between Rigel Pharmaceuticals, Inc. and Cantor Fitzgerald & Co., dated as of May 30, 2017. (9)
10.5#	Rigel Pharmaceuticals, Inc. 2011 Equity Incentive Plan, as amended.
10.6#	Rigel Pharmaceuticals, Inc. 2000 Non-Employee Directors' Stock Option Plan, as amended.
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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+ Management contract or compensatory plan.

#Filed herewith

- (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.

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- (6) Filed as an exhibit to Rigel's Current Report on Form 8-K (No. 000-29889) filed on February 8, 2017, and incorporated herein by reference.
  
- (7) Filed as an exhibit to Rigel's Annual Report on Form 10-K (No. 000-29889), filed on March 7, 2017, and incorporated herein by reference.
  
- (8) Filed as an exhibit to Rigel's Current Report on Form 8 K (No. 000 29889) filed on October 11, 2016, and incorporated herein by reference.
  
- (9) Filed as an exhibit to Rigel's Current Report on Form 8 K (No. 000 29889) filed on May 30, 2017, and incorporated herein by reference.

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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: August 1, 2017

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

Date: August 1, 2017

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INDEX TO EXHIBITS

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+	2017 Cash Incentive Plan. (6)
10.2+	Rigel Pharmaceuticals, Inc. Inducement Plan, as amended. (7)
10.3+	Form of Stock Option Grant Notice, Option Agreement and Notice of Exercise under the Rigel Pharmaceuticals, Inc. Inducement Plan. (8)
10.4	Amendment No. 1 to the Controlled Equity OfferingSM Sales Agreement, by and between Rigel Pharmaceuticals, Inc. and Cantor Fitzgerald & Co., dated as of May 30, 2017. (9)
10.5#	Rigel Pharmaceuticals, Inc. 2011 Equity Incentive Plan, as amended.
10.6#	Rigel Pharmaceuticals, Inc. 2000 Non-Employee Directors' Stock Option Plan, as amended.
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

+ Management contract or compensatory plan.

#Filed herewith

- (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) Filed as an exhibit to Rigel's Current Report on Form 8-K (No. 000-29889) filed on February 8, 2017, and incorporated herein by reference.

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- (7) Filed as an exhibit to Rigel's Annual Report on Form 10-K (No. 000-29889), filed on March 7, 2017, and incorporated herein by reference.
  
- (8) Filed as an exhibit to Rigel's Current Report on Form 8 K (No. 000 29889) filed on October 11, 2016, and incorporated herein by reference.
  
- (9) Filed as an exhibit to Rigel's Current Report on Form 8 K (No. 000 29889) filed on May 30, 2017, and incorporated herein by reference.