RIGEL PHARMACEUTICALS INC
Form 10-Q
August 08, 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 JUNE 30, 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 August 2, 2018, there were 166,443,442 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 JUNE 30 2018

INDEX

Table of Contents

PART I. FINANCIAL INFORMATION

Item 1.Financial Statements

RIGEL PHARMACEUTICALS, INC.

CONDENSED BALANCE SHEETS

(In thousands)

	June 30, 2018 (unaudited)	December 31, 2017(1)
Assets		
Current assets:		
Cash and cash equivalents	$ 65,078	$ 38,290
Short-term investments	69,914	77,461
Accounts receivable, net	1,620	—
Inventories	494	—
Deferred rent	400	—
Prepaid and other current assets	1,551	1,682
Total current assets	139,057	117,433
Property and equipment, net	1,484	875
Other assets	678	803
	$ 141,219	$ 119,111
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,342	$ 2,636
Accrued compensation	5,440	7,059
Accrued research and development	5,494	5,028
Other accrued liabilities	3,089	3,330
Deferred liability – sublease, current portion	—	284
Total current liabilities	17,365	18,337
Long-term portion of deferred rent	249	90
Other long-term liabilities	38	38
Commitments		

Stockholders' equity:		
Preferred stock	—	—
Common stock	166	147
Additional paid-in capital	1,312,246	1,239,435
Accumulated other comprehensive loss	(49)	(82)
Accumulated deficit	(1,188,796)	(1,138,854)
Total stockholders' equity	123,567	100,646
	$ 141,219	$ 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 June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenues:				
Product sales, net	$ 1,787	$ —	$ 1,787	$ —
Contract revenues from collaborations	—	—	—	3,584
Total revenues	1,787	—	1,787	3,584
Costs and expenses:				
Cost of product sales	30	—	30	—
Research and development	10,797	11,524	22,039	23,900
Selling, general and administrative	17,071	7,820	30,563	15,230
Total costs and expenses	27,898	19,344	52,632	39,130
Loss from operations	(26,111)	(19,344)	(50,845)	(35,546)
Interest income	554	197	903	353
Gain on disposal of assets	—	—	—	732
Net loss	$ (25,557)	$ (19,147)	$ (49,942)	$ (34,461)
Net loss per share, basic and diluted	$ (0.16)	$ (0.16)	$ (0.32)	$ (0.29)
Weighted average shares used in computing net loss per share, basic and diluted	161,577	122,500	154,385	118,074

See Accompanying Notes.

Table of Contents

RIGEL PHARMACEUTICALS, INC.

CONDENSED STATEMENTS OF COMPREHENSIVE LOSS

(In thousands)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Net loss	$ (25,557)	$ (19,147)	$ (49,942)	$ (34,461)
Other comprehensive income (loss):				
Net unrealized gain (loss) on short-term investments	38	10	33	(1)
Comprehensive loss	$ (25,519)	$ (19,137)	$ (49,909)	$ (34,462)

See Accompanying Notes.

Table of Contents

RIGEL PHARMACEUTICALS, INC.

CONDENSED STATEMENTS OF CASH FLOWS

(In thousands)

(unaudited)

	Six Months Ended June 30,	
	2018	2017
Operating activities		
Net loss	$ (49,942)	$ (34,461)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation	2,652	2,055
Gain on disposal of assets	—	(732)
Loss on sublease	—	495
Depreciation and amortization	272	240
Net amortization of premium on short-term investment	(317)	(107)
Changes in assets and liabilities:		
Accounts receivable	(1,620)	—
Inventories	(476)	—
Prepaid and other current assets	131	(42)
Other assets	125	60
Accounts payable	706	(3,508)
Accrued compensation	(1,619)	(426)
Accrued research and development	466	(710)
Other accrued liabilities	(241)	(37)
Deferred rent and other long term liabilities	(525)	(3,059)
Net cash used in operating activities	(50,388)	(40,232)
Investing activities		
Purchases of short-term investments	(41,619)	(44,920)
Maturities of short-term investments	49,516	52,449
Proceeds from disposal of assets	—	732
Capital expenditures	(881)	(55)
Net cash provided by investing activities	7,016	8,206
Financing activities		
Net proceeds from issuances of common stock upon exercise of options and participation in employee stock purchase plan	2,998	810
Proceeds from sale and issuance of common stock, net of offering costs	67,162	46,175
Net cash provided by financing activities	70,160	46,985
Net increase in cash and cash equivalents	26,788	14,959
Cash and cash equivalents at beginning of period	38,290	17,632

Cash and cash equivalents at end of period	$ 65,078	$ 32,591

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. Our pioneering research focuses on signaling pathways that are critical to disease mechanisms.

Our first FDA approved product, TAVALISSE™ (fostamatinib disodium hexahydrate), an oral spleen tyrosine kinase (SYK) inhibitor, for the treatment of adult patients with chronic immune thrombocytopenia (ITP) who have had an insufficient response to a previous treatment, was approved by the U.S. Food and Drug Administration (FDA) in April 2018.

Our current clinical programs include Phase 2 studies of fostamatinib in autoimmune hemolytic anemia and IgA nephropathy, and a Phase 1 study for our interleukin receptor associated kinase (IRAK) program. In addition, we have product candidates in clinical development with partners BerGenBio AS, Daiichi Sankyo, and Aclaris Therapeutics.

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 Accounting Standards Codification (ASC) Topic 605, Revenue Recognition, and most industry-specific guidance under the ASC. Prior to January 1, 2018, 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 previous accounting standard in many respects, such as in the accounting for variable consideration, including milestone payments or contingent payments. Under our

Table of Contents

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.

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.

In March 2018, the FASB issued ASU No. 2018-05—Income Taxes (Topic 740): Amendments to SEC Paragraphs Pursuant to SEC Staff Accounting Bulletin No. 118 (SAB 118), which provides guidance on accounting for the tax effects of the U.S. Tax Cuts and Jobs Act (Tax Act) that was enacted in December 2017. SAB 118 provides a measurement period that should not extend beyond one year from the Tax Act enactment date for companies to complete the accounting. In accordance with this guidance, we have determined that $117.3 million of the deferred tax expense offset by a full valuation allowance recorded in connection with the remeasurement of certain deferred tax assets and liabilities was a provisional amount and a reasonable estimate at December 31, 2017. We have not made any additional measurement-period adjustments related to these items during the three and six months ended June 30, 2018 and will continue to assess future guidance issued by the U.S. Treasury Department, Internal Revenue Service (IRS), FASB, and other standard-setting and regulatory bodies. In accordance with ASU No. 2018-05, we will complete our accounting for the income tax effects of the Tax Act within the one-year measurement period in 2018 once we have fully analyzed all necessary information related to the Tax Act.

Inventories

Inventories are stated at the lower of cost or estimated net realizable value. We determine the cost of inventories using the standard cost method, which approximates actual cost based on a first-in, first-out (FIFO) basis. Inventories consist primarily of third-party manufacturing costs and allocated internal overhead costs. We began capitalizing inventory costs associated with our product upon regulatory approval when, based on management's judgment, future commercialization was considered probable and the future economic benefit was expected to be realized

Prior to FDA approval of TAVALISSE™ (fostamatinib disodium hexahydrate), all manufacturing costs were charged to research and development expense in the period incurred. At June 30, 2018, our physical inventory included active pharmaceutical product (API) of which costs have been previously charged to research and development expense.

Table of Contents

However, manufacturing of drug product, finished bottling and other labeling activities that occurred post FDA approval are included in the inventory value at June 30, 2018.

We provide reserves for potential excess, dated or obsolete inventories based on an analysis of forecasted demand compared to quantities on hand and any firm purchase orders, as well as product shelf life. At June 30, 2018, we determined that such reserves are not required.

Cost of Product Sales

Cost of product sales consists of third-party manufacturing costs, transportation and freight, and indirect overhead costs associated with the manufacture and distribution of TAVALISSE™ (fostamatinib disodium hexahydrate). A portion of the cost of producing the product sold to date was expensed as research and development prior to the Company's New Drug Application (NDA) approval for TAVALISSE and therefore is not included in the cost of product sales during this period.

Accounts Receivable

Accounts receivable are recorded net of customer allowances for prompt payment discounts and any allowance for doubtful accounts. We estimate the allowance for doubtful accounts based on existing contractual payment terms, actual payment patterns of its customers and individual customer circumstances. To date, we have determined that an allowance for doubtful accounts is not required.

Revenue Recognition

Revenues from product sales are recognized when the specialty distributors (SDs), who are our customers, obtain control of our product, which occurs at a point in time, upon delivery to such SDs. These SDs subsequently resell our products to specialty pharmacy providers, health care providers, hospitals and clinics. In addition to distribution agreements with these SDs, we also enter into arrangements with specialty pharmacy providers, in-office dispensing providers, group purchasing organizations, and government entities that provide for government-mandated and/or privately-negotiated rebates, chargebacks and discounts with respect to the purchase of our products. Prior to recognizing revenue, we make estimates of the transaction price, including variable consideration that is subject to a constraint. Amounts of variable consideration are included in the transaction price to the extent that 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. Revenue from product sales are recorded net of certain variable considerations including estimated government-mandated rebates and chargebacks, distribution fees,

estimated product returns and other deductions.

Provisions for returns and other adjustments are provided for in the period the related revenue is recorded. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known. The following are our significant categories of sales discounts and allowances:

Sales Discounts. We provide our customers prompt payment discounts that are explicitly stated in our contracts and are recorded as a reduction of revenue in the period the related product revenue is recognized.

Product Returns. We offer our SDs a right to return product purchased directly from us, which is principally based upon the product's expiration date. Product return allowances are estimated and recorded at the time of sale.

Government Rebates: We are subject to discount obligations under state Medicaid programs and Medicare prescription drug coverage gap program. We estimate our Medicaid and Medicare prescription drug coverage gap rebates based upon a range of possible outcomes that are probability-weighted for the estimated payor mix. These reserves are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability that is included as part of Other Accrued Liabilities account in the Balance Sheet. Our liability for these rebates consists primarily of estimates of claims for the current quarter, and estimated future

Table of Contents

claims that will be made for product that has been recognized as revenue, but remains in the distribution channel inventories at the end of each reporting period.

Chargebacks and Discounts: Chargebacks for fees and discounts represent the estimated obligations resulting from contractual commitments to sell products to certain specialty pharmacy providers, in-office dispensing providers, group purchasing organizations, and government entities at prices lower than the list prices charged to our SDs who directly purchase the product from us. These SDs charge us for the difference between what they pay for the product and our contracted selling price to these specialty pharmacy providers, in-office dispensing providers, group purchasing organizations, and government entities. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue. Chargeback amounts are generally determined at the time of resale to the specialty pharmacy providers, in-office dispensing providers, group purchasing organizations, and government entities by our SDs.

Co-Payment Assistance: We offer co-payment assistance to commercially insured patients meeting certain eligibility requirements. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that we expect to receive associated with product that has been recognized as revenue.

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 not related to our approved drug, purchased for us by third parties are expensed at the time of purchase.

4. Stock Award Plans

On May 16, 2018, our stockholders approved the adoption of the Company’s 2018 Equity Incentive Plan (2018 Plan). The 2018 Plan is the successor plan to the 2011 Equity Incentive Plan, the 2000 Equity Incentive Plan, and the 2000 Non-Employee Directors' Stock Option Plan.

To date, we have two stock option plans, our 2018 Plan and the Inducement Plan (collectively, the Equity Incentive Plans), 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

Table of Contents

event actually occurring, we recognize the related stock-based compensation expense when the event occurs or when we can determine that the performance condition is probable of achievement. In those cases, we recognize the change in estimate at the time we determine the condition is probable of achievement (by recognizing stock-based compensation expense as cumulative catch-up adjustment as if we had estimated at the grant date that the performance condition would have been achieved) and recognize the remaining compensation cost up to the date when we expect the performance condition will be achieved, if any.

5.Net Loss Per Share

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding during the period and the number of additional shares of common stock that would have been outstanding if potentially dilutive securities had been issued. Potentially dilutive securities include 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 June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Outstanding stock options	21,762	21,958	21,762	21,958
Purchase Plan	257	187	163	124
Total	22,019	22,145	21,925	22,082

6.Stock-Based Compensation

Total stock-based compensation related to all of our share-based payments that we recognized for the three and six months ended June 30, 2018 and 2017 were as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Selling, general and administrative	$ 779	$ 764	$ 1,719	$ 1,359
Research and development	333	336	933	696
Total stock-based compensation	$ 1,112	$ 1,100	$ 2,652	$ 2,055

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.

Table of Contents

· Expected term—For options granted to consultants, we use the contractual term of the option, which is generally ten years, for the initial valuation of the option and the remaining contractual term of the option for the succeeding periods. We analyzed various historical data to determine the applicable expected term for each of the other option groups. This data included: (1) for exercised options, the term of the options from option grant date to exercise date; (2) for cancelled options, the term of the options from option grant date to cancellation date, excluding non-vested option forfeitures; and (3) for options that remained outstanding at the balance sheet date, the term of the options from option grant date to the end of the reporting period and the estimated remaining term of the options. The consideration and calculation of the above data gave us reasonable estimates of the expected term for each employee group. We also considered the vesting schedules of the options granted and factors surrounding exercise behavior of the option groups, our current market price and company activity that may affect our market price. In addition, we considered the optionee type (i.e., officers and directors or all other employees) and other factors that may affect the expected term of the 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 and six months ended June 30, 2018 and 2017:

	Three Months Ended June 30,				Six Months Ended June 30,			
	2018		2017		2018		2017	
Risk-free interest rate	2.9	%	2.1	%	2.7	%	2.2	%
Expected term (in years)	6.6		6.6		6.7		6.8	
Dividend yield	0.0	%	0.0	%	0.0	%	0.0	%
Expected volatility	66.2	%	63.7	%	65.0	%	63.0	%

Starting in May 2018, the exercise price of stock options granted under our 2018 Plan is at the market price of our common stock on the date of grant. The exercise price of stock options under our prior equity incentive plans 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 4,486,225 shares of common stock during the six months ended June 30, 2018 with a grant-date weighted-average fair value of $2.68 per share. As of June 30, 2018, we have 2,790,000 shares related to outstanding performance-based stock option awards with a grant date fair value of $5.9 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 that have vested or wherein the achievement of the corresponding

corporate-based milestones was probable. Accordingly, we recognized $1.6 million in stock-based compensation expense, of which $488,000 was recorded during the six months ended June 30, 2018.

As of June 30, 2018, there was approximately $15.5 million of unrecognized stock-based compensation cost related to all unvested time-based and performance-based stock options granted under our equity incentive plans, of which approximately $4.3 million related to the unvested performance stock options.

At June 30, 2018, there were 14,597,716 shares of common stock available for future grant under our equity incentive plan and 823,130 options to purchase shares were exercised during the six months ended June 30, 2018.

Table of Contents

Employee Stock Purchase Plan

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

The fair value of awards granted under our Purchase Plan is estimated on the date of grant using the Black-Scholes option pricing model, which uses weighted-average assumptions. Our Purchase Plan provides for a twenty-four month offering period comprised of four six-month purchase periods with a look-back option. A look-back option is a provision in our Purchase Plan under which eligible employees can purchase shares of our common stock at a price per share equal to the lesser of 85% of the fair market value on the first day of the offering period or 85% of the fair market value on the purchase date. Our Purchase Plan also includes a feature that provides for a new offering period to begin when the fair market value of our common stock on any purchase date during an offering period falls below the fair market value of our common stock on the first day of such offering period. This feature is called a “reset.” Participants are automatically enrolled in the new offering period.

As of June 30, 2018, there were 1,790,451 shares reserved for future issuance under the Purchase Plan and there were $2.2 million of unrecognized stock-based compensation cost related to our employee stock purchase plan. The following table summarizes the weighted-average assumptions related to our Purchase Plan for the three and six months ended June 30, 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.

	Six Months Ended June 30,			
	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.Revenue from Product Sales

Our first FDA approved product, TAVALISSE™ (fostamatinib disodium hexahydrate), an oral spleen tyrosine kinase (SYK) inhibitor, for the treatment of adult patients with chronic ITP who have had an insufficient response to a previous treatment, was approved by the U.S. FDA in April 2018. We commenced commercial sale of TAVALISSE in the U.S. in May 2018. We recorded net product sales of $1.8 million during the three and six months ended June 30, 2018 related to the sale of TAVALISSE.

In addition to the distribution agreements with our customers, the SDs, we also enter into arrangements with specialty pharmacy providers, in-office dispensing providers, group purchasing organizations, and government entities that provide for government-mandated and/or privately-negotiated rebates, chargebacks and discounts with respect to the purchase of our products which reduced our gross product sales. Also refer to Revenue Recognition policy discussion in Note 3. The following table summarizes the provisions related to our current product sales for the six-month ended June 30, 2018 (in thousands). There were no credits or payments made during the six months ended June 30, 2018 related to these revenue reserves.

	June 30, 2018
Chargebacks, discounts and fees	$ 234
Government and other rebates	220
Returns	46
Total	$ 500

Table of Contents

The above provisions, which represent our contract liability as of June 30, 2018, are included as part of Other Accrued Liabilities in the balance sheet.

8.Inventories

The following table summarizes inventories as of June 30, 2018 and December 31, 2017 (in thousands):

	June 30, 2018	December 31, 2017
Work in process	$ 222	$ —
Finished goods	272	—
Total inventories	$ 494	$ —

9.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 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 July 2018, BMS

informed us that they will be terminating their preclinical collaboration with us.

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.

Table of Contents

10.Cash, Cash Equivalents and Short-Term Investments

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

	June 30, 2018	December 31, 2017
Cash	$ 964	$ 582
Money market funds	8,165	2,795
U.S. treasury bills	5,240	6,726
Government-sponsored enterprise securities	4,184	7,826
Corporate bonds and commercial paper	116,439	97,822
	$ 134,992	$ 115,751
Reported as:		
Cash and cash equivalents	$ 65,078	$ 38,290
Short-term investments	69,914	77,461
	$ 134,992	$ 115,751

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

June 30, 2018	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
U.S. treasury bills	$ 5,247	$ —	$ (7)	$ 5,240
Government-sponsored enterprise securities	4,194	—	(10)	4,184
Corporate bonds and commercial paper	116,471	7	(39)	116,439
Total	$ 125,912	$ 7	$ (56)	$ 125,863

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

As of June 30, 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 June 30, 2018 through their respective maturity dates. At June 30, 2018, we had no investments that had been in a continuous unrealized loss position for more than 12 months. As of June 30, 2018, a total of 27 individual securities had been in an unrealized loss position for 12 months or less, and the losses were determined to be temporary. The gross unrealized losses above were caused by interest rate increases. No significant facts or circumstances have arisen to indicate that there has been any significant deterioration in the creditworthiness of the issuers of the securities held by us. Based on our review of these securities, including the assessment of the duration and severity of the unrealized losses and our ability and intent to hold the investments until maturity, there were no other-than-temporary impairments for these securities at June 30, 2018.

Table of Contents

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

June 30, 2018	Fair Value	Unrealized Losses
U. S. treasury bills	$ 5,240	$ (7)
Government-sponsored enterprise securities	4,184	(10)
Corporate bonds and commercial paper	33,654	(39)
Total	$ 43,078	$ (56)

11.Fair Value

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

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

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

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

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

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

Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities and which reflect management's best estimate of what market participants would use in pricing the asset or liability at the reporting date. Consideration is given to the risk inherent in the valuation technique and the risk inherent in the inputs to the model.

We do not have fair valued assets 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):

Table of Contents

	Assets at Fair Value as of June 30, 2018			
	Level 1	Level 2	Level 3	Total
Money market funds	$ 8,165	$ —	$ —	$ 8,165
U.S. treasury bills	—	5,240	—	5,240
Government-sponsored enterprise securities	—	4,184	—	4,184
Corporate bonds and commercial paper	—	116,439	—	116,439
Total	$ 8,165	$ 125,863	$ —	$ 134,028

	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

12.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 $20.2 million in future sublease income (excluding our subtenant’s share of facilities operating expenses) through January 2023.

13.Common Stock

Authorized Shares of Common Stock

On May 18, 2018, we amended our Certificate of Incorporation (the “Charter Amendment”) to increase the number of authorized shares of common stock from 200,000,000 to 400,000,000 shares. This Charter Amendment was approved by our stockholders at the annual meeting held on May 16, 2018. The Charter Amendment became effective upon the filing with the Secretary of State of the State of Delaware on May 18, 2018.

Common Stock Public Offering

In the second quarter of 2018, we completed an underwritten public offering in which we sold 18,400,000 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 $67.2 million after deducting underwriting discounts and commissions and offering expenses.

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 and six months ended June 30, 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 ongoing commercial launch of TAVALISSE in the U.S., 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, and a Phase 1 study for our IRAK program. 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, contract payments under our collaboration agreements and recently, product sales from TAVALISSE. Our research and development activities, including preclinical studies and clinical trials, consume substantial amounts of capital. As of June 30, 2018, we had approximately $135.0 million in cash, cash equivalents and short term investments. In April 2018, we completed an underwritten public offering in which we sold 18,400,000 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 $67.2 million after deducting underwriting discounts and commissions and offering expenses.

We believe that our existing capital resources will be sufficient to support our current and projected funding requirements, including our ongoing 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

In May 2018, we announced that TAVALISSE™ (fostamatinib disodium hexahydrate) was available by prescription for the treatment of thrombocytopenia in adult patients with chronic ITP who have had an insufficient response to a previous treatment. TAVALISSE, an oral SYK inhibitor that targets the underlying autoimmune cause of the disease by impeding platelet destruction, was approved by the U.S. FDA in April 2018. We plan to enter into partnership with third parties to commercialize fostamatinib in Europe and Asia.

Our revenues have consisted of revenues from sponsored research and license agreements with our corporate collaborators and recently, product sales from TAVALISSE. Our potential future revenues may include product sales from TAVALISSE, and 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.

TAVALISSE™ (fostamatinib disodium hexahydrate) - ITP

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 TAVALISSE™ (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 launched TAVALISSE in the U.S. on our own in 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

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. To support these efforts, we have hired experienced commercial professionals, including sales representatives in the hematology area, and commercial operations, marketing, and market access professionals. In the ordinary course of business, we also entered into contractual agreements with third parties to support our commercial activities.

Competitive landscape for TAVALISSE

Our industry is intensely competitive and subject to rapid and significant technological change. TAVALISSE is competing with other 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 TAVALISSE.

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—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.

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. We expect to initiate our pivotal program in AIHA by the end of this year.

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

Table of Contents

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 in late 2018. We expect to meet with the FDA in August 2018 to determine the path forward for this program.

R835, an IRAK1/4 Inhibitor for Autoimmune and Inflammatory Diseases

During the second quarter of 2018, we selected R835, a proprietary molecule from our interleukin receptor associated kinase (IRAK) preclinical development program, for human clinical trials. This investigational candidate, R835, is an orally available, potent and selective inhibitor of IRAK1 and IRAK4 that blocks inflammatory cytokine production in response to toll-like receptor (TLR) and the interleukin-1 family receptor (IL-1R) signaling. TLRs and IL-1Rs play a critical role in the innate immune response and dysregulation of these pathways can lead to a variety of inflammatory conditions including psoriasis, rheumatoid arthritis, inflammatory bowel disease and gout (among others). R835 prevents cytokine release in response to TLR and IL-1R activation in vitro. R835 is active in multiple rodent models of inflammatory disease including psoriasis, arthritis, lupus, multiple sclerosis and gout. Preclinical studies show that R835 inhibits both the IRAK1 and IRAK4 signaling pathways, which play a key role in inflammation and immune responses to tissue damage. Dual inhibition of IRAK1 and IRAK4 allows for more complete suppression of pro-inflammatory cytokine release.

We initiated a Phase 1 study to assess safety, tolerability, pharmacokinetics and pharmacodynamics of R835 in healthy subjects in the second quarter of 2018. This Phase 1 study is a randomized, placebo-controlled, double-blind trial in up to 91 healthy subjects, ages 18 to 55. The study design aims to assess the tolerability and safety of R835 in both single ascending and multiple ascending doses.

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 alopecia areata (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.

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. In June 2018, Aclaris announced positive interim data from its Phase 2 clinical trial of ATI-502 for the treatment of Alopecia Totalis (AT) or Alopecia Universalis (AU), the more severe variants of AA. The randomized, double-blind clinical trial is being conducted at two study sites and will evaluate the pharmacokinetics, pharmacodynamics and safety of ATI-502 compared with vehicle in 11 patients with AT or AU over 28 days of treatment, followed by a 6-month open label period when all patients receive the drug.

Table of Contents

Pharmacokinetic Results:

- Systemic exposure was low as indicated by plasma drug levels that were below the limits of quantification (1ng/ml) in all subjects at day 28.
- Consistent with low systemic exposure, no significant changes in circulating T, B and NK cells were observed at day 28.
- To assess skin penetration, punch biopsies were obtained at baseline and day 28 which demonstrated topical ATI-502 absorption consistent with pre-clinical skin models.
- Mean of 5650 nanograms/gram at day 28 (range 3130 - 8170 ng/g).

Pharmacodynamic / RNA Sequencing Results:

- Two patients had marked elevation of the interferon gamma and cytotoxic T-cell gene expression signatures at baseline: one received active drug and one received vehicle. At day 28, the patient on active drug demonstrated a positive change in the two biomarkers, while the patient on vehicle did not demonstrate a positive change in the 2 biomarkers.
- Two patients had low elevation of the interferon gamma and cytotoxic T-cell gene expression signatures at baseline: one received active drug, one received vehicle. At day 28 both patients showed a partial positive change in the two biomarkers.
- Two patients had no elevation of the interferon gamma and cytotoxic T-cell gene expression signatures at baseline: both patients received active drug. At day 28, neither patient showed any material change in the two biomarkers.

Safety

- No serious adverse events (SAEs) were reported during the 28-day dosing period. One subject receiving ATI-502 withdrew from the trial due to an unrelated SAE of major depression during the open label extension period.

The above interim data is the first indication that ATI-502 is absorbed through human skin in the clinical setting and engages the target. The results also demonstrate the pharmacodynamic effect of modulating the appropriate genes associated with the interferon gamma pathway and cytotoxic T-lymphocytes, which are 2 of 3 biomarker components of the Alopecia Areata Disease Activity Index (ALADIN) score.

In July 2018, Aclaris also announced that the FDA has granted Fast Track designation to ATI-502 for the treatment of AA, including patchy AA and the more severe variants of the disease, AT and AU. Aclaris recently initiated its Phase 2 trials for ATI-501 in the second half of 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 acute myeloid leukemia (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 AML and myelodysplastic syndrome (MDS); and in combination with erlotinib (Tarceva®) in advanced (EGFR-positive) NSCLC. BerGenBio is also conducting 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

Table of Contents

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.

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.

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.

Table of Contents

Results of Operations

Three and Six Months Ended June 30, 2018 and 2017

Revenues

	Three Months Ended June 30,			Six Months Ended June 30,		
	2018	2017	Aggregate Change	2018	2017	Aggregate Change
		(in thousands)			(in thousands)	
Product sales, net	$ 1,787	$ —	$ 1,787	$ 1,787	$ —	$ 1,787
Contract revenues from collaborations	—	—	—	—	3,584	(3,584)
Total revenues	$ 1,787	$ —	$ 1,787	$ 1,787	$ 3,584	$ (1,797)

Product sales during the three and six months ended June 30, 2018 relates to sales of TAVALISSE™ (fostamatinib disodium hexahydrate) in the U.S. There were no product sales during the three and six months ended June 30, 2017.

There were no contract revenues from collaborations during the three months ended June 30, 2018 and 2017. Contract revenues from collaborations of $3.6 million during the six months ended June 30, 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, to a Phase 2 clinical study.

Our potential future revenues may include product sales from TAVALISSE, 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.

Cost of Product Sales

	Three Months Ended June 30,			Six Months Ended June 30,		
	2018	2017	Aggregate Change	2018	2017	Aggregate Change
		(in thousands)			(in thousands)	

Cost of product sales	$ 30	$ —	$ 30	$ 30	$ —	$ 30

We recognized $30,000 in cost of sales related to our product, TAVALISSE, which was approved by the FDA in April 2018. Prior to the FDA approval, manufacturing and related costs were charged to reseach and development expense. Therefore, these costs were not capitalized and as a result, are not fully reflected in the costs of sales during the current period. We will continue to have a lower cost of product sales that excludes the cost of the API that was produced prior to FDA approval until we sell TAVALISSE that includes newly manufactured API. We expect that this will be the case for the near-term and as a result, our cost of product sales in the near-term will be less than we anticipate it will be in future periods. As we produce TAVALISSE in the future, our inventory cost in the Balance Sheet and Cost of Product Sales will increase as that would reflect the full cost of manufacturing.

Research and Development Expense

	Three Months Ended June 30,			Six Months Ended June 30,		
	2018	2017	Aggregate Change	2018	2017	Aggregate Change
		(in thousands)			(in thousands)	
Research and development expense	$ 10,797	$ 11,524	$ (727)	$ 22,039	$ 23,900	$ (1,861)
Stock-based compensation expense included in research and development expense	$ 333	$ 336	$ (3)	$ 933	$ 696	$ 237

The decreases in research and development expense for the three months ended June 30, 2018, compared to the same period in 2017, were primarily due to the completion of our pivotal Phase 3 clinical trials in ITP in 2017, as well as

Table of Contents

the completion of the submission of our NDA for fostamatinib in ITP in 2017 of $1.9 million, and allocated facility costs of $675,000, partially offset by increases in personnel costs of $781,000 and research and development costs for our clinical trials in IRAK and AIHA programs of $943,000 and $421,000, respectively.

The decreases in research and development expense for the six months ended June 30, 2018, compared to the same period in 2017, were primarily due the completion of our pivotal Phase 3 clinical trials in ITP as well as the completion of the related submission of our NDA for fostamatinib in ITP in 2017 of $4.8 million and allocated facility costs of $1.0 million, partially offset by increases in certain personnel costs of $2.1 million and research and development costs for our clinical trials in IRAK and AIHA programs of $1.4 million and $602,000, respectively.

We expect our research and development expense in the second half of 2018 to increase compared with the previous quarters in 2018.

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

We do not track fully burdened research and development costs separately for each of our drug candidates. We review our research and development expenses by focusing on three categories: research, development, and other. Our research team is focused on creating a portfolio of product candidates that can be developed into small molecule therapeutics in our own proprietary programs or with potential collaborative partners and utilizes our robust discovery engine to rapidly discover and validate new product candidates in our focused range of therapeutic indications. “Research” expenses relate primarily to personnel expenses, lab supplies, fees to third party research consultants and compounds. Our development group leads the implementation of our clinical and regulatory strategies and prioritizes disease indications in which our compounds may be studied in clinical trials. “Development” expenses relate primarily to clinical trials, personnel expenses, costs related to the submission and management of our NDA, lab supplies and fees to third party research consultants. “Other” expenses primarily consist of allocated facilities costs and allocated stock based compensation expense relating to personnel in research and development groups.

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

We do not have reliable estimates regarding the timing of our clinical trials. Preclinical testing and clinical development are long, expensive and uncertain processes. In general, biopharmaceutical development involves a

series of steps, beginning with identification of a potential target and including, among others, proof of concept in animals and Phase 1, 2 and 3 clinical trials in humans. Significant delays in clinical testing could materially impact our product development costs and timing of completion of the clinical trials. We do not know whether planned clinical trials will begin on time, will need to be halted or revamped or will be completed on schedule, or at all. Clinical trials can be delayed for a variety of reasons, including delays in obtaining regulatory approval to commence a trial, delays from scale up, delays in reaching agreement on acceptable clinical trial agreement terms with prospective clinical sites, delays in 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.

Table of Contents

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

	Three Months Ended June 30,		Six Months Ended June 30,		
	2018	2017	2018	2017	From January 1, 2007* to June 30, 2018
Categories:					
Research	$ 2,431	$ 2,500	$ 4,938	$ 5,127	$ 231,304
Development	6,952	6,939	13,590	14,471	355,759
Other	1,414	2,085	3,511	4,302	233,837
	$ 10,797	$ 11,524			