RIGEL PHARMACEUTICALS INC
Form 10-Q
May 01, 2018
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

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 MARCH 31, 2018

OR

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

FOR THE TRANSITION PERIOD FROM TO

Commission File Number 0-29889

Rigel Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

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

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

(650) 624-1100

(Registrant's telephone number, including area code)

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 April 26, 2018, there were 163,580,297 shares of the registrant's Common Stock outstanding.

Table of Contents

RIGEL PHARMACEUTICALS, INC.

QUARTERLY REPORT ON FORM 10-Q

FOR THE QUARTERLY PERIOD ENDED MARCH 31 2018

INDEX

Table of Contents

PART I. FINANCIAL INFORMATION

Item 1.Financial Statements

RIGEL PHARMACEUTICALS, INC.

CONDENSED BALANCE SHEETS

(In thousands)

	March 31, 2018 (unaudited)	December 31, 2017(1)
Assets		
Current assets:		
Cash and cash equivalents	$ 40,125	$ 38,290
Short-term investments	54,175	77,461
Prepaid and other current assets	2,320	1,682
Total current assets	96,620	117,433
Property and equipment, net	1,439	875
Other assets	754	803
	$ 98,813	$ 119,111
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,623	$ 2,636
Accrued compensation	4,746	7,059
Accrued research and development	5,872	5,028
Other accrued liabilities	5,549	3,330
Deferred liability – sublease, current portion	—	284
Total current liabilities	18,790	18,337
Long-term portion of deferred rent	178	90
Other long-term liabilities	38	38
Commitments		
Stockholders' equity:		
Preferred stock	—	—

Common stock	148	147
Additional paid-in capital	1,242,985	1,239,435
Accumulated other comprehensive loss	(87)	(82)
Accumulated deficit	(1,163,239)	(1,138,854)
Total stockholders' equity	79,807	100,646
	$ 98,813	$ 119,111

(1) The balance sheet at December 31, 2017 has been derived from the audited financial statements included in Rigel's Annual Report on Form 10-K for the year ended December 31, 2017.

See Accompanying Notes.

Table of Contents

RIGEL PHARMACEUTICALS, INC.

CONDENSED STATEMENTS OF OPERATIONS

(In thousands, except per share amounts)

(unaudited)

	Three Months Ended March 31,	
	2018	2017
Contract revenues from collaborations	$ —	$ 3,584
Costs and expenses:		
Research and development	11,242	12,376
General and administrative	13,492	7,410
Total costs and expenses	24,734	19,786
Loss from operations	(24,734)	(16,202)
Interest income	349	156
Gain on disposal of assets	—	732
Net loss	$ (24,385)	$ (15,314)
Net loss per share, basic and diluted	$ (0.17)	$ (0.13)
Weighted average shares used in computing net loss per share, basic and diluted	147,114	113,598

See Accompanying Notes.

Table of Contents

RIGEL PHARMACEUTICALS, INC.

CONDENSED STATEMENTS OF COMPREHENSIVE LOSS

(In thousands)

(unaudited)

	Three Months Ended March 31,	
	2018	2017
Net loss	$ (24,385)	$ (15,314)
Other comprehensive income (loss):		
Net unrealized loss on short-term investments	(5)	(11)
Comprehensive loss	$ (24,390)	$ (15,325)

See Accompanying Notes.

Table of Contents

RIGEL PHARMACEUTICALS, INC.

CONDENSED STATEMENTS OF CASH FLOWS

(In thousands)

(unaudited)

	Three Months Ended March 31,	
	2018	2017
Operating activities		
Net loss	$ (24,385)	$ (15,314)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	1,540	955
Gain on disposal of assets	—	(732)
Loss on sublease	—	495
Depreciation and amortization	113	126
Net amortization of premium on short-term investment	(134)	(33)
Changes in assets and liabilities:		
Prepaid and other current assets	(638)	65
Other assets	533	33
Accounts payable	(493)	(4,385)
Accrued compensation	(2,313)	(1,464)
Accrued research and development	844	349
Other accrued liabilities	2,184	439
Deferred rent and other long term liabilities	(645)	(1,296)
Net cash used in operating activities	(23,394)	(20,762)
Investing activities		
Purchases of short-term investments	(5,235)	(33,385)
Maturities of short-term investments	28,650	32,544
Proceeds from disposal of assets	—	732
Capital expenditures	(197)	(41)
Net cash provided by (used in) investing activities	23,218	(150)
Financing activities		
Net proceeds from issuances of common stock upon exercise of options and participation in employee stock purchase plan	2,011	341
Proceeds from sale and issuance of common stock, net of offering costs	—	43,083
Net cash provided by financing activities	2,011	43,424
Net increase in cash and cash equivalents	1,835	22,512
Cash and cash equivalents at beginning of period	38,290	17,632
Cash and cash equivalents at end of period	$ 40,125	$ 40,144

See Accompanying Notes.

Table of Contents

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 a biotechnology company dedicated to discovering, developing and providing novel small molecule drugs that significantly improve the lives of patients with immune and hematologic 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, 2017 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, 2017.

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.Summary of Significant Accounting Policies

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (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. 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. 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 previous accounting policy, we recognized 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 adopted this new standard on January 1, 2018 using the modified retrospective approach. Because all of the performance obligations for our outstanding collaboration agreements had been completed prior to December 31, 2017, we did not record any adjustment on the opening balance of Accumulated Deficit as of January 1, 2018.

Table of Contents

Under this new guidance, the Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for those goods or services. To determine whether arrangements are within the scope of this new guidance, the Company performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the Company satisfies its performance performance obligation. The Company applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of this new guidance, we assess the goods or services promised within each contract and identify, as a performance obligation, and assess whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

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.

Contingencies

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 milestone events occur. As of March 31, 2018, we concluded that certain future milestone events are probable of achievement. Accordingly, we recorded a contingent liability of $3.0 million as of March 31, 2018. Of this amount, $1.5 million was recognized as expense during the three months ended March 31, 2018 and was recorded as part of general and administrative expenses in the Statements of Operations.

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. We 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 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

Table of Contents

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 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 March 31,	
	2018	2017
Outstanding stock options	20,985	21,756
Purchase Plan	94	63
Total	21,079	21,819

6.Stock-based Compensation

Total stock-based compensation expense related to all of our share-based payments that we recognized for the three months ended March 31, 2018 and 2017 were as follows (in thousands):

	Three Months Ended	
	2018	2017
General and administrative	$ 940	$ 595
Research and development	600	360
Total stock-based compensation expense	$ 1,540	$ 955

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

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

Table of Contents

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 options.

- 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.

The following table summarizes the weighted-average assumptions relating to options granted pursuant to our equity incentive plans for the three months ended March 31, 2018 and 2017:

	Three Months Ended March 31,			
	2018		2017	
Risk-free interest rate	2.7	%	2.3	%
Expected term (in years)	6.7		6.8	
Dividend yield	0.0	%	0.0	%
Expected volatility	64.6	%	62.9	%

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 3,396,975 shares of common stock during the three months ended March 31, 2018 with a grant-date weighted-average fair value of $2.79 per share. As of March 31, 2018, we have 2,672,500 shares related to outstanding performance-based stock option awards with a grant date fair value of $5.7 million which will vest upon achievement of certain corporate performance-based milestones. Of this amount, 1,160,000 shares related to performance-based stock option awards wherein the achievement of the corresponding corporate-based milestones was probable. Accordingly, we recognized $1.6 million in stock-based compensation expense, of which $402,000 was recorded in the first quarter of 2018. As of March 31, 2018, there was approximately $4.2 million of unrecognized compensation cost related to these outstanding performance stock options.

As of March 31, 2018, there was approximately $14.2 million of unrecognized stock-based compensation cost related to all unvested time-based and performance-based stock options granted under our equity incentive plans.

At March 31, 2018, there were 10,544,883 shares of common stock available for future grant under our equity incentive plans and 652,891 options to purchase shares were exercised during the three months ended March 31, 2018.

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

Table of Contents

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 is being recognized as expense from July 1, 2016 to June 30, 2018.

As of March 31, 2018, there were approximately 2,115,568 shares reserved for future issuance under the Purchase Plan. The following table summarizes the weighted-average assumptions related to our Purchase Plan for the three months ended March 31, 2018 and 2017. 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.

	Three Months Ended March 31,			
	2018		2017	
Risk-free interest rate	0.6	%	0.5	%
Expected term (in years)	2.0		1.5	
Dividend yield	0.0	%	0.0	%
Expected volatility	63.8	%	63.1	%

7.Research and Development Accruals

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

8.Sponsored Research and License Agreements

We conduct research and development programs independently and in connection with our corporate collaborators. Currently, we are a party to collaboration agreements, but do not have ongoing performance obligations, 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, Aclaris Therapeutics International Limited (Aclaris) for the development and commercialization of 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 AXL inhibitors in oncology, and Daiichi Sankyo (Daiichi) to pursue research related to MDM2 inhibitors, a novel class of

Table of Contents

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, payments contingent upon specified 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 $532.4 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 $145.5 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 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. All deliverables under the agreement had been previously delivered, as such, the above payments of $3.3 million was recognized as revenue in the first quarter of 2017.

9.Cash, Cash Equivalents and Short-Term Investments

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

	March 31, 2018	December 31, 2017
Cash	$ 2,986	$ 582
Money market funds	4,686	2,795
U.S. treasury bills	6,729	6,726
Government-sponsored enterprise securities	6,179	7,826
Corporate bonds and commercial paper	73,720	97,822
	$ 94,300	$ 115,751
Reported as:		
Cash and cash equivalents	$ 40,125	$ 38,290
Short-term investments	54,175	77,461
	$ 94,300	$ 115,751

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

March 31, 2018	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury bills	$ 6,741	$ —	$ (12)	$ 6,729
Government-sponsored enterprise securities	6,192	—	(13)	6,179
Corporate bonds and commercial paper	73,782	1	(63)	73,720
Total	$ 86,715	$ 1	$ (88)	$ 86,628

December 31, 2017	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury bills	$ 6,733	$ —	$ (7)	$ 6,726
Government-sponsored enterprise securities	7,835	—	(9)	7,826
Corporate bonds and commercial paper	97,888	1	(67)	97,822
Total	$ 112,456	$ 1	$ (83)	$ 112,374

Table of Contents

As of March 31, 2018, our cash equivalents and short-term investments, which have contractual maturities within one year, had a weighted-average time to maturity of approximately 85 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 March 31, 2018 through their respective maturity dates. At March 31, 2018, we had no investments that had been in a continuous unrealized loss position for more than 12 months. As of March 31, 2018, a total of 32 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 March 31, 2018.

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):

March 31, 2018	Fair Value	Unrealized Losses
U. S. treasury bills	$ 6,729	$ (12)
Government-sponsored enterprise securities	6,179	(13)
Corporate bonds and commercial paper	35,513	(63)
Total	$ 48,421	$ (88)

10.Fair Value

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

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

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

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

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

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

Table of Contents

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 and liabilities 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 March 31, 2018			
	Level 1	Level 2	Level 3	Total
Money market funds	$ 4,686	$ —	$ —	$ 4,686
U.S. treasury bills	—	6,729	—	6,729
Government-sponsored enterprise securities	—	6,179	—	6,179
Corporate bonds and commercial paper	—	73,720	—	73,720
Total	$ 4,686	$ 86,628	$ —	$ 91,314

	Assets at Fair Value as of December 31, 2017			
	Level 1	Level 2	Level 3	Total
Money market funds	$ 2,795	$ —	$ —	$ 2,795
U.S. treasury bills	—	6,726	—	6,726
Government-sponsored enterprise securities	—	7,826	—	7,826
Corporate bonds and commercial paper	—	97,822	—	97,822
Total	$ 2,795	$ 112,374	$ —	$ 115,169

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 was originally set to expire in 2018. The lease term provides for renewal option for up to two additional periods of five years each. In July 2017, we exercised our option to extend the term of our lease for another five years through January 2023 and modified the amount of monthly base rent during such renewal period. We reevaluated our lease classification and continue to classify our lease as an operating lease during the renewal period.

In December 2014, we entered into a sublease agreement, which was amended in 2017, with an unrelated third party to occupy approximately 57,000 square feet of our research and office space. 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. Effective July 2017, the sublease agreement was amended primarily to extend the term of the sublease through January 2023 and modified the monthly base rent to equal the amount we will pay our landlord. Because the future sublease income under the extended sublease agreement is the same as the amount we will pay our landlord, we did not recognize any loss on sublease relative to this amendment. We expect to receive approximately $21.2 million in future sublease income (excluding our subtenant’s share of facilities operating expenses) through January 2023.

12.Subsequent Events

In April 2018, we completed an underwritten public offering in which we sold 16,000,000 shares of our common stock pursuant to an effective registration statement at a price to the public of $3.90 per share. We received net proceeds of approximately $58.4 million after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

Table of Contents

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, 2017. Operating results for the three months ended March 31, 2018 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 ability to successfully launch TAVALISSE in the United States by the end of May 2018; 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 providing novel small molecule drugs that significantly improve the lives of patients with immune and hematologic disorders, cancer and rare diseases. Our pioneering research focuses on signaling pathways that are critical to disease mechanisms. Our first FDA approved product is TAVALISSE™ (fostamatinib disodium hexahydrate), an oral spleen tyrosine kinase (SYK) inhibitor, for the treatment of adult patients with chronic immune thrombocytopenia who have had an insufficient response to a previous treatment. Our current clinical programs include Phase 2 studies of fostamatinib in autoimmune hemolytic anemia and IgA nephropathy. In addition, we have 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 March 31, 2018, we had approximately $94.3 million in cash, cash equivalents and short term investments. In April 2018, we completed an underwritten public offering in which we sold 16,000,000 shares of our common stock pursuant to an effective registration statement at a price to the public of $3.90 per share. We received net proceeds of approximately $58.4 million after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including the commercial launch of TAVALISSE in the U.S., through at least the next 12 months from the Form 10-Q filing date. We also continue to evaluate ex-U.S. partnerships for fostamatinib and other partnering opportunities across our pipelines.

Table of Contents

Our revenues have consisted primarily of revenues from sponsored research and license agreements with our corporate collaborators. Our potential future revenues may include sales from the launch of TAVALISSE (fostamatinib disodium hexahydrate), 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.

On April 17, 2018, we announced that the FDA had approved fostamatinib for the treatment of thrombocytopenia in adult patients with chronic ITP who have had an insufficient response to a previous treatment. On April 30, 2018, we announced that the American Journal of Hematology published positive results from the FIT Phase 3 clinical program. We expect to launch fostamatinib in the U.S. on our own in late May 2018. We plan to enter into partnership with third parties to commercialize fostamatinib in Europe and Asia.

Fostamatinib—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.

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 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. Using the most conservative sensitivity analysis, rather than the protocol's prespecified analysis, one more patient in the second study is considered a non-responder, resulting in 8 of 50 (16%) responders on fostamatinib (p = 0.256 vs. placebo). When the data from both studies are combined, however, this difference is statistically significant (p=0.007).

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 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

Table of Contents

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.

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, without rescue medication. In the post-study analysis we performed, a clinically-relevant platelet response was defined to include patients achieving one platelet count over 50,000/uL during the first 12 weeks of treatment, in absence of rescue medication, but who did not otherwise meet the stable response criteria. Once the platelet count of greater than 50,000/uL is achieved, a loss of response was defined as two consecutive platelet counts of less than 30,000/uL in any subsequent visits. In the combined dataset of both stable and clinically-relevant platelet responders for the FIT studies, the response rate was 43% (43/101), compared to 14% (7/49) for placebo (p=0.0006).

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 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 PDUFA. On April 17, 2018, we announced that the FDA had approved TAVALISSETM (fostamatinib disodium hexahydrate) for the treatment of thrombocytopenia in adult patients with chronic ITP who have had an insufficient response to a previous treatment. On April 30, 2018, we announced that the American Journal of Hematology published positive results from the FIT Phase 3 clinical program. We expect to launch TAVALISSETM in the U.S. on our own in late May 2018. We plan to enter into partnership with third parties to commercialize fostamatinib in Europe and Asia.

Commercial launch activities, including sales and marketing

We expect to commercialize TAVALISSE in the U.S. on our own in late May 2018. We plan to enter into partnerships with third parties to commercialize fostamatinib in Europe, Asia and rest of the world. A significant portion of our operating expenses in 2018 will be related to our commercial launch activities for TAVALISSE. Specifically, our marketing and sales efforts will be focused on targeting approximately 3,000 hematologists and hematologist-oncologists in the United States, who manage chronic adult ITP patients. We expect to continue to hire and recruit experienced commercial professionals, including sales representatives in the hematology area, and commercial operations, marketing, and market access professionals to support these efforts.

Competitive landscape for TAVALISSE

Our industry is intensely competitive and subject to rapid and significant technological change. Fostamatinib 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.

Currently, corticosteriods remain the most common first line therapy for ITP, occasionally in conjunction with intravenous immuglobulin (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 the sequence of their use, according to the most recent ITP guideline from the American Society of Hematology. Options include splenectomy, thrombopoietin receptor agonists (TPO-RAs) and various immunosuppressants (such as rituximab). The response rate criteria of the abovementioned options vary, precluding a comparison of response rates for individual therapies.

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

Table of Contents

treatment options could theoretically be beneficial 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 in the U.S. that are approved by the FDA to increase platelet production through binding and TPO receptors on megakaryocyte precursors include PROMACTA® (Novartis) and Nplate® (Amgen, Inc.).

Clinical Stage Programs

Fostamatinib—IgAN

Disease background. Immunoglobulin A Nephropathy (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 whom will eventually require 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, reduce the deposition of IgA immune complexes 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 its first and second cohorts. 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 second cohort evaluates a higher dose of fostamatinib (150mg BID) and completed enrollment in August 2017.

On April 3, 2018, we announced that trial did not achieve statistical significance for its primary endpoint, which was mean change in proteinuria comparing fostamatinib dose groups to placebo controls in all patients studied. However, in a pre-specified subgroup analysis of patients with greater than 1 gram/day of proteinuria at baseline, the initial data showed a greater reduction in proteinuria in fostamatinib-treated patients relative to placebo patients (this finding did not reach statistical significance). Patients with greater than 1 gram/day of proteinuria have an increased risk of disease progression and represent an unmet medical need. Current guidance for clinical trials in IgAN recommends studying patients with greater than 1 gram/day of proteinuria at entry. Further analysis, including histology, are expected later in late 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, also known as SOAR study, is currently enrolling patients with warm AIHA in the second stage of the trial. The trial is an open-label, multi-center, two-stage study that will evaluate the efficacy and safety of fostamatinib in patients with warm AIHA who have previously received treatment for the disorder, but have relapsed. Stage 1 completed enrollment for 19 patients (17 patients evaluable for efficacy) who received 150 mg of fostamatinib orally twice a day for a period of 12 weeks, with an option of entering into a long-term extension study. The patients returned to the clinic every two weeks for blood draws and medical assessment. The primary efficacy endpoint of this study was 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.

Table of Contents

In October 2017, we announced that, on a top-line, preliminary basis, Stage 1 of the AIHA study enrolled 17 patients who have had at least one post-baseline hemoglobin measure. In January 2018, we also announced the updated top-line data as of December 2017 for this open-label study of which 47% of these patients (8 patients out of 17) have responded to fostamatinib treatment. Of the 17, six patients, including the last two patients enrolled, responded during the 12-week evaluation period and an additional two patients met the response criteria in the extension study after 12 weeks of dosing. In February 2018, an additional patient in the Stage 1 extension study met the response criteria. As of February 2018, 53% of evaluable patients (9 of 17) have responded to fostamatinib treatment. The safety profile was consistent with the existing fostamatinib safety database. Given that the Stage 1 of the study met its primary efficacy endpoint, we have begun enrollment of Stage 2 of this study, in which 20 patients will be enrolled under the same protocol. In January 2018, the FDA granted our request for Orphan Drug designation for fostamatinib for the treatment of AIHA.

Partnered Clinical Programs

R548 (ATI-501 and ATI-502) - Aclaris

Aclaris is developing ATI-501 and ATI-502 an oral and topical Janus Kinase (JAK) 1/3 inhibitor. ATI- 501 is being developed as an oral treatment for patients with AA, including the more severe forms of AA that result in total scalp hair loss, known as alopecia totalis, and total hair loss on the scalp and body, known as alopecia universalis. This Phase 1 cross-over trial was conducted in 12 healthy volunteers at one investigational center in the U.S. to assess the safety, bioavailability, and pharmacodynamics of ATI-501. Aclaris is expected to initiate Phase 2 trials in the second half of 2018.

In the trial, treatment with ATI-501 capsules was well tolerated, with a safety profile similar to placebo. No clinically significant laboratory abnormalities were observed. These data are consistent with results from an earlier Phase 1 clinical trial in 44 healthy volunteers in which the study drug was well tolerated at all doses, with a safety profile similar to placebo. During the fourth quarter of 2017, three Phase 2 studies with the topical treatment ATI-502 in AA and Vitilago were initiated with initial results expected in 2018.

BGB324 - BerGenBio

BerGenBio’s first-in-class selective AXL kinase inhibitor, BGB324, has demonstrated compelling efficacy as a single agent, and in combination with standard of care cancer therapies and checkpoint inhibitors, thereby supporting clinical utility across multiple cancers in preclinical studies. Early clinical studies in healthy volunteers and cancer patients have shown BGB324 to be well-tolerated with a favorable safety profile, and encouraging evidence of single agent

and combination activity in AML and NSCLC. A strong correlation has also been observed with predictive biomarkers and the patients that respond. BGB324 has received Orphan Drug Designation in the U.S. for AML.

BerGenBio initiated Phase 1/2 studies with BGB324 as a single agent in relapsed acute myeloid leukaemia (AML) and myelodysplastic syndrome (MDS); and in combination with erlotinib (Tarceva®) in advanced (EGFR-positive) NSCLC. BerGenBio is also initiating Phase 2 studies with BGB324 in combination with KEYTRUDA® (pembrolizumab) in non-small cell adenocarcinoma of the lung and triple negative breast cancer (TNBC) in collaboration with another company.

DS-3032 - Daiichi

DS-3032 is an investigational oral selective inhibitor of the murine double minute 2 (MDM2) protein currently being investigated by Daiichi in three Phase 1 clinical trials for solid and hematological malignancies including acute myeloid leukemia (AML), acute lymphocytic leukemia (ALL), chronic myeloid leukemia (CML) in blast phase, lymphoma and myelodysplastic syndrome (MDS). DS-3032 has not been approved by any regulatory authority for uses under investigation.

Table of Contents

Preliminary safety and efficacy data from a Phase 1 study of DS-3032 suggests that DS-3032 may be a promising treatment for hematological malignancies including relapsed/refractory AML and high-risk MDS. Evaluation of additional dosing schedules of DS-3032 is underway and combination studies currently being planned by Daiichi.

Research/Preclinical Programs

We are conducting proprietary research in the broad disease areas of inflammation/immunology, immuno-oncology and cancers. 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.

During the second quarter of 2017, we selected a molecule from our IRAK program for preclinical development. The molecule was selected for development based on its ability to inhibit both the IRAK 1 and IRAK 4 signaling pathways in preclinical studies, potentially providing a clinical benefit in autoimmune and inflammatory diseases such as psoriasis, lupus, gout, psoriatic arthritis and multiple sclerosis. We expect to initiate clinical trials in the second quarter of 2018.

Sponsored Research and License Agreements

We conduct research and development programs independently and in connection with our corporate collaborators. Currently, we are a party to collaboration agreements, but do not have ongoing performance obligations, with BMS for the discovery, development and commercialization of cancer immunotherapies based on our small molecule TGF beta receptor kinase inhibitors, Aclaris for the development and commercialization of 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 AXL inhibitors in oncology, and Daiichi to pursue research related to MDM2 inhibitors, 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, payments contingent upon specified 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 $532.4 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 $145.5 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 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. All deliverables under the agreement had been previously delivered, as such, the above payments of $3.3 million was recognized as revenue in the first quarter of 2017.

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.

Table of Contents

“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 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 March 31,		From January 1, 2007*
	2018	2017	to March 31, 2018

Categories:			
Research	$ 2,507	$ 2,627	$ 228,873
Development	6,638	7,532	348,807
Other	2,097	2,217	232,423
	$ 11,242	$ 12,376	$ 810,103

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

“Other” expenses mainly represent allocated facilities costs of approximately $1.5 million and $1.9 million for the three months ended March 31, 2018 and 2017, respectively, and allocated stock-based compensation expenses of approximately $600,000 and $360,000 for the three months ended March 31, 2018 and 2017, respectively.

For the three months ended March 31, 2018 and 2017, a major portion of our total research and development expense was associated with salaries of our research and development personnel, our ITP, IRAK, IgAN and AIHA programs, and allocated facilities costs.

Table of Contents

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

Results of Operations

Three Months Ended March 31, 2018 and 2017

Revenues

	Three Months Ended March 31,		Aggregate Change
	2018	2017	
		(in thousands)	
Contract revenues from collaborations	$ —	$ 3,584	$ (3,584)

There were no contract revenues from collaborations during the three months ended March 31, 2018. Contract revenues from collaborations of $3.6 million during the three months ended March 31, 2017 is comprised primarily of the $3.3 million payment from BerGenBio as a result of advancing BGB324, a selective, potent and orally available small molecule.

Our potential future revenues may include sales from the launch of fostamatinib in ITP, 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 March 31,		Aggregate Change
	2018	2017	
		(in thousands)	
Research and development expense	$ 11,242	$ 12,376	$ (1,134)

Stock-based compensation expense included in research and development expense	$ 600	$ 360	$ 240

The decrease in research and development expense for the three ended March 31, 2018, compared to the same period in 2017, was primarily due to the decreases in 2018 of costs related to clinical trials as well as the submission of our NDA for fostamatinib in ITP of $2.2 million, allocated facility costs of $367,000 and research supplies of $180,000, partially offset by increases in personnel costs.

We expect our research and development expense in 2018 to remain relatively consistent on a quarterly basis.

General and Administrative Expense

	Three Months Ended March 31,		Aggregate Change
	2018	2017	
		(in thousands)	
General and administrative expense	$ 13,492	$ 7,410	$ 6,082
Stock-based compensation expense included in general and administrative expense	$ 940	$ 595	$ 345

The increase in general and administrative expense for the three months ended March 31, 2018, compared to the same period in 2017, was primarily due to the increased expenses related to commercial launch preparation costs of fostamatinib in ITP of $5.0 million, including personnel costs.

Table of Contents

We expect our general and administrative expense in 2018 to increase as we as continue our efforts in the commercial launch of TAVALISSE (fostamatinib disodium hexahydrate), including hiring experienced commercial professionals, as well as sales representatives in the hematology and hematology-oncology area.

Interest Income

	Three Months Ended March 31,		Aggregate
	2018	2017	Change
		(in thousands)	
Interest income	$ 349	$ 156	$ 193

Interest income results from our interest-bearing cash and investment balances. The increases in interest income for the three months ended March 31, 2018, as compared to the same period in 2017 were primarily due to the higher yield on our investments.

Gain on Disposal of Assets

	Three Months Ended March 31,		Aggregate
	2018	2017	Change
		(in thousands)	
Gain on disposal of assets	$ —	$ 732	$ (732)

Gain on disposal of assets during the three months ended March 31, 2017 related to the proceeds from the sale of our fully depreciated property and equipment.

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 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 with the exception of adopting ASU 2014-09 as of January 1, 2018, as discussed below, 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, 2017, 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. 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. 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 previous accounting policy, we recognized 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

Table of Contents

the variable consideration is subsequently resolved. We adopted this new standard on January 1, 2018 using the modified retrospective approach. Because all of the performance obligations for our outstanding collaboration agreements had been completed prior to December 31, 2017, we did not record any adjustment on the opening balance of Accumulated Deficit as of January 1, 2018.

Under this new guidance, the Company recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the Company expects to receive in exchange for those goods or services. To determine whether arrangements are within the scope of this new guidance, the Company performs the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the Company satisfies its performance performance obligation. The Company applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of this new guidance, we assess the goods or services promised within each contract and identify, as a performance obligation, and assess whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

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.

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 and our preparation for commercial launch of TAVALISSE (fostamatinib disodium hexahydrate).

As of March 31, 2018, we had approximately $94.3 million in cash, cash equivalents and short term investments, as compared to approximately $115.8 million as of December 31, 2017, a decrease of approximately $21.5 million. The decrease was primarily attributable to the payments associated with funding our operating expenses during the three

months ended March 31, 2018, partially offset by the $2.0 million proceeds from issuances of common stock upon exercise of options.

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. Effective July 2017, the sublease agreement was amended primarily to extend the term of the sublease through January 2023. During the three months ended March 31, 2018, we received approximately $1.4 million of sublease income and reimbursements. We expect to receive approximately $21.2 million in future sublease income (excluding our subtenant's share of facility's operating expenses) through January 2023. In April 2018, we completed an underwritten public offering in which we sold 16,000,000 shares of our common stock pursuant to an effective registration statement at a price to the public of $3.90 per share. We received net proceeds of approximately $58.4 million after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including the commercial launch of TAVALISSE in the U.S., through at least the next 12 months from the Form 10-Q filing date. 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

Table of Contents

could utilize our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with commercial launch, the development of our product candidates and other research and development activities, we are unable to estimate with certainty our future product revenues, our revenues from our current and future collaborative partners, 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 the proceeds from exercise of stock options and 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 costs to commercialize TAVALISSE for the treatment of ITP in the United States, or any other future product candidates, if any such candidate receives regulatory approval for commercial sale;
- 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 costs and timing of regulatory filings and approvals by us and our collaborators;
- the progress of research and development programs carried out by us and our collaborative partners;
- 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 commercial efforts and/or 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 three months ended March 31, 2018 and 2017, 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

Table of Contents

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

	Three Months Ended March 31,	
	2018	2017
	(in thousands)	
Net cash provided by (used in):		
Operating activities	$ (23,394)	$ (20,762)
Investing activities	23,218	(150)
Financing activities	2,011	43,424
Net increase in cash and cash equivalents	$ 1,835	$ 22,512

Net cash used in operating activities was approximately $23.4 million for the three months ended March 31, 2018, compared to approximately $20.8 million for the three months ended March 31, 2017. Net cash used in operating activities for the three months ended March 31, 2018 was primarily due to the cash payments related to our research and development programs and commercial launch preparation costs. Net cash used in operating activities for the three months ended March 31, 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. The timing of cash requirements may vary from period to period depending on our commercial launch activities related to TAVALISSE (fostamatinib disodium hexahydrate), 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 $23.2 million for the three months ended March 31, 2018, compared net cash used in investing activites of approximately $150,000 for the three months ended March 31, 2017. Net cash provided by investing activities during the three months ended March 31, 2018 related to net maturities of short-term investments, partially offset by capital expenditures. Net cash used in investing activities during the three months ended March 31, 2017 related to net purchases of short-term investments as well as capital expenditures, partially offset by the proceeds from disposal of property and equipment. Capital expenditures were approximately $197,000 for the three months ended March 31, 2018, compared to approximately $41,000 for the same period in 2017.

Net cash provided by financing activities was approximately $2.0 million for the three months ended March 31, 2018, compared to approximately $43.4 million for the three months ended March 31, 2017. Net cash provided by financing activities for the three months ended March 31, 2018 related to the cash proceeds received from the exercise of stock options. Net cash provided by financing activities for the three months ended March 31, 2017 consisted of net proceeds of $43.0 million from issuance of common stock pursuant to the underwritten public offering, as well as

proceeds from exercise of stock options.

Off-Balance Sheet Arrangements

As of March 31, 2018, we had no off-balance sheet arrangements (as defined in Item 303(a)(4)(ii) of Regulation S-K under the Exchange Act).

Contractual Obligations

We conduct our research and development programs internally and through third parties that include, among others, arrangements with universities, consultants and contract research organizations (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.

Table of Contents

We have agreements with certain CROs to conduct our clinical trials and with third parties relative to our commercial launch of fostamatinib. 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 and various activities related to commercial launch. We will continue to enter into contracts in the normal course of business with various third parties who support our clinical trials, support our preclinical research studies, and provide other services related to our operating purposes as well as our commercial launch of TAVALISSE (fostamatinib disodium hexahydrate). 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 provided 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 March 31, 2018, we had the following contractual commitments:

		Payment Due By Period			
		Less than	1 -	3 -	More than
	Total	1 Year	3 Years	5 Years	5 Years
	(in thousands)				
Facilities lease (1)	$ 47,203	$ 9,052	$ 19,204	$ 18,947	$ —

(1) In December 2014, we entered into a sublease agreement, which was amended in 2017, 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 approximately $21.2 million which we expect to receive over the term of the sublease through January 2023.

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 milestone events occur. As of March 31, 2018, we concluded that certain future milestone events are probable of achievement. Accordingly, we recorded a contingent liability of $3.0 million as of March 31, 2018. Of this amount, $1.5 million was recognized as expense during the three months ended March 31, 2018 and was recorded as part of general and administrative expenses in the Statements of Operations.

Item 3.Quantitative and Qualitative Disclosures About Market Risk

During the three months ended March 31, 2018, 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, 2017.

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 principal accounting 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. Effective March 2018, we implemented a new accounting system to support our financial reporting (as defined in Rule 13a-15(f). Therefore, modifications to the design and documentation of internal control processes and procedures relating to the new systems to replace and supplement existing internal controls over financial reporting were made as appropriate. The changes were undertaken to enhance our system and reporting capabilities to support our growth, and were not undertaken in response to any actual or perceived deficiencies in our internal control over financial reporting.

Table of Contents

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 6, 2018.

Even if we, or any of our collaborative partners, are able to commercialize TAVALISSE (fostamatinib disodium hexahydrate) or any product candidate that we, or they, develop, the product may become subject to unfavorable pricing regulations, third-party payor reimbursement practices or labeling restrictions, any of which could harm our business.*

The commercial success of any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future will depend substantially on the extent to which the costs of our product candidates will be paid by third-party payors, including government health care programs and private health insurers. If coverage is not available, or reimbursement is limited, we, or any of our collaborative partners, may not be able to successfully commercialize TAVALISSE or any of our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us, or any of our collaborative partners, to establish or maintain pricing sufficient to realize a sufficient return on our or their investments. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement levels for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time consuming and costly process that may require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we, or any of our collaborative partners, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which

Table of Contents

may negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability or the ability of any future collaborators to recoup our or their investment in one or more product candidates, even if our product candidates obtain marketing approval.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Therefore, our ability, and the ability of any of our collaborative partners, to successfully commercialize fostamatinib or any of our product candidates will depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from third-party payors.

Additionally, the approved labeling ultimately approved for any of our product candidates for which we have or may obtain regulatory approval may include restrictions on their uses and may be subject to ongoing FDA requirements governing the labeling, packaging, storage, distribution, safety surveillance, advertising, promotion, record-keeping and reporting of safety and other post-market information. If we or any of our collaborative partners do not timely obtain or comply with the labeling approval by the FDA on any of our product candidates, it may delay or inhibit our ability to successfully commercialize our products and generate revenues.

Our prospects are highly dependent on the successful commercialization of TAVALISSE™ (fostamatinib disodium hexahydrate), which received approval in April 2018 from the FDA for patients with chronic ITP who have had an insufficient response to a previous treatment. To the extent that TAVALISSE is not commercially successful, our business, financial condition and results of operations may be materially adversely affected and the price of our common stock may decline. *

TAVALISSE is our only drug that has been approved for sale and it has only been approved in the United States for patients with chronic ITP who have had an insufficient response to a previous treatment. We are focusing a significant portion of our activities and resources on fostamatinib, and we believe our prospects are highly dependent on, and a significant portion of the value of our Company relates to, our ability to successfully commercialize TAVALISSE in the United States.

Successful commercialization of TAVALISSE is subject to many risks. We have never, as an organization, launched or commercialized a product, and there is no guarantee that we will be able to do so successfully with fostamatinib for its approved indication. There are numerous examples of unsuccessful product launches and failures to meet high expectations of market potential, including by pharmaceutical companies with more experience and resources than us.

Market acceptance of fostamatinib and any our or collaborative partners' future product candidates that may receive approval, will depend on a number of factors, including:

- the efficacy and safety as demonstrated in clinical trials;
- the timing of market introduction of the product as well as competitive products;
- the clinical indications for which the product is approved;
- acceptance by physicians, the medical community and patients of the product as a safe and effective treatment;
- the ability to distinguish safety and efficacy from existing, less expensive generic alternative therapies, if any;
- the convenience of prescribing, administrating and initiating patients on the product;
- the potential and perceived advantages of the product over alternative treatments;

Table of Contents

- the potential and perceived value of the product over alternative treatments;

- the cost of treatment in relation to alternative treatments, including any similar generic treatments;

- the availability of coverage and adequate reimbursement and pricing by third-party payors and government authorities;

- the prevalence and severity of adverse side effects; and

- the effectiveness of sales and marketing efforts.

Even if we are successful in building out our commercial team, there are many factors that could cause the launch and commercialization of TAVALISSE to be unsuccessful, including a number of factors that are outside our control. The commercial success of TAVALISSE depends on the extent to which patients and physicians accept and adopt TAVALISSE for patients with chronic ITP who have had an insufficient response to a previous treatment. We also do not know how physicians, patients and payors will respond to the pricing of fostamatinib. In particular, our insight into pricing sensitivity may be delayed because as part of our initial launch strategy, we intend to provide some free product as samples during a trial period, and do not know whether physicians that initially use TAVALISSE will continue to do so after using the free product samples.

Physicians may not prescribe TAVALISSE and patients may be unwilling to use TAVALISSE if coverage is not provided or reimbursement is inadequate to cover a significant portion of the cost. Additionally, any negative development for fostamatinib in clinical development in additional indications, may adversely impact the commercial results and potential of fostamatinib. Thus, significant uncertainty remains regarding the commercial potential of fostamatinib.

If the launch or commercialization of TAVALISSE is unsuccessful or perceived as disappointing, our stock price could decline significantly and the long-term success of the product and our company could be harmed.

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.

If we are unable to effectively train and equip our sales force, our ability to successfully commercialize TAVALISSE (fostamatinib disodium hexahydrate) will be harmed.*

TAVALISSE will be a newly-marketed drug and, therefore, none of the members of our sales force will have ever promoted TAVALISSE prior to its launch. As a result, we will be required to expend significant time and resources to train our sales force to be credible, persuasive and compliant with applicable laws in marketing TAVALISSE for patients with chronic ITP who have had an insufficient response to a previous treatment. In addition, we must train our sales force to ensure that an appropriate and compliant message about TAVALISSE is being delivered. If we are unable to effectively train our sales force and equip them with compliant and effective materials, including medical and sales literature to help them appropriately inform and educate regarding its potential benefits and proper administration, our efforts to successfully commercialize TAVALISSE could be put in jeopardy, which would negatively impact our ability to generate product revenues.

Enacted or future legislation, including potentially unfavorable pricing regulations or other healthcare reform initiatives, may increase the difficulty and cost for us to obtain regulatory approval of our product candidates and/or commercialize fostamatinib or our product candidates, once approved, and affect the prices we may set or obtain.*

Table of Contents

The regulations that govern, among other things, regulatory approvals, coverage, pricing and reimbursement for new drug products vary widely from country to country. In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could prevent or delay regulatory approval of our product candidates, restrict or regulate post-approval activities and affect our ability to successfully sell fostamatinib or any product candidates for which we obtain regulatory approval in the future. 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, which substantially changes the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Affordable Care Act and its implementing regulations, among other things, addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for certain drugs and biologics, including our approved product and product candidates, 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% (and 70% starting January 1, 2019) 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 United States 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 of at least $1.2 trillion for the years 2013 through 2021, was unable to reach 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, due to subsequent legislative amendments, will remain in effect through 2027, unless additional Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, 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.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- the demand for fostamatinib or our product candidates, if we obtain regulatory approval;

- our ability to set a price that we believe is fair for our products;

- our ability to generate revenue and achieve or maintain profitability;

- the level of taxes that we are required to pay; and

- the availability of capital.

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

Since its enactment, there have been judicial and Congressional challenges to numerous provisions of the Affordable Care Act, as well as recent efforts by the Trump administration to repeal or replace certain aspects of the Affordable Care Act. Since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the Affordable Care Act or otherwise circumvent some of the requirements for

Table of Contents

health insurance mandated by the Affordable Care Act. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the Affordable Care Act. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the Affordable Care Act have been enacted. The Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the "individual mandate". Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain mandated fees under the Affordable Care Act, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, among other things, amends the Affordable Care Act, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole". Congress may consider other legislation to repeal and replace elements of the Affordable Care Act. Any repeal and replace legislation may have the effect of limiting the amounts that government agencies will pay for healthcare products and services, which could result in reduced demand for our products or additional pricing pressure, or may lead to significant deregulation, which could make the introduction of competing products and technologies much easier. Policy changes, including potential modification or repeal of all or parts of the Affordable Care Act or the implementation of new health care legislation could result in significant changes to the health care system, which could have a material adverse effect on our business, results of operations and financial condition.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the regulatory approvals of our product candidates, if any, may be.

In the United States, the European Union and other potentially significant markets for our current and future products, government authorities and third-party payors are increasingly attempting to limit or regulate the price of medical products and services, particularly for new and innovative products and therapies, which has resulted in lower average selling prices. For example, in the United States, there have been several recent Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, to encourage importation from other countries and bulk purchasing. Furthermore, the increased emphasis on managed healthcare in the United States and on country and regional pricing and reimbursement controls in the European Union will put additional

pressure on product pricing, reimbursement and usage, which may adversely affect our sales and results of operations. These pressures can arise from rules and practices of managed care groups, judicial decisions and governmental laws and regulations related to Medicare, Medicaid and healthcare reform, pharmaceutical reimbursement policies and pricing in general.

We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws and other federal and state healthcare laws, and the failure to comply with such laws could result in substantial penalties. Our employees, independent contractors, consultants, principal investigators, CROs, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.*

Table of Contents

Our business operations and current and future arrangements with investigators, healthcare professionals, consultants, third-party payers and customers, may expose us to broadly applicable fraud and abuse and other healthcare laws and regulations. These laws may constrain the business or financial arrangements and relationships through which we conduct our operations, including how we research, market, sell and distribute any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, including off-label uses of our products, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use or misrepresentation of information obtained in the course of patient recruitment for clinical trials, creating fraudulent data in our preclinical studies or clinical trials or illegal misappropriation of drug product, which could result in regulatory sanctions and cause serious harm to our reputation. The laws that may affect our ability to operate include, but are not limited to:

- the Federal Anti-Kickback Statute, which prohibits, among other things, individuals and entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual for, or the purchase, lease, order or recommendation of, any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs. A person or entity does not need to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation;

- federal civil and criminal false claims laws and civil monetary penalty laws, including the federal civil False Claims Act, which impose criminal and civil penalties, through government or civil whistleblower, or qui tam, actions, on individuals and entities for, among other things, knowingly presenting, or causing to be presented, claims for payment or approval from the federal government, including federal healthcare programs, such as Medicare, Medicaid that are false, fictitious or fraudulent, or knowingly making, using or causing to be made or used, a false statement to avoid, decrease or conceal an obligation to pay money to the federal government. Entities can be held liable under the federal civil False Claims Act if they are deemed to “cause” the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers, promoting a product off label, or for providing medically unnecessary services or items. In addition, the government may assert that a claim including items and services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act;

- the Federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which imposes criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private), willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false, fictitious or fraudulent statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the healthcare fraud statute implemented under HIPAA or specific intent to violate it in order to have committed a violation;

· HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses, as well as their respective business associates that perform services for them that involve the creation, use, maintenance or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization;

Table of Contents

- the federal physician payment transparency requirements, sometimes referred to as the "Physician Payments Sunshine Act," created under the Affordable Care Act, and its implementing regulations, which require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the United States Department of Health and Human Services, or HHS, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members;

- the U.S. Federal Food, Drug and Cosmetic Act, or FDCA, which prohibits, among other things, the adulteration or misbranding of drugs and medical devices; and

- federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Additionally, we are subject to state and foreign equivalents of each of the healthcare fraud and abuse laws described above, among others, some of which may be broader in scope and may apply regardless of the payor. We may also be subject to: state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; state laws that e restrict payments that may be made to healthcare providers; state laws that require pharmaceutical manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; state and local laws that require the registration of pharmaceutical sales representatives; and equivalent foreign laws and regulations. Further, we may be subject to state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

We are also exposed to the risk of fraud, misconduct or other illegal activity by our employees, independent contractors, consultants, principal investigators, CROs, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to: comply with the laws of the FDA and other similar foreign regulatory bodies; provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies; comply with manufacturing standards we have established; comply with federal and state data privacy, security, fraud and abuse and other healthcare laws and regulations in the United States and similar foreign fraudulent misconduct laws; or report financial information or data accurately or to disclose unauthorized activities to us. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations.

We are also subject to the risk that a person or government could allege such fraud or other misconduct, even if none occurred. Efforts to ensure that our business arrangements will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and

we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, individual imprisonment, additional reporting obligations and oversight if we become subject to a corporate integrity agreement or other agreement to resolve allegations of non-compliance with these laws, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

Table of Contents

Even for those product candidates that have or may 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, in which case we may not generate significant revenues or become profitable.*

For our product candidates that haave or may 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;
- the strength of marketing and distribution support; and
- the availability of third-party coverage or reimbursement.

Efforts to educate the physicians, patients, healthcare payors and others in the medical community on the benefits of our product candidates may require significant resources and may not be successful. 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 may be relying on a single distribution facility for the potential sale of any of our product candidates.*

Our distribution operations, if and when we launch any of our product candidate, may be concentrated in a single distribution center owned by a third party logistics provider. Any significant disruption in the operation of the facility due to natural disaster or severe weather, or events such as fire, accidents, power outages, system failures, or other unforeseen causes, could devalue or damage a significant portion of our inventory and could adversely affect our product distribution and sales until such time as we could secure an alternative facility. If we encounter difficulties with our distribution facility or other problems or disasters arise, we cannot ensure that critical systems and operations will be restored in a timely manner or at all, and this would have a material adverse effect on our business. In addition, growth could require us to further expand our current facility, which could affect us adversely in ways that we cannot predict.

We lack the capability to manufacture compounds for clinical development and we intend to rely on third parties for commercial supply, manufacturing and distribution if any of our product candidates which receive regulatory approval and we may be unable to obtain required material or product 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 TAVALISSE (fostamatinib disodium hexahydrate) or any product candidates for clinical trials, including fostamatinib in AIHA and

Table of Contents

IgAN. We currently use one manufacturer of fostamatinib. We do not currently have, nor do we plan to acquire the infrastructure or capability to supply, manufacture or distribute preclinical, clinical or commercial quantities of drug substances or products. 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. Our ability to develop our product candidates, and our ability to commercially supply our products will depend, in part, on our ability to successfully obtain the APIs and other substances and materials used in our product candidates from third parties and to have finished products manufactured by third parties in accordance with regulatory requirements and in sufficient quantities for preclinical and clinical testing and commercialization. If we fail to develop and maintain supply relationships with these third parties, we may be unable to continue to develop or commercialize our product candidates.

We rely and will continue to rely on certain third parties as the sole source of the materials they supply or the finished products they manufacture. The drug substances and other materials used in our product candidates are currently available only from one supplier and one manufacturer and certain of our finished product candidates are manufactured by one or a limited number of contract manufacturers. Any of these existing supplier or manufacturer may:

- fail to supply us with product on a timely basis or in the requested amount due to unexpected damage to or destruction of facilities or equipment or otherwise;

- fail to increase manufacturing capacity and produce drug product and components in larger quantities and at higher yields in a timely or cost-effective manner, or at all, to sufficiently meet our commercial needs;

- be unable to meet our production demands due to issues related to their reliance on sole-source suppliers and manufacturers;

- supply us with product that fails to meet regulatory requirements;

- become unavailable through business interruption or financial insolvency;

- lose regulatory status as an approved source;

- be unable or unwilling to renew current supply agreements when such agreements expire on a timely basis, on acceptable terms or at all; or

- discontinue production or manufacturing of necessary drug substances or products.

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.

Table of Contents

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.

Forecasting potential sales for any of our product candidates will be difficult, and if our projections are inaccurate, our business may be harmed and our stock price may be adversely affected.

Our business planning requires us to forecast or make assumptions regarding product demand and revenues for any of our product candidates if they are approved despite numerous uncertainties. These uncertainties may be increased if we rely on our collaborators or other third parties to conduct commercial activities in certain geographies and provide us with accurate and timely information. Actual results may differ materially from projected results for various reasons, including the following, as well as risks identified in other risk factors:

- the efficacy and safety of any of our product candidates, including as relative to marketed products and product candidates in development by third parties;
- pricing (including discounting or other promotions), reimbursement, product returns or recalls, competition, labeling, adverse events and other items that impact commercialization;
- the rate of adoption in the particular market, including fluctuations in demand for various reasons;
- lack of patient and physician familiarity with the drug;
- lack of patient use and physician prescribing history;
- lack of commercialization experience with the drug;

- actual sales to patients may significantly differ from expectations based on sales to wholesalers; and

- uncertainty relating to when the drug may become commercially available to patients and rate of adoption in other territories.

We expect that our revenues from sales of any of our product candidates will continue to be based in part on estimates, judgment and accounting policies. Any incorrect estimates or disagreements with regulators or others regarding such estimates or accounting policies may result in changes to our guidance, projections or previously reported results. Expected and actual product sales and quarterly and other results may greatly fluctuate, including in the near-term, and such fluctuations can adversely affect the price of our common stock, perceptions of our ability to forecast demand and revenues, and our ability to maintain and fund our operations.

Table of Contents

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 April 2018, we announced that our Phase 2 clinical trial in patients with IgAN did not achieve statistical significance for its primary endpoint, which was mean change in proteinuria comparing fostamatinib dose groups to placebo controls in all patients studied.

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 successfully commercialize fostamatinib, our business will be harmed.

Any product for which we have obtained regulatory approval, or for which we obtain approval in the future, is subject to, or will be subject to, extensive ongoing regulatory requirements by the FDA, EMA and other comparable regulatory authorities, and if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, we may be subject to penalties, we will be unable to generate revenue from the sale of such products, our potential for generating positive cash flow will be diminished, and the capital necessary to fund our operations will be increased.*

In April 2018, we announced that the FDA had approved TAVALISSE™ (fostamatinib disodium hexahydrate) for the treatment of thrombocytopenia in adult patients with chronic ITP who have had insufficient response to previous treatment. We expect to launch fostamatinib in the United States on our own in late May 2018. We plan to enter into partnerships with third parties to commercialize fostamatinib in Europe and Asia. To date, none of our other product candidates have received regulatory approval. Any product for which we have obtained regulatory approval, or for which we obtain regulatory approval in the future, along with the manufacturing processes and practices, post-approval clinical research, product labeling, advertising and promotional activities for such product, are subject to continual requirements of, and review by, the FDA, the EMA and other comparable international regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing

Table of Contents

requirements, current good manufacturing practices (cGMP) requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians, import and export requirements and recordkeeping.

Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, the FDA often requires post-marketing testing and surveillance to monitor the effects of products. The FDA, the EMA and other comparable international regulatory agencies may condition approval of our product candidates on the completion of such post-marketing clinical studies. These post-marketing studies may suggest that a product causes undesirable side effects or may present a risk to the patient. Additionally, the FDA may require Risk Evaluation and Mitigation Strategies, or REMS, 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.

Discovery after approval of previously unknown problems with any of our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in actions such as:

- restrictions on our ability to conduct clinical trials, including full or partial clinical holds on ongoing or planned trials
- restrictions on product manufacturing processes;
- restrictions on the marketing of a product;
- restrictions on product distribution;
- requirements to conduct post-marketing clinical trials;
- untitled or warning letters or other adverse publicity;

- withdrawal of products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- refusal to permit the import or export of our products;
- product seizure;
- fines, restitution or disgorgement of profits or revenue;
- refusal to allow us to enter into supply contracts, including government contracts;
- injunctions; or
- imposition of civil or criminal penalties.

Table of Contents

If such regulatory actions are taken, the value of our company and our operating results will be adversely affected. Additionally, if the FDA, the EMA or any other comparable international regulatory agency withdraws its approval of a product that is or may be approved, we will be unable to generate revenue from the sale of that product in the relevant jurisdiction, our potential for generating positive cash flow will be diminished and the capital necessary to fund our operations will be increased. Accordingly, we continue to expend significant time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance, post-marketing studies and quality control.

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.

Table of Contents

Outside the United States, our ability, or that of our collaborative partners, to market a product is contingent upon receiving a marketing authorization from the appropriate regulatory authorities. This foreign regulatory approval process typically includes all of the risks and costs associated with FDA approval described above and may also include additional risks and costs, such as the risk that such foreign regulatory authorities, which often have different regulatory and clinical trial requirements, interpretations and guidance from the FDA, may require additional clinical trials or results for approval of a product candidate, any of which could result in delays, significant additional costs or failure to obtain such regulatory approval. For example, there can be no assurance that we or our collaborative partners will not have to provide additional information or analysis, or conduct additional clinical trials, before receiving approval to market product candidates.

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 and our preparation for the commercial launch of TAVALISSE (fostamatinib disodium hexahydrate). 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 commercial launch of TAVALISSE in the U.S. in late May 2018, through at least the next 12 months from the Form 10-Q filing date. 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 commercial launch, the development of our product candidates and other research and development activities, we are unable to estimate with certainty our future product revenues, our revenues from our current and future collaborative partners, 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 commercial launch of TAVALISSE and 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 proceeds from exercise of stock options and 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 costs to commercialize fostamatinib for the treatment of ITP in the United States, or any other future product candidates, if any such candidate receives regulatory approval for commercial sale;

Table of Contents

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

- the costs and timing of regulatory filings and approvals by us and our collaborators;

- the progress of research and development programs carried out by us and our collaborative partners;

- 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 commercial efforts and/or 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.

Table of Contents

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 commercial efforts and/or 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.

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, 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.

43

Table of Contents

We have obtained orphan drug designation from the FDA for fostamatinib for the treatment of ITP and AIHA, but we may not be able to obtain or maintain orphan drug designation or exclusivity for fostamatinib for the treatment of ITP, warm AIHA or our other product candidates, or we may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

We have obtained orphan drug designation in the United States for fostamatinib for the treatment of ITP and AIHA. We may seek orphan drug designation for other product candidates in the future. 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.

We cannot assure you that any future application for orphan drug designation with respect to any other product candidate will be granted. If we are unable to obtain orphan drug designation with respect to other product candidates in the United States, we will not be eligible to obtain the period of market exclusivity that could result from orphan drug designation or be afforded the financial incentives associated with orphan drug designation. Even though we have received orphan drug designation for fostamatinib for the treatment of ITP and warm AIHA, 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 for fostamatinib for the treatment of ITP, AIHA or any future product candidate 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 $24.4 million for the three months ended March 31, 2018. 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, and recently our significant expenses related to the costs in preparation for the commercial launch of TAVALISSE (fostamatinib disodium hexahydrate). We expect to continue to incur losses from operations, at least in the next twelve months, and there can be no assurance that we will generate

Table of Contents

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 future 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 March 31, 2018, we had an accumulated deficit of approximately $1.2 billion. The extent of our future losses or profitability, if any, is highly uncertain.

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.

Table of Contents

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.

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 March 31, 2018, we had 58 pending patent applications and 377 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.

Table of Contents

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.

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.

The recently passed comprehensive tax reform bill could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law new tax legislation, or the Tax Act, which significantly reforms the Internal Revenue Code of 1986, as amended. The Tax Act, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%; limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses); limitation of the deduction for net operating losses generated after 2017 to 80% of current year taxable income, indefinite carryforward of net operating losses and elimination of net operating loss carrybacks; changes in the treatment of offshore earnings regardless of whether they are repatriated; mandatory capitalization of research and development expenses beginning in 2022; immediate deductions for certain new investments instead of deductions for depreciation expense over time; further deduction limits on executive compensation; and modifying, repealing and

Table of Contents

creating many other business deductions and credits, including the reduction in the orphan drug credit from 50% to 25% of qualifying expenditures. Our federal net operating loss carryovers will be carried forward indefinitely pursuant to the Tax Act. We continue to examine the impact this tax reform legislation may have on our business. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the Tax Act is uncertain and our business and financial condition could be adversely affected. The impact of this tax reform on holders of our common stock is also uncertain and could be adverse. This periodic report does not discuss any such tax legislation or the manner in which it might affect us or our stockholders in the future. We urge our stockholders to consult with their legal and tax advisors with respect to such legislation.

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. Federal net operating losses generated prior to 2018 will continue to be governed by the net operating loss tax rules as they existed prior to the adoption of the new Tax Act, which means that generally they will expire 20 years after they were generated if not used prior thereto. Many states have similar laws. Accordingly, our federal and state net operating losses could expire unused and be unavailable to offset future income tax liabilities. Under the newly enacted Tax Act, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited to 80% of current year taxable income. It is uncertain if and to what extent various states will conform to the newly enacted federal tax law. In addition, utilization of net operating losses to offset potential future taxable income and related income taxes that would otherwise be due is subject to annual limitations under the "ownership change" provisions of Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (Internal Revenue Code) and similar state provisions, which may result in the expiration of net operating losses before future utilization. In general, under the Code, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating losses and other pre-change tax attributes (such as research and development credit carryforwards) to offset its post-change taxable income or taxes may be limited. Our equity offerings and other changes in our stock ownership, some of which are outside of our control, may have resulted or could in the future result in an ownership change. Although we have completed studies to provide reasonable assurance that an ownership change limitation would not apply, we cannot be certain that a taxing authority would reach the same conclusion. If, after a review or audit, an ownership change limitation were to apply, utilization of our domestic net operating losses and tax credit carryforwards could be limited in future periods and a portion of the carryforwards could expire before being available to reduce future income tax liabilities.

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

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

Table of Contents

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.

Global economic conditions could adversely impact our business.*

The U.S. government has indicated its intent to alter its approach to international trade policy and in some cases to renegotiate, or potentially terminate, certain existing bilateral or multi-lateral trade agreements and treaties with foreign countries, including the North American Free Trade Agreement ("NAFTA"). In addition, the U.S. government has initiated or is considering imposing tariffs on certain foreign goods. Related to this action, certain foreign governments, including China, have instituted or are considering imposing tariffs on certain U.S. goods. It remains

unclear what the U.S. Administration or foreign governments will or will not do with respect to tariffs, NAFTA or other international trade agreements and policies. A trade war or other governmental action related to tariffs or international trade agreements or policies has the potential to disrupt our research activities, affect our suppliers and/or the U.S. economy or certain sectors thereof and, thus, could adversely impact our businesses.

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. 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

Table of Contents

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

Competition may also arise from:

· new or better methods of target identification or validation;

· other drug development technologies and methods of preventing or reducing the incidence of disease;

· new small molecules; or

· other classes of therapeutic agents.

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

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

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

- identifying and validating targets;

- screening compounds against targets; and

- undertaking preclinical testing and clinical trials.

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

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

Table of Contents

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;
- 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

Table of Contents

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 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.

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 and the sale of any products for which we obtain marketing approval exposes us to the risk of product liability claims. Product liability claims might be brought against us by consumers, health care providers, pharmaceutical companies or others selling or otherwise coming into contact with our products. 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. If and when we obtain marketing approval for our product candidates, we intend to expand our insurance coverage to include the sale of commercial products; however, we may be unable to obtain product liability insurance on commercially reasonable terms or in adequate amounts. 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

Table of Contents

into consulting arrangements, exclusive or otherwise, with competing pharmaceutical or biotechnology companies, any of which would have a detrimental impact on our research objectives and could have a material adverse effect on our business, financial condition and results of operations.

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

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

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

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

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

Our facilities are located in the San Francisco Bay Area near known earthquake fault zones and are vulnerable to significant damage from earthquakes. We are also vulnerable to damage from other types of disasters, including fires, floods, power loss, communications failures and similar events. If any disaster were to occur, our ability to operate our business at our facilities would be seriously, or potentially completely, impaired, and our research could be lost or destroyed. In addition, the unique nature of our research activities and of much of our equipment could make it

difficult for us to recover from a disaster. The insurance we maintain may not be adequate to cover our losses resulting from disasters or other business interruptions.

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

Because we will continue to need additional capital in the future to continue to expand our business and our 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 March 2018 and declared effective by the SEC in April 2018, 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 $200 million. To date, we have $137.6 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 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. Furthermore, if we obtain funds through a credit facility or through the issuance of debt or preferred

Table of Contents

securities, these securities would likely have rights senior to the rights of our common stockholders, which could impair the value of our common stock.

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

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

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

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

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

None.

Item 3.Defaults Upon Senior Securities

None.

Item 4.Mine Safety Disclosures

Not applicable.

Item 5.Other Information

None.

Table of Contents

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+#	Executive Severance Plan
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

+ 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.

Table of Contents

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: May 1, 2018

By: /s/ NELSON D. CABATUAN
Nelson D. Cabatuan
Interim Principal Accounting Officer

Date: May 1, 2018