

PORTOLA PHARMACEUTICALS INC  
Form 10-Q  
August 05, 2015

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, 2015

OR

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

For the transition period from \_\_\_\_\_ to \_\_\_\_\_

Commission file number: 001-35935

PORTOLA PHARMACEUTICALS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware 20-0216859  
(State or Other Jurisdiction of Incorporation or Organization) (I.R.S. Employer Identification No.)

270 E. Grand Avenue

South San Francisco, California 94080  
(Address of Principal Executive Offices) (Zip Code)  
(650) 246-7000

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(Registrant's Telephone Number, Including Area Code)

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

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

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

Large accelerated filer ☒

Accelerated filer ☐

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

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

As of July 30, 2015, the number of outstanding shares of the registrant's common stock, par value \$0.001 per share, was 52,563,503.

PORTOLA PHARMACEUTICALS, INC.

FORM 10-Q FOR THE QUARTER ENDED JUNE 30, 2015

INDEX

	Page
Part I — <u>Financial Information</u>	
<u>Item 1. Condensed Consolidated Financial Statements</u>	F-3
<u>Condensed Consolidated Balance Sheets-June 30,2015(unaudited) and December 31, 2014</u>	F-3
<u>Condensed Consolidated Statements of Operations (Unaudited)-three and six month ended June 30,2015 and 2014</u>	F-4
<u>Condensed Consolidated Statements of Comprehensive Loss (Unaudited)-three and six month ended June 30,2015 and 2014</u>	F-5
<u>Condensed Consolidated Statements of Cash Flows (Unaudited)-six months ended June 30, 2015 and 2014</u>	F-6
<u>Notes to Condensed Consolidated Financial Statements (Unaudited)</u>	F-7
<u>Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations</u>	20
<u>Item 3. Quantitative and Qualitative Disclosures About Market Risk</u>	30
<u>Item 4. Controls and Procedures</u>	31
Part II — <u>Other Information</u>	
<u>Item 1. Legal Proceedings</u>	32
<u>Item 1A. Risk Factors</u>	32

<u>Item 2. Unregistered Sales of Equity Securities and Use of Proceeds</u>	55
<u>Item 5. Other Information</u>	55
<u>Item 6. Exhibits</u>	55
<u>Signatures</u>	56

ITEM 1. FINANCIAL STATEMENTS  
PORTOLA PHARMACEUTICALS, INC.

Condensed Consolidated Balance Sheets

(In thousands, except share and per share data)

	June 30, 2015 (Unaudited)	December 31, 2014
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 68,911	\$ 57,514
Short-term investments	332,596	251,759
Receivables from collaborators	—	57
Prepaid expenses and other current assets	15,967	5,747
<b>Total current assets</b>	<b>417,474</b>	<b>315,077</b>
Property and equipment, net	4,584	2,776
Long-term investments	6,091	83,030
Prepaid and other long-term assets	13,910	15,612
<b>Total assets</b>	<b>\$ 442,059</b>	<b>\$ 416,495</b>
<b>Liabilities and stockholders' equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 12,625	\$ 14,084
Accrued compensation and employee benefits	2,989	3,512
Accrued and other liabilities	24,492	13,966
Deferred revenue, current portion	9,021	9,569
<b>Total current liabilities</b>	<b>49,127</b>	<b>41,131</b>
Deferred revenue, long-term	22,820	27,016
Other long-term liabilities	2,079	546
<b>Total liabilities</b>	<b>74,026</b>	<b>68,693</b>
<b>Stockholders' equity:</b>		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized at June 30,		
2015 and December 31, 2014; no shares issued and outstanding at June 30,		
2015 and December 31, 2014		
	—	—
Common stock, \$0.001 par value, 100,000,000 shares authorized at June 30,		
2015 and December 31, 2014; 52,394,531 and 48,766,806 shares issued and		
outstanding at June 30, 2015 and December 31, 2014, respectively		
	52	49

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Additional paid-in capital	896,110	770,789
Accumulated deficit	(528,038 )	(422,797)
Accumulated other comprehensive loss	(91 )	(239 )
Total stockholders' equity	368,033	347,802
Total liabilities and stockholders' equity	\$ 442,059	\$ 416,495

See accompanying notes to the unaudited condensed consolidated financial statements.

F-3

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## PORTOLA PHARMACEUTICALS, INC.

## Condensed Consolidated Statements of Operations

(Unaudited)

(In thousands, except share and per share data)

	Three Months Ended		Six Months Ended	
	June 30, 2015	2014	June 30, 2015	2014
Collaboration and license revenue	\$2,385	\$2,415	\$4,744	\$4,786
Operating expenses:				
Research and development	52,300	28,983	92,158	57,138
Selling, general and administrative	8,912	4,937	17,917	10,177
Total operating expenses	61,212	33,920	110,075	67,315
Loss from operations	(58,827 )	(31,505 )	(105,331 )	(62,529 )
Interest and other income, net	498	155	89	453
Net loss	\$(58,329 )	\$(31,350 )	\$(105,242 )	\$(62,076 )
Net loss per share attributable to common stockholders:				
Basic and diluted	\$(1.12 )	\$(0.76 )	\$(2.07 )	\$(1.51 )
Shares used to compute net loss per share attributable to common stockholders:				
Basic and diluted	52,147,146	41,228,885	50,844,697	41,119,310

See accompanying notes to the unaudited condensed consolidated financial statements.

PORTOLA PHARMACEUTICALS, INC.

Condensed Consolidated Statements of Comprehensive Loss

(Unaudited)

(In thousands)

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2015	2014	2015	2014
Net loss	\$ (58,329)	\$ (31,350)	\$ (105,242)	\$ (62,076)
Other comprehensive income (loss):				
Unrealized gain (loss) on available-for-sale securities, net of				
tax	(21 )	(5 )	148	(33 )
Total comprehensive loss	\$ (58,350)	\$ (31,355)	\$ (105,094)	\$ (62,109)

See accompanying notes to the unaudited condensed consolidated financial statements.



## PORTOLA PHARMACEUTICALS, INC.

## Condensed Consolidated Statements of Cash Flows

(Unaudited)

(In thousands)

	Six Months Ended	
	June 30, 2015	2014
Operating activities		
Net loss	\$(105,242)	\$(62,076 )
Adjustments to reconcile net loss to cash used in operating activities:		
Depreciation and amortization	629	742
Amortization of premium on investment securities	1,803	1,848
Stock-based compensation expense	9,931	4,726
Gain on retirement of equipment	(11 )	—
Unrealized loss on foreign currency forward contracts	—	1
Changes in operating assets and liabilities:		
Receivables from collaborators	57	127
Prepaid expenses and other current assets	(10,220 )	(1,276 )
Prepaid and other long-term assets	1,702	47
Accounts payable	(1,341 )	2,701
Accrued compensation and employee benefits	(538 )	(321 )
Accrued and other liabilities	10,526	1,521
Deferred revenue	(4,744 )	18,214
Other long-term liabilities	1,533	514
Net cash used in operating activities	(95,915 )	(33,232 )
Investing activities		
Purchases of property and equipment	(2,555 )	(963 )
Proceeds from sale of equipment	11	—
Purchases of investments	(167,652)	(143,493)
Proceeds from sales of investments	—	3,000
Proceeds from maturities of investments	162,099	111,016
Net cash used in investing activities	(8,097 )	(30,440 )
Financing activities		
Proceeds from public offering of common stock, net of underwriters discount	108,772	—
Payment of public offering cost	(358 )	—
Proceeds from issuance of common stock pursuant to equity award plans	6,995	2,967
Net cash provided by financing activities	115,409	2,967
Net increase (decrease) in cash and cash equivalents	11,397	(60,705 )
Cash and cash equivalents at beginning of period	57,514	117,773

Cash and cash equivalents at end of period	\$68,911	\$57,068
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See accompanying notes to the unaudited condensed consolidated financial statements.

## PORTOLA PHARMACEUTICALS, INC.

### Notes to Condensed Consolidated Financial Statements

#### 1. Organization

Portola Pharmaceuticals, Inc. (the “Company” or “we” or “our” or “us”) is a biopharmaceutical company focused on the development and commercialization of novel therapeutics in the areas of thrombosis, other hematologic diseases and inflammation for patients who currently have limited or no approved treatment options. We were incorporated in September 2003 in Delaware. Our headquarters and operations are located in South San Francisco, California and we operate in one segment.

Our Phase 3 programs address significant unmet medical needs in the area of thrombosis, or blood clots. Betrixaban is a novel oral once-daily inhibitor of Factor Xa in a Phase 3 clinical trial for extended duration prophylaxis, or preventive treatment, of a form of thrombosis known as venous thromboembolism, in acute medically ill patients for 35 days of in-hospital and post-discharge use. Our second Phase 3 compound, Andexanet alfa, an FDA-designated breakthrough therapy and orphan drug, is a recombinant protein designed to reverse anticoagulant activity in patients treated with a Factor Xa inhibitor. Andexanet alfa has potential indications to reverse anticoagulant activity in patients taking a direct or indirect Factor Xa inhibitor who suffer a major bleeding episode or require emergency surgery. Our third product candidate Cerdulatinib, is an orally available kinase inhibitor that inhibits spleen tyrosine kinase, or Syk, and janus kinases, or JAK, enzymes that regulate important signaling pathways and is being developed for hematologic, or blood, cancers and inflammatory disorders. We are currently in a Phase 1/2a proof-of-concept study with Cerdulatinib in non-Hodgkin’s lymphoma and chronic lymphocytic leukemia. In the Phase 1 dose escalation portion of the study, we have yet to reach the maximum tolerated dose and enrollment continues. Based on interim Phase 1 data, we are advancing Cerdulatinib to the Phase 2a portion of the study which includes expansion cohorts in select hematologic cancers. We also have a proprietary Syk specific inhibitor partnered with Ora, Inc. or Ora.

In March 2015, we completed an underwritten public offering of 2,870,000 shares of our common stock, which included 374,348 shares of common stock issued pursuant to the over-allotment option granted to our underwriters, at a public offering price of \$40.00 per share. The net proceeds from the offering to us including the over-allotment option, net of underwriting discounts, commissions and offering expenses of approximately \$358,000, were approximately \$108.4 million.

#### 2. Summary of Significant Accounting Policies

##### Consolidation and Basis of Presentation

The accompanying unaudited condensed consolidated financial statements include the amounts of the Company and our wholly-owned subsidiary and have been prepared in accordance with U.S. generally accepted accounting principles (“U.S. GAAP”), and follow the requirements of the Securities and Exchange Commission (“SEC”) for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required

by U.S. GAAP can be condensed or omitted. These condensed consolidated financial statements have been prepared on the same basis as our annual consolidated financial statements and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments that are necessary for a fair statement of our financial information. The results of operations for the three and six months ended June 30, 2015 are not necessarily indicative of the results to be expected for the year ending December 31, 2015. The condensed consolidated balance sheet as of December 31, 2014 has been derived from audited financial statements at that date but does not include all of the information required by U.S. GAAP for complete financial statements.

The accompanying condensed consolidated financial statements and related financial information should be read in conjunction with the audited financial statements and the related notes thereto for the year ended December 31, 2014 included in our Annual Report on Form 10-K filed March 2, 2015 with the SEC.

#### Use of Estimates

The preparation of condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent assets and liabilities and the reported amounts of revenues and expenses in the condensed consolidated financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including those related to revenue recognition, clinical trial accruals, fair value of assets and liabilities, income taxes and stock-based compensation. Management bases its estimates on historical experience and on various other market-specific and relevant assumptions that management believes to be reasonable under the circumstances. Actual results may differ from those estimates.

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

## Variable Interest Entities

We review agreements we enter into with third party entities, pursuant to which we may have a variable interest in the entity, in order to determine if the entity is a variable interest entity (VIE). If the entity is a VIE, we assess whether or not we are the primary beneficiary of that entity. In determining whether we are the primary beneficiary of an entity, we apply a qualitative approach that determines whether we have both (1) the power to direct the economically significant activities of the entity and (2) the obligation to absorb losses of, or the right to receive benefits from, the entity that could potentially be significant to that entity. If we determine we are the primary beneficiary of a VIE, we consolidate the statements of operations and financial condition of the VIE into our consolidated financial statements.

Our determination about whether we should consolidate such VIEs is made continuously as changes to existing relationships or future transactions may result in a consolidation or deconsolidation event.

## Cash and Cash Equivalents

Cash and cash equivalents consist of cash and other highly liquid investments with original maturities of three months or less from the date of purchase.

## Investments

All investments have been classified as “available-for-sale” and are carried at estimated fair value as determined based upon quoted market prices or pricing models for similar securities. Management determines the appropriate classification of its investments in debt securities at the time of purchase and reevaluates such designation as of each balance sheet date. Unrealized gains and losses are excluded from earnings and are reported as a component of accumulated comprehensive loss. Realized gains and losses and declines in fair value judged to be other than temporary, if any, on available-for-sale securities are included in interest and other income (expense), net. The cost of securities sold is based on the specific-identification method. Interest on marketable securities is included in interest and other income (expense), net.

## Customer Concentration

Customers who accounted for 10% or more of total collaboration and license revenues were as follows:

	Three Months Ended			Six Months Ended		
	June 30,			June 30,		
	2015	2014	%	2015	2014	%
Bristol-Myers Squibb Company and Pfizer Inc.	16%	21	%	16%	20	%
Bayer Pharma, AG and Janssen Pharmaceuticals, Inc.	34%	49	%	34%	44	%

## Revenue Recognition

We generate revenue from collaboration and license agreements for the development and commercialization of our products. Collaboration and license agreements may include non-refundable or partially refundable upfront license fees, partial or complete reimbursement of research and development costs, contingent consideration payments based on the achievement of defined collaboration objectives and royalties on sales of commercialized products. Our performance obligations under our collaborations include the transfer of intellectual property rights (licenses), obligations to provide research and development services and related clinical drug supply, obligation to provide regulatory approval services and obligations to participate on certain development and/or commercialization committees with the collaborators. Upfront payments are recorded as deferred revenue in our condensed consolidated balance sheet and are recognized as collaboration revenue over our estimated period of performance that is consistent with the terms of the research and development obligations contained in each collaboration agreement. We regularly review the estimated periods of performance related to our collaborations based on the progress made under each arrangement. Our estimates of our performance period may change over the course of the collaboration term. Such a change could have a material impact on the amount of revenue we record in future periods.

PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

Payments that are contingent upon achievement of a substantive milestone are recognized in their entirety in the period in which the milestone is achieved. A milestone is defined as an event that can only be achieved based on our performance and there is substantive uncertainty about whether the event will be achieved at the inception of the arrangement. Events that are contingent only on the passage of time or only on counterparty performance are not considered milestones subject to this guidance. Further, the amounts received must relate solely to prior performance, be reasonable relative to all of the deliverables and payment terms within the agreement and commensurate with our performance to achieve the milestone after commencement of the agreement. Payments contingent upon achievement of events that are not considered substantive milestones are allocated to the respective arrangements unit of accounting when received and recognized as revenue based on the revenue recognition policy for that unit of accounting.

Amounts from sales of licenses are recognized as revenue. Amounts received as funding of research and development or regulatory approval activities are recognized as revenue if the collaboration arrangement involves the sale of our research or development and regulatory approval services at amounts that exceed our cost. However, such funding is recognized as a reduction in research and development expense when we engage in a research and development project jointly with another entity, with both entities participating in project activities and sharing costs and potential benefits of the arrangement.

Amounts related to research and development and regulatory approval funding are recognized as the related services or activities are performed, in accordance with the contract terms. Payments may be made to or by us based on the number of full-time equivalent researchers assigned to the collaboration project and the related research and development expenses incurred.

Foreign Currency Transactions and Hedging

We have transactions denominated in foreign currencies, primarily the Euro and British Pound, and, as a result, are exposed to changes in foreign currency exchange rates. We manage a portion of these cash flow exposures through the purchase of Euros and the use of foreign currency forward contracts. Our foreign currency forward contracts are not designated as hedges for accounting purposes. Gains or losses on foreign currency forward contracts are intended to offset gains or losses on the underlying net exposures in an effort to reduce the earnings and cash flow volatility resulting from fluctuating foreign currency exchange rates. Foreign currency deposits are remeasured using period end spot rates. Foreign currency forward contracts are marked to market at the end of each period and recorded as gains and losses in the condensed consolidated statements of operations.

We held no foreign currency forward contracts at June 30, 2015 or December 31, 2014.

We recorded an unrealized loss of \$18,000 and \$1,000 in interest and other income (expense), net on our condensed statements of operations related to these foreign currency forward contracts for the three and six months ended June 30, 2014, respectively. During the three and six months ended June 30, 2014, we settled foreign currency forward contracts and recognized a realized loss of \$107,000 and \$250,000, respectively, in interest and other income (expense), net.

Our foreign exchange forward contracts expose us to credit risk to the extent that the counterparty, a major financial institution, is unable to meet the terms of the agreement. Our management does not expect material losses as a result of defaults by the counterparty.

#### Net Loss per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding for the period. Diluted net loss per share attributable to common stockholders is computed by giving effect to all potential dilutive common stock equivalents outstanding for the period. Diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since the effects of potentially dilutive securities are antidilutive.

F-9

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PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

Recent Accounting Pronouncements

In May 2014, the Financial Accounting Standards Board (the “FASB”), jointly with the International Accounting Standards Board, issued ASU 2014-09, Revenue from Contracts with Customers. On July 9, 2015, the FASB approved a one-year deferral of the effective date of this standard to 2018 for public companies, with an option that would permit companies to adopt the standard as early as the original effective date of 2017. Early adoption prior to the original effective date is not permitted. The new standard may be adopted either retrospectively or on a modified retrospective basis whereby the new standard would be applied to new contracts and existing contracts with remaining performance obligations as of the effective date, with a cumulative catch-up adjustment recorded to beginning retained earnings at the effective date for existing contracts with remaining performance obligations. We are currently evaluating the impact of our pending adoption of this standard on our consolidated financial statements.

In February 2015, the Financial Accounting Standards Board (the “FASB”) issued ASU 2015-02, Consolidation: amendment to the consolidation analysis that modifies existing consolidation guidance for reporting organizations that are required to evaluate whether they should consolidate certain legal entities. The new consolidation guidance is effective for the Company in the first quarter of fiscal 2016 and requires either a retrospective or a modified retrospective approach to adoption. Early adoption is permitted. We are currently evaluating the impact of our pending adoption of this standard on our consolidated financial statements.

### 3. Fair Value Measurements

Financial assets and liabilities are recorded at fair value. The carrying amounts of certain of our financial instruments, including cash and cash equivalents, investments, receivables from collaborators, prepaid expenses and other current assets and accounts payable, approximate their fair value due to their short term nature. The accounting guidance for fair value provides a framework for measuring fair value, clarifies the definition of fair value, and expands disclosures regarding fair value measurements. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability (an exit price) in an orderly transaction between market participants at the reporting date. The accounting guidance establishes a three-tiered hierarchy, which prioritizes the inputs used in the valuation methodologies in measuring fair value as follows:

Level 1 – Inputs are unadjusted, quoted prices in active markets for identical assets or liabilities at the measurement date.

Level 2 – Inputs (other than quoted market prices included in Level 1) are either directly or indirectly observable for the asset or liability through correlation with market data at the measurement date and for the duration of the instrument’s anticipated life.

Level 3 – Inputs reflect management’s best estimate of what market participants would use in pricing the asset or liability at the measurement date. Consideration is given to the risk inherent in the valuation technique and the risk

inherent in the inputs to the model.

The following table sets forth the fair value of our financial assets and liabilities, allocated into Level 1, Level 2 and Level 3, that were measured on a recurring basis (in thousands):

	June 30, 2015			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	\$20,483	\$—	\$ —	\$20,483
Corporate notes and commercial paper	—	214,566	—	214,566
U.S. government agency securities	—	155,805	—	155,805
Total financial assets	\$20,483	\$370,371	\$ —	\$390,854

  

	December 31, 2014			
	Level 1	Level 2	Level 3	Total
Financial Assets:				
Money market funds	\$24,915	\$—	\$ —	\$24,915
Corporate notes and commercial paper	—	226,047	—	226,047
U.S. government agency securities	—	120,169	—	120,169
Total financial assets	\$24,915	\$346,216	\$ —	\$371,131

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

We estimate the fair values of our corporate notes and commercial paper and U.S government agency securities by taking into consideration valuations obtained from third-party pricing services. The pricing services utilize industry standard valuation models, including both income- and market-based approaches, for which all significant inputs are observable, either directly or indirectly, to estimate fair value. These inputs include reported trades of and broker/dealer quotes on the same or similar securities; issuer credit spreads; benchmark securities; prepayment/default projections based on historical data; and other observable inputs.

There were no transfers between Level 1 and Level 2 during the periods presented.

## 4. Financial Instruments

Cash equivalents and investments, all of which are classified as available-for-sale securities, consisted of the following (in thousands):

	June 30, 2015				December 31, 2014			
	Cost	Unrealized Gain	Unrealized (Loss)	Estimated Fair Value	Cost	Unrealized Gain	Unrealized (Loss)	Estimated Fair Value
Money market funds	\$20,483	\$ —	\$ —	\$20,483	\$24,915	\$ —	\$ —	\$24,915
Corporate notes and commercial paper	214,666	5	(105 )	214,566	226,209	8	(170 )	226,047
U.S. government agency securities	155,796	26	(17 )	155,805	120,246	4	(81 )	120,169
	\$390,945	\$ 31	\$ (122 )	\$390,854	\$371,370	\$ 12	\$ (251 )	\$371,131
Classified as:								
Cash equivalents				\$52,167				\$36,342
Short-term investments				332,596				251,759
Long-term investments				6,091				83,030
Total cash equivalents and investments				\$390,854				\$371,131

At June 30, 2015 and December 31, 2014, the remaining contractual maturities of available-for-sale securities were less than two years. There have been no significant realized gains or losses on available-for-sale securities for the periods presented.

## 5. Balance Sheet Components

Accrued and other liabilities consist of the following (in thousands):

	June 30, 2015	December 31, 2014
Research and development related	\$22,263	\$ 12,545
Legal and accounting fees	419	354
Deferred rent	398	127
Commercial related	707	—
Other	705	940
Total accrued and other liabilities	\$24,492	\$ 13,966

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

## 6. Collaboration and License Agreements

## Summary of Collaboration-Related Revenue

We have recognized revenue from our collaboration and license agreements as follows (in thousands):

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
BMS and Pfizer	\$ 384	\$ 503	\$763	\$945
Bayer and Janssen	807	1,194	1,606	2,106
Lee's Pharmaceutical	52	63	104	125
Daiichi Sankyo	1,142	655	2,271	1,610
Total collaboration and license revenue	\$ 2,385	\$ 2,415	\$4,744	\$4,786

## Biogen Idec, Inc. ("Biogen Idec")

In October 2011, we entered into an exclusive, worldwide license and collaboration agreement with Biogen Idec to develop and commercialize selective, novel oral Syk inhibitors for the treatment of autoimmune and inflammatory diseases. In November 2012, we exercised our option to convert the agreement to a fully out-licensed agreement. After the election, we relinquished our right to share profits from sales of products related to PRT2607 and other selective Syk inhibitors, but were entitled to receive future payments up to approximately \$370.0 million based on the occurrence of certain development and regulatory events for all licensed compounds. In April 2014, we entered into an amendment to the Biogen Idec license and collaboration agreement under which Biogen Idec released one of the Syk kinase inhibitors to us and we would be required to pay Biogen Idec \$15.0 million upon the completion of certain commercial milestones and pay royalties on sales of products approved for the Syk inhibitor.

In May 2015, our agreement with Biogen Idec terminated in its entirety effective July 2015. The effect of termination will result in return to us of all compounds subject to the license and collaboration agreement and eliminate all potential future payments from and to Biogen Idec. We did not record any reduction to research and development expense pursuant to the agreement for the three and six months ended June 30, 2015. During the three and six months ended June 30, 2014 we recorded a reduction in research expense of \$86,000 and \$164,000, respectively.

## Bristol-Myers Squibb Company ("BMS") and Pfizer Inc. ("Pfizer")

In January 2014, we entered into a collaboration agreement with BMS and Pfizer to further study Andexanet alfa as a reversal agent for their jointly owned FDA approved oral Factor Xa inhibitor, apixaban, through Phase 3 studies. We initiated Phase 3 studies in the first half of 2014. We are responsible for the cost of conducting this clinical study. Pursuant to our agreement with BMS and Pfizer we are obligated to provide research, development and regulatory approval services and participate in the Joint Collaboration Committee ("JCC") in exchange for a partially refundable

upfront fee of \$13.0 million and up to \$12.0 million of contingent milestone payments due upon achievement of certain development and regulatory events. All consideration received and to be earned under this agreement is subject to a 50% refund contingent upon certain regulatory and/or clinical events.

We identified the following non-cancellable performance deliverables under the January 2014 agreement: 1) the obligation to provide research and development services, which include manufacturing and supplying Andexanet alfa and providing various reports, 2) the obligation to provide regulatory approval services, and 3) the obligation to participate on the JCC. We considered the provisions of the multiple-elements arrangement guidance in determining how to recognize the total agreement consideration. We determined that none of the deliverables have standalone value and all of these obligations will be delivered throughout the estimated period of performance and therefore are accounted for as a single unit of accounting. The non-contingent upfront consideration under this agreement of \$6.5 million is being recognized on a straight-line basis over the estimated period of performance. In the third quarter of 2014, we revised the remaining estimated period of performance from the first quarter of 2017 to the first quarter of 2018 to reflect a modification to our clinical development and regulatory plans. The contingent upfront consideration of \$6.5 million will be recognized if and when the refundable nature of these amounts lapses based upon the achievement of specified regulatory and/or clinical events.

PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

The contingent milestone payments under the January 2014 agreement are not considered substantive because a portion may be refunded upon certain events. The non-contingent portion of the milestone payment will be recognized as collaboration revenue on a straight-line basis over the estimated period of performance, which is now through the first quarter of 2018. The contingent portion of the milestone payments will be recognized if and when the refundable nature of these amounts lapses based upon the achievement of specified regulatory and/or clinical events. None of these milestones had been earned or received at June 30, 2015.

During the three and six months ended June 30, 2015 and 2014, we recognized \$384,000 and \$763,000, and \$503,000 and \$945,000, respectively in collaboration revenue under this agreement, respectively. The deferred revenue balance under this agreement as of June 30, 2015 was \$10.7 million.

Lee's Pharmaceutical (HK) Ltd ("Lee's")

In January 2013, we entered into an agreement with Lee's to jointly expand our Phase 3 APEX Study of Betrixaban into China. Under the terms of the agreement, Lee's provided us with an upfront and non-refundable fee of \$700,000 and will reimburse our costs in connection with the expansion of our APEX study into China. Lee's will lead this study and the regulatory interactions with China's State Food and Drug Administration. We granted Lee's an exclusive option to negotiate for the exclusive commercial rights to Betrixaban in China, which may be exercised by Lee's for 60 days after it receives the primary data analysis report from the Phase 3 APEX study.

We identified the following deliverables under the agreement with Lee's: 1) the granting of an exclusive option to negotiate for the exclusive commercial rights to Betrixaban in China, 2) the obligation to manufacture and supply product in support of the APEX study in China, 3) the obligation to participate in a joint working group, and 4) the delivery of the primary data analysis report from our APEX study. We considered the provisions of the multiple-element arrangement guidance in determining how to recognize the total consideration of the agreement. We determined that none of the deliverables have standalone value and therefore are accounted for as a single unit of accounting with the upfront fee recognized as revenue on a straight-line basis over the estimated period of performance. Any reimbursements we may receive from Lee's for the costs we incur in connection with this agreement are expected to be immaterial.

During the three and six months ended June 30, 2015 and 2014, we recognized \$52,000 and \$104,000, and \$63,000 and \$125,000, in collaboration revenue under this agreement, respectively. The deferred revenue balance under this agreement as of June 30, 2015 was \$158,000.

Aciex Therapeutics, Inc. ("Aciex")

In February 2013, we entered into a license and collaboration agreement with Aciex pursuant to which we granted Aciex an exclusive license to co-develop and co-commercialize Cerdulatinib (PRT2070) and certain related compounds for nonsystemic indications, such as the treatment and prevention of ophthalmological diseases by topical administration and allergic rhinitis by intranasal administration. In April 2014, this agreement was amended to release all rights for Cerdulatinib to Portola. The collaboration is now focused on development of other related compounds for topical ophthalmic indications. Under the terms of this risk and cost sharing agreement, Portola and Aciex will each incur and report their own internal research and development costs. Further, third-party related development costs will

be shared by Aciex and us 60% and 40%, respectively, until the end of the Phase 2 clinical study, and then equally afterwards. Also, we are entitled to receive either 50% of the profits, if any, generated by future sales of the products developed under the agreement, or royalty payments. Aciex has the primary responsibility for conducting the research and development activities under this agreement. We are obligated to provide assistance in accordance with the agreed upon development plan as well as participate on various committees. We can opt out of our obligation to share in the development costs at various points in time, the timing of which impacts future royalties we may receive based on product sales made by Aciex. All net costs we incur in connection with this agreement will be recognized as research and development expenses. During the three and six months ended June 30, 2015 and 2014 no such costs have been incurred related to this agreement.

In October 2014, Aciex was acquired by Nicox S.A..As of June 30, 2015, there has been no change to our agreement with Aciex.

F-13

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PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

Bayer Pharma, AG (“Bayer”) and Janssen Pharmaceuticals, Inc. (“Janssen”)

In February 2013, we entered into a three-way agreement with Bayer and Janssen to include subjects dosed with rivaroxaban, their Factor Xa inhibitor product, in one of our Phase 2 proof-of-concept studies of Andexanet alfa. We are responsible for the cost of conducting this clinical study. Under the terms of the agreement, Bayer and Janssen have each provided us with an upfront and non-refundable fee of \$2.5 million, for an aggregate fee of \$5.0 million. The agreement also provides for additional non-refundable payments to us from Bayer and Janssen of \$250,000 each for an aggregate of \$500,000 following the delivery of the final written study report of our Phase 2 proof-of-concept studies of Andexanet alfa. Also, we are obligated to participate on a JCC with Bayer and Janssen to oversee the collaboration activities under the agreement.

We identified the following performance deliverables under the agreement: 1) the obligation to provide research and development services, which includes supplying Andexanet alfa and providing a final written report, and 2) the obligation to participate on a JCC. We considered the provisions of the multiple-element arrangement guidance in determining how to recognize the revenue associated with these two deliverables. We have accounted for the research and development services and our participation on the JCC as a single unit of accounting as neither deliverable has standalone value and both obligations will be delivered throughout the estimated period of performance. We originally estimated the period of performance to be through the fourth quarter of 2013. During 2013, we added more cohorts than originally planned as part of the original study design at the inception of our agreement and therefore adjusted our period of performance to be through the fourth quarter of 2014. The total consideration under this agreement was recognized on a straight-line basis over the estimated performance period through the fourth quarter of 2014.

During the three and six months ended June 30, 2014, we recognized \$406,000 and \$807,000 respectively, in collaboration revenue under this agreement. There was no deferred revenue balance under this agreement as of June 30, 2015.

In January 2014, we entered into a three-way agreement with Bayer and Janssen to study the safety and efficacy of Andexanet alfa as a reversal agent to their oral Factor Xa inhibitor, rivaroxaban, in our Phase 3 studies. We are responsible for the cost of conducting this clinical study. Pursuant to our agreement with Bayer and Janssen we are obligated to provide research, development and regulatory services and to participate in a JCC in exchange for an upfront nonrefundable fee of \$10.0 million, up to three contingent payments totaling \$7.0 million which are payable upon achievement of certain events associated with scaling up our manufacturing process to support a commercial launch, and up to three payments totaling \$8.0 million which are payable upon initiation of our Phase 3 study and regulatory approval of Andexanet alfa as a reversal agent to rivaroxaban by the FDA and European Medicines Agency (“EMA”).

We identified the following non-cancellable performance deliverables under the agreement: 1) the obligation to provide research and development services, which include manufacturing and supplying Andexanet alfa and providing various reports, 2) the obligation to provide regulatory approval services, and 3) the obligation to participate on the JCC. We considered the provisions of the multiple-element arrangement guidance in determining how to recognize the total consideration of the agreement. We determined that none of the deliverables have standalone value; all of these obligations will be delivered throughout the estimated period of performance and therefore are accounted for as a single unit of accounting. The total upfront consideration under this agreement is being recognized as revenue on a

straight-line basis over the estimated period of performance period. In the third quarter of 2014 we updated our estimated period of performance from the first quarter of 2017 to the first quarter of 2018 to reflect a modification to our clinical development and regulatory plans.

We have determined all but one of the future contingent payments meet the definition of a milestone and that such milestones are substantive in that the consideration is reasonable relative to all of the deliverables and payment terms within the agreement and commensurate with our performance to achieve the milestone after commencement of the agreement. Accordingly, revenue for the achievement of these milestones will be recognized in the period when the milestone is achieved and collectability is reasonably assured. As of June 30, 2015, no amounts had been recognized as collaboration revenue for any of these milestones. The contingent payment of \$3.0 million, not considered to be a substantive milestone, was received in the third quarter of 2014 and is being recognized as collaboration revenue on a straight-line basis over the estimated performance period through the first quarter of 2018.

During the three and six months ended June 30, 2015 and 2014, we recognized \$807,000 and \$1,606,000, and \$788,000 and \$1.3 million, in collaboration revenue under this agreement, respectively. The deferred revenue balance under this agreement as of June 30, 2015 was \$8.9 million.

PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

Daiichi Sankyo, Inc. (“Daiichi Sankyo”)

In June 2013, we entered into an agreement with Daiichi Sankyo to include subjects dosed with edoxaban, their Factor Xa inhibitor product, in one of our Phase 2 proof-of-concept studies of Andexanet alfa. We are responsible for the cost of conducting this clinical study. Under the terms of the agreement, Daiichi Sankyo provided us with an upfront fee of \$6.0 million, \$3.0 million of which was subject to refund if Daiichi Sankyo decided to terminate the agreement. We are obligated to participate on a JCC with Daiichi Sankyo to oversee the collaboration activities under the agreement.

We identified the following performance deliverables under the agreement: 1) the obligation to provide research and development services, which includes supplying Andexanet alfa and providing a final written report, and 2) the obligation to participate on the JCC.

We considered the provisions of the multiple-element arrangement guidance in determining how to recognize the revenue associated with these two deliverables. We have accounted for the research and development services and our participation on the JCC as a single unit of accounting as neither deliverable has standalone value and both obligations will be delivered throughout the estimated period of performance.

The total non-contingent consideration under this agreement of \$3.0 million was fully recognized as revenue on a straight-line basis over the estimated non-contingent performance period through the first quarter of 2014. The recognition of contingent consideration under this agreement of \$3.0 million commenced upon resolution of the contingency in the first quarter of 2014 and was originally being recognized over the remaining estimated period of performance through the first quarter of 2015. During the fourth quarter of 2014 we decided to include edoxaban data in our initial BLA submission and thus updated the performance period associated with the contingent payment to be through the fourth quarter of 2015.

During the three and six months ended June 30, 2015 and 2014, we recognized \$261,000 and \$519,000, and \$655,000 and \$1.6 million, respectively, in collaboration revenue associated with the contingent and non-contingent elements of this arrangement. The deferred revenue balance under this agreement as of June 30, 2015 was \$527,000.

In July 2014, we entered into an agreement with Daiichi Sankyo to study the safety and efficacy of Andexanet alfa as a reversal agent to their oral Factor Xa inhibitor, edoxaban, in our Phase 3 and Phase 4 studies. We are responsible for the cost of conducting these clinical studies. Pursuant to our agreement with Daiichi Sankyo we are obligated to provide research, development and regulatory services and to participate in a JCC in exchange for an upfront nonrefundable fee of \$15.0 million, up to two contingent payments totaling \$5.0 million which are payable upon the initiation of our Phase 3 study and achievement of certain events associated with scaling up our manufacturing process to support a commercial launch, and up to four payments totaling \$20.0 million which are payable upon acceptance of filing and regulatory approval of Andexanet alfa as a reversal agent to edoxaban by the FDA and EMA.

We identified the following non-cancellable performance deliverables under the agreement: 1) the obligation to provide research and development services, which include manufacturing and supplying Andexanet alfa and providing various reports, 2) the obligation to provide regulatory approval services, and 3) the obligation to participate on the JCC. We considered the provisions of the multiple-element arrangement guidance in determining how to recognize the total consideration of the agreement. We determined that none of the deliverables have standalone value; all of

these obligations will be delivered throughout the estimated period of performance and therefore are accounted for as a single unit of accounting. The total upfront consideration under this agreement is being recognized as revenue on a straight-line basis over the estimated performance period through the third quarter of 2018.

We have determined all but one of the future contingent payments meet the definition of a milestone and that such milestones are substantive in that the consideration is reasonable relative to all of the deliverables and payment terms within the agreement are commensurate with our performance to achieve the milestone after commencement of the agreement. Accordingly, revenue for the achievement of these milestones will be recognized in the period when the milestone is achieved and collectability is reasonably assured. As of June 30, 2015, no amounts had been recognized as collaboration revenue for any of these milestones. Amounts for the contingent payment not considered to be a substantive milestone will be deferred when received and recognized as collaboration revenue on a straight-line basis over the remaining performance period.

During the three and six months ended June 30, 2015, we recognized \$881,000 and \$1,752,000, respectively, in collaboration revenue under this agreement. The deferred revenue balance under this agreement as of June 30, 2015 was \$11.5 million.

PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

Ora, Inc. (“Ora”)

In May 2015, we entered into a license and collaboration agreement with Ora pursuant to which we granted Ora an exclusive license to co-develop and co-commercialize one of our specific SyK inhibitors, PRT2761. Ora has the primary responsibility for conducting the research and development and regulatory activities under this agreement. We are obligated to provide assistance in accordance with the agreed-upon development plan as well as participate on various committees.

Under the terms of this risk and cost sharing agreement, each party will incur its own share of development cost and third-party related development costs will be shared by Ora and us at approximately 60% and 40%, respectively, until an End of Phase 2 meeting with the FDA, and equally thereafter. We are entitled to receive either 50% of the profits, if any, generated by future sales of the products developed under the agreement or royalty payments on such sales, should we opt out of the agreement.

Pursuant to the Ora agreement, we may opt out of the agreement any time prior to 90 days after an End of Phase 2 meeting with the FDA. The timing of the exercise of our opt out rights would impact future royalties we would be entitled to receive from Ora. Each party may buy out the rights and interests in the licensed compound by paying the greater of \$6.0 million or two times the actual aggregate development cost incurred by both parties on or before End of Phase 2 opt out point.

All costs we incur in connection with this agreement will be recognized as research and development expenses. During the three and six months ended June 30, 2015 no costs have been incurred related to this agreement.

## 7. Commercial Supply Agreement

In July 2014, we entered into an agreement with CMC ICOS Biologics, Inc. (“CMC Biologics”), a subsidiary of CMC Biologics S.à.r.l., a privately-held contract manufacturing organization, pursuant to which CMC Biologics will manufacture clinical and commercial supply of Andexanet alfa and perform pre-validation and validation work on our behalf.

Under the agreement, we are required to purchase an aggregate fixed number of batches of Andexanet alfa from CMC Biologics beginning in 2015 through 2021. Total batch commitments under the agreement can be increased or decreased based on the achievement of milestones relating to the regulatory approval process for Andexanet alfa, expansion of existing manufacturing capacity and operational qualification of CMC Biologics’ manufacturing facilities. We made an upfront payment to CMC Biologics in the amount of \$10.0 million in July 2014 and a reservation payment to CMC Biologics of \$4.6 million in November 2014. Both payments will be credited against our future purchases of batches under the agreement.

Total fixed commitments under the agreement for the purchases of clinical and commercial batches, not taking into account possible price and batch adjustments per the terms of the agreement, are approximately \$293.9 million. Payments made for purchases of batches under the agreement as of June 30, 2015 amount to \$10.6 million.

The term of the agreement is seven years and may be early terminated by either party for the other party's uncured material breach or insolvency. We may also terminate the agreement if CMC Biologics is unable to add additional manufacturing capacity on a timely basis, if certain manufacturing-related regulatory events do not occur before certain deadlines, or if the batch yield is below a certain threshold, in which case we are not obligated to pay CMC Biologics a termination payment and CMC Biologics will be obligated to refund the uncredited amounts of the upfront payment and reservation payment.

In addition, we may terminate the agreement unilaterally if we discontinue the development and commercialization of Andexanet alfa for regulatory, safety, efficacy or other commercial reasons, or if the projected market demand or gross margin of Andexanet alfa is below a minimum threshold. A termination agreement under these provisions will obligate us to pay CMC Biologics a termination fee between \$5.0 million and \$30.0 million, depending on the date of termination. The termination fee is highest from 2015 through 2017, and then decreases through 2021. Any remaining upfront payments or reservation payments we have made, not yet credited against the purchase of batches, at the time of termination will be applied against the termination fee.

Under the lease accounting guidance, we determined that the agreement does not contain an embedded lease because the agreement does not convey the right to control the use of CMC Biologics' facility. We based this determination on, among other factors, our right to physically access and/or operate CMC Biologics' facility and one or more parties, other than us, and taking more than a minor amount of the output that will be produced or generated by the CMC Biologics facility during the term of our agreement.

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

Under the consolidation guidance, we determined that CMC Biologics is a VIE, but that we are not CMC Biologics' primary beneficiary and therefore consolidation of CMC Biologics by us is not required. We based this determination on, among other factors, the upfront and reservation payment being akin to a form of subordinated financing, the fixed pricing terms of the arrangement creating variability that is absorbed by us, and that we do not have the power to direct the activities that most significantly affect the economic performance of CMC Biologics.

As of June 30, 2015, we have not provided financial, or other, support to CMC Biologics that was not previously contractually required. The upfront fee of \$14.6 million is recorded as \$13.1 million in prepaid and other long-term assets and \$1.5 million is recorded in prepaid expenses and other current assets in the condensed consolidated balance sheet. The payments made for purchases of batches of \$9.5 million are recorded in prepaid expenses and other current assets in the condensed consolidated balance sheet. These assets represent our maximum exposure to loss under this agreement at June 30, 2015. The upfront payment will be charged to research and development expense, or cost of sales, upon regulatory approval of Andexanet alfa, as batches are delivered over the term of the agreement. We are currently not able to quantify the exposure to losses associated with the fixed pricing terms of this agreement.

## 8. Stock Based Compensation

In January 2013, our Board of Directors adopted our 2013 Equity Incentive Plan, or the 2013 Plan, which became effective upon the closing of our initial public offering in May 2013. On January 1, 2015, the number of shares available for issuance under the 2013 Plan automatically increased by a number of shares equal to 5% of the total common stock outstanding at December 31, 2014. As of June 30, 2015 there were 9,445,589 shares reserved under the 2013 Plan for the future issuance of equity awards.

The following table summarizes option activity under our 2013 Equity Incentive Plan and related information during the six months ended June 30, 2015:

	Shares Subject to Outstanding Options	Weighted- Average Exercise Price Per Share
Balance at December 31, 2014	4,249,168	\$ 14.77
Options granted	1,392,841	\$ 35.41
Options exercised	(736,163 )	\$ 9.51
Options canceled	(108,928 )	\$ 25.95
Balance at June 30, 2015	4,796,918	\$ 21.21

The estimated grant date fair values of the employee stock options were calculated using the Black Scholes valuation model, based on the following assumptions:

	Three Month Ended June 30, 2015 2014			Six Month Ended June 30, 2015 2014		
Risk-free interest rate	1.85 %	1.89 %		1.70 %	1.89 %	
Expected life	6.0 years	5.8 years		6.0 years	5.9 years	
Expected volatility	64 %	80 %		65 %	80 %	
Dividend yield	—	—		—	—	



## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

The following table summarizes restricted stock units (RSUs) activity under our 2013 Equity Incentive Plan and related information during the six months ended June 30, 2015:

	Shares Subject to Outstanding RSUs	Weighted- Average grant date fair value per share
Balance at December 31, 2014	—	\$ —
RSUs granted	182,825	\$ 30.22
RSUs exercised	—	\$ —
RSUs canceled	(9,250 )	\$ 29.72
Balance at June 30, 2015	173,575	\$ 30.24

During the first quarter of 2015, we granted restricted stock units (RSUs) to certain employees of the Company. RSUs are share awards that entitle the holder to receive freely tradable shares of our common stock upon vesting. The RSUs cannot be transferred, and until they vest, the shares are subject to forfeiture if employment terminates prior to the release of the vesting restrictions. The RSUs, generally vest equal amounts on each of the first three year anniversaries of the grant date, provided the employee remains continuously employed with us. The fair value of the RSUs is equal to the closing price of our Common Stock on the grant date.

In January and June 2015, we granted market-based performance stock unit (PSU) awards to our executive officers. Each PSU represents a contingent right to receive one share of the Company's Common Stock upon achievement of a market-based performance measurement and subject to the recipients continued employment. At any time during the four years following the date of the grant, a portion of the PSUs will vest one year after the date the average closing price of Portola's stock on the NASDAQ Global Select Market is above \$50.00 per share for 45 consecutive trading days, and the remaining portion of the grant will vest one year after the date the average closing price of Portola's stock is above \$60.00 per share for 45 consecutive trading days. The weighted-average fair value of the PSUs was determined using the Monte Carlo simulation models incorporating the following weighted average assumptions:

	Six Month Ended
	June 30, 2015
	PSUs
Number of PSUs granted	165,000
Weighted-average grant date stock price	\$31.95
Weighted-average risk-free interest rate	1.13 %
Weighted-average volatility	62 %
Dividend yield	—
Weighted- average fair value per share of PSUs granted (\$50 Vesting Hurdle)	\$25.65
Weighted- average fair value per share of PSUs granted (\$60 Vesting Hurdle)	\$23.24

The estimated PSU expense is being recognized, on an accelerated basis over the estimated requisite service period, with no adjustments in the future periods based upon the Company's actual stock price over the performance period.

In June 2015, the Compensation Committee of our Board of Directors approved a program to award up to 69,625 performance-based RSUs to certain employees based on the achievement of goals related to the development of Andexanet alfa and Betrixaban. Each award represents a contingent right to receive one share of the Company's Common Stock upon the achievement of certain performance conditions by pre-specified dates and the award recipients continued employment. At June 30, 2015 certain performance conditions related to these awards had not yet been finalized or objectively defined. Therefore, the fair value of these awards is being re-measured using the Company's stock price at each reporting period until the time at which the performance conditions are completed or sufficiently defined. The estimated expense associated with these awards is also being recognized, on an accelerated basis, over the estimated requisite service period.

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

The table below sets forth the functional classification of stock-based compensation expense, net of estimated forfeitures, for the periods presented (in thousands):

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2015	2014	2015	2014
Research and development	\$2,542	\$1,283	\$4,751	\$2,330
Selling, general and administrative	2,216	1,140	5,180	2,396
Total stock-based compensation	\$4,758	\$2,423	\$9,931	\$4,726

## 9. Net Loss per Share Attributable to Common Stockholders

Basic net loss per share attributable to common stockholders has been computed by dividing the net loss by the weighted-average number of shares of common stock outstanding during the period. Diluted net loss per share attributable to common stockholders is calculated by dividing net loss by the weighted average number of shares of common stock and potential dilutive securities outstanding during the period.

The following common equivalent shares were excluded from the computation of diluted net loss per share attributable to common stockholders for the periods presented because including them would have been anti-dilutive:

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2015	2014	2015	2014
Stock options to purchase common stock	4,796,918	4,226,170	4,796,918	4,226,170
Common stock warrants	1,500	13,873	1,500	13,873
Restricted stock units	173,575	—	173,575	—
Performance stock units	165,000	—	165,000	—

F-19

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## ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the consolidated financial statements and notes thereto included elsewhere in this report and with the audited consolidated financial statements and related notes thereto included as part of our Annual Report on Form 10-K for the year ended December 31, 2014.

### Special note regarding forward-looking statements

This report contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those discussed in the forward-looking statements. The statements contained in this report that are not purely historical are forward-looking statements within the meaning of Section 27A of the Securities Act and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. Forward-looking statements are often identified by the use of words such as, but not limited to, "anticipate," "believe," "can," "continue," "could," "estimate," "expect," "intend," "may," "plan," "project," "seek," "should," "strategy," "target," "will," "would" and similar expressions or variations thereof to identify forward-looking statements. These statements are based on the beliefs and assumptions of our management based on information currently available to management. Such forward-looking statements are subject to risks, uncertainties and other important factors that could cause actual results and the timing of certain events to differ materially from future results expressed or implied by such forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those identified below and those discussed in the section titled "Risk Factors" included under Part II, Item 1A below. Furthermore, such forward-looking statements speak only as of the date of this report. Except as required by law, we undertake no obligation to update any forward-looking statements to reflect events or circumstances after the date of such statements.

### OVERVIEW

We are a biopharmaceutical company focused on the development and commercialization of novel therapeutics in the areas of thrombosis, other hematologic disorders and inflammation for patients who currently have limited or no approved treatment options. We are advancing our three wholly-owned compounds using novel biomarker and genetic approaches that may increase the likelihood of clinical, regulatory and commercial success of our potentially life-saving therapies.

Our Phase 3 programs address significant unmet medical needs in the area of thrombosis, or blood clots. Betrixaban is a novel oral once-daily inhibitor of Factor Xa in a Phase 3 clinical trial for extended duration prophylaxis, or preventive treatment, of a form of thrombosis known as venous thromboembolism, or VTE, in acute medically ill patients for 35 days of in-hospital and post-discharge use. Currently, there is no anticoagulant approved for extended duration VTE prophylaxis in the acute medically ill population. Our second Phase 3 compound Andexanet alfa, a Food and Drug Administration, or FDA-designated breakthrough therapy and orphan drug, is a recombinant protein designed to reverse anticoagulant activity in patients treated with a Factor Xa inhibitor. Andexanet alfa has potential indications to reverse anticoagulant activity in patients who are taking a direct or indirect Factor Xa inhibitor and who suffer a major bleeding episode or require emergency surgery. We are currently evaluating Andexanet alfa in Phase 3 clinical trials and a Phase 4 confirmatory trial. Our third product candidate, Cerdulatinib, is an orally available kinase inhibitor that inhibits spleen tyrosine kinase, or Syk, and janus kinases, or JAK, enzymes that regulate important signaling pathways. Cerdulatinib is being developed for hematologic, or blood, cancers and inflammatory disorders. We are currently conducting a Phase 1/2a proof-of-concept study for Cerdulatinib in patients with non-Hodgkin's lymphoma, or NHL, or chronic lymphocytic leukemia, or CLL, who have failed or relapsed on existing marketed therapies or products in development, including patients with identified mutations. In the Phase 1 dose escalation portion of the study, we have yet to reach the maximum tolerated dose and enrollment continues. Based on interim Phase 1 data, we are advancing Cerdulatinib to the Phase 2a portion of the study which includes expansion cohorts in

select hematologic cancers. We also have a program of highly selective Syk inhibitors one of which is partnered with Ora, Inc. or Ora.

## Our product candidates and collaboration agreements

### Betrixaban

Betrixaban is a novel oral once-daily inhibitor of Factor Xa in development for extended duration VTE prophylaxis in acute medically ill patients for 35 days of in-hospital and post discharge use. Acute medically ill patients are those who are hospitalized for serious non-surgical conditions, such as heart failure, stroke, infection, rheumatic disorders and pulmonary disorders. In 2012, we initiated our pivotal biomarker-based Phase 3 APEX study, a randomized, double-blind, active-controlled, multicenter, multinational study to evaluate a once-daily dose of Betrixaban for 35 days for superiority as compared to in-hospital administration of enoxaparin once daily for 6 to 14 days followed by placebo. Our APEX study enrollment is on track for blinded, pooled event rates and quality metrics in 35 countries worldwide. We believe Betrixaban has the potential to succeed in this patient population, in part due to its validated mechanism of action, but most importantly, due to its properties that differentiate it from other anticoagulants. First, it has the longest half-life, making it a true, once-daily therapy allowing for a narrow peak-to-trough concentration ratio that helps maintain a less variable anticoagulant effect over the course of a day. Second, it has the lowest renal clearance of all the Factor Xa inhibitors, which may result in a lower rate of bleeding. And finally, it is not metabolized in the liver by an enzyme called CYP 3A4, which may result in reduced potential for drug-on-drug interactions. These properties are critically important for acute medically ill patients who are often renally compromised and on multiple concomitant medications. The pooled, blinded event rates for APEX remain on target per the protocol. We have completed a planned protocol defined sample size re-assessment and we now plan to increase the size of the trial from 6,850 to approximately 7,500 patients. The increase in sample size ensures APEX statistical power for the primary efficacy analysis patient cohort and increases power in the overall patient population analysis. We are in active discussions with the FDA on the final statistical analysis plan. We still expect APEX to complete patient enrollment by the end of 2015, report top line data in early 2016 and file the NDA in third quarter 2016.

In January 2013, we entered into a clinical collaboration agreement with Lee's Pharmaceutical (HK) Ltd, or Lee's, to jointly expand the Phase 3 APEX study of Betrixaban into China with an exclusive option for Lee's to negotiate for the exclusive commercial rights to Betrixaban in China.

### Andexanet alfa

Andexanet alfa, an FDA-designated breakthrough therapy and orphan drug, is a recombinant protein designed to reverse anticoagulant activity in patients treated with a Factor Xa inhibitor. Andexanet alfa has potential indications to reverse anticoagulant activity in patients who are taking a direct or indirect Factor Xa inhibitor and who suffer a major bleeding episode or who require emergency surgery. Currently, there is no antidote or reversal agent approved for use against Factor Xa inhibitors. Andexanet alfa is the first therapy to demonstrate reversal of the anticoagulant activity of Factor Xa inhibitors as measured by anti-Factor Xa levels. We have initiated two Phase 3 ANNEXA™ (Andexanet Alfa a Novel Antidote to the Anticoagulant Effects of fXA Inhibitors) studies – one with Bristol-Myers Squibb Company, or BMS, and Pfizer Inc.'s, or Pfizer, Factor Xa inhibitor, apixaban and one with Bayer Pharma AG, or Bayer, and Janssen Pharmaceuticals, Inc.'s, or Janssen's, Factor Xa inhibitor, rivaroxaban. Our Phase 3 studies each consist of two parts. In the first part of each study, the effect of a single bolus of Andexanet alfa was evaluated in healthy volunteers who had been given apixaban or rivaroxaban. In the second part of each study, the ability of Andexanet alfa to sustain reversal of the anticoagulant effects of apixaban and rivaroxaban is being evaluated by administering a bolus plus infusion of Andexanet alfa to healthy volunteers who have been given apixaban or rivaroxaban. The first part of our Phase 3 ANNEXA™ studies of a single bolus of Andexanet alfa with apixaban and with rivaroxaban both met their primary and secondary endpoints with high statistical significance (p-values of less than 0.0001). The second part of our Phase 3 ANNEXA™ with bolus plus infusion of Andexanet alfa with apixaban also met its primary and secondary endpoints with high statistical significance (p-value of less than 0.0001). The final

Phase 3 ANNEXA™ study of bolus plus infusion of Andexanet alfa with rivaroxaban is ongoing with results expected in 2015. In early 2015, we initiated a Phase 4 ANNEXA™ confirmatory patient study, as agreed to by the FDA and EMA. This study is part of an accelerated approval pathway in the United States for Andexanet alfa. This open-label, single-arm study is being conducted in patients receiving apixaban, rivaroxaban, edoxaban, or enoxaparin (a low molecular weight heparin) who present with an acute major bleed. Pursuant to discussions with the FDA, we plan to include data from the initial patients enrolled in this study in our Biologics License Application, or BLA, which we expect to submit in 2015 for conditional approval.

We have completed a series of Phase 2 proof-of-concept studies evaluating the safety and activity of Andexanet alfa in healthy volunteers who were administered one of several Factor Xa inhibitors. Analysis of anticoagulation markers in blood samples taken from the subjects in these studies demonstrated that Andexanet alfa produced immediate reversal of anticoagulant activity of the Factor Xa inhibitors apixaban, rivaroxaban and enoxaparin and that the reversal could be sustained. Additionally, we are conducting a Phase 2 proof-of-concept study evaluating the reversal of edoxaban and we plan to initiate a Phase 2 study evaluating the reversal of Betrixaban in 2015.



Our current Phase 2 and Phase 3 ANNEXA™ studies are using clinical material from CMC Biologics, Inc., or CMC Biologics. We are currently continuing and expanding our ongoing work with CMC Biologics from clinical supply to commercial supply for a potential BLA filing and U.S. launch. Under our commercial supply agreement with CMC Biologics they plan to increase production capacity at a lower cost than that of our current clinical supply for Andexanet alfa. We are also working with Lonza Group Ltd, or Lonza, to develop a large-scale manufacturing process. Under our manufacturing supply agreement with Lonza we plan to further increase our production capacity and to enhance our manufacturing process at Lonza to support broader worldwide supply following our potential BLA filing.

In January 2014, we entered into a collaboration agreement with BMS and Pfizer to further study Andexanet alfa as a reversal agent for their jointly owned FDA-approved oral Factor Xa inhibitor apixaban through Phase 3 studies. We initiated Phase 3 studies in the first quarter of 2014. Under the terms of the agreement, we received an upfront payment of \$13.0 million, subject to a 50% refund provision, and are eligible to receive additional development and regulatory milestone and contingent payments of up to \$12.0 million, subject to a 50% refund provision. These payments represent the total consideration under this agreement. BMS and Pfizer will continue to provide development and regulatory guidance for the program. Under both agreements with BMS and Pfizer, we retain full, worldwide development and commercial rights to Andexanet alfa. This Phase 3 collaboration agreement will continue in force until the approval of Andexanet alfa as a reversal agent for apixaban by the FDA and EMA. BMS and Pfizer may terminate this agreement for convenience with 60 days' advance written notice or for our bankruptcy or change of control. In addition, either party may terminate this agreement for the other party's uncured material breach, material safety issues, or failure of the Phase 3 studies.

In February 2014, we entered into a second collaboration agreement with Bayer and Janssen to evaluate Andexanet alfa as a reversal agent for the FDA-approved oral Factor Xa inhibitor rivaroxaban through Phase 3 studies. Our original collaboration agreement with Bayer and Janssen covers the conduct of a Phase 2 proof-of-concept study. The new collaboration agreement covers the conduct of Phase 3 studies of Andexanet alfa with rivaroxaban and any potential U.S. and EU regulatory approval of Andexanet alfa as reversal agent of rivaroxaban. The part 2 of the phase 3 study is currently ongoing. Under this collaboration agreement, we received an upfront payment of \$10.0 million and are eligible to receive additional development and regulatory milestone payments of up to \$15.0 million. These payments represent the total consideration under this agreement. Bayer and Janssen will continue to provide development and regulatory guidance for the program.

Under both agreements with Bayer and Janssen, we retain full, worldwide development and commercial rights to Andexanet alfa. This Phase 3 collaboration agreement will continue in force until the approval of Andexanet alfa as a reversal agent for rivaroxaban by the FDA and EMA. Bayer and Janssen may terminate this agreement for convenience with 60 days' advance written notice or for our bankruptcy or change of control. In addition, either party may terminate this agreement for the other party's uncured material breach or material safety issues, or we can also terminate this agreement for failure of the Phase 3 studies.

In July 2014, we entered into a collaboration agreement with Daiichi Sankyo to include subjects dosed with edoxaban, their Factor Xa inhibitor product, in one of our Phase 3 studies of Andexanet alfa. We are responsible for the cost of conducting this clinical study. Daiichi Sankyo will work closely with us on both development and regulatory aspects of Andexanet alfa in connection with our Phase 3 study to the extent such matters relate to edoxaban. Pursuant to our agreement with Daiichi Sankyo we are obligated to provide research and development and regulatory approval services and participate on various committees. Under this collaboration agreement, we received an upfront payment of \$15.0 million in the third quarter of 2014 and are eligible to receive additional payments of up to \$25.0 million due upon the achievement of certain milestones associated with the progress of our Phase 3 study.

Cerdulatinib

In addition to our thrombosis products, we are developing orally available kinase inhibitors to treat hematologic disorders and inflammation. Cerdulatinib is an orally available, potent inhibitor of enzymes that regulate two important signaling pathways, spleen tyrosine kinase, or Syk, and janus kinase. We are developing Cerdulatinib for the treatment of certain B-cell hematologic cancers. We are currently in a Phase 1/2a proof-of-concept study with Cerdulatinib in non-Hodgkin's lymphoma and chronic lymphocytic leukemia. In the Phase 1 dose escalation portion of the study, we have yet to reach the maximum tolerated dose and enrollment continues. Based on interim Phase 1 data, we are advancing Cerdulatinib to the Phase 2a portion of the study which includes expansion cohorts in select hematologic cancers.

#### Selective Syk Inhibitors

Syk is an important mediator of immune response in a number of different types of immune cells. Our selective Syk inhibitors have been successfully evaluated in 131 subjects in several Phase 1 clinical studies. Ora is leading the pre-clinical study of a selective Syk inhibitor for ophthalmic diseases.

In May 2015, we entered into a license and collaboration agreement with Ora pursuant to which we granted Ora an exclusive license to co-develop and co-commercialize one of our specific SyK inhibitors, PRT02761. Ora has the primary responsibility for conducting the research and development and regulatory activities under this agreement. We are obligated to provide assistance in accordance with the agreed upon development plan as well as participate on various committees.

Under the terms of this risk and cost sharing agreement, each party will incur its own share of development cost and third-party related development costs will be shared by Ora and us at approximately 60% and 40%, respectively, until an End of Phase 2 meeting with the FDA, and equally thereafter. We are entitled to receive either 50% of the profits, if any, generated by future sales of the products developed under the agreement or royalty payments on such sales, should we opt out of the agreement.

Pursuant to the agreement, we may opt out of the agreement any time prior to 90 days after an End of Phase 2 meeting with the FDA. The timing of the exercise of our opt out rights would impact future royalties we would be entitled to receive from Ora. Each party may buy out the rights and interests in the licensed compound by paying the greater of \$6.0 million or two times the actual aggregate development cost incurred by both parties on or before End of Phase 2 opt out point.

All costs we incur in connection with this agreement will be recognized as research and development expenses. During the three and six months ended June 30, 2015 no costs have been incurred related to this agreement.

In October 2011, we entered into an exclusive, worldwide license and collaboration agreement with Biogen Idec to develop and commercialize selective Syk kinase inhibitors for the treatment of autoimmune and inflammatory diseases. Under this agreement, Biogen Idec is responsible for all development-related expenses. In April 2014, we entered into an amendment to the Biogen Idec license and collaboration agreement under which Biogen Idec released to us one of the Syk kinase inhibitors for use in topical ophthalmic indications. In May 2015, our agreement with Biogen Idec was terminated in its entirety.

For purposes of this discussion and analysis of our financial condition and results of operations, we refer to our agreements with Millennium Pharmaceuticals, Inc., Merck & Co. Inc., or Merck, Lee's, BMS and Pfizer, Bayer and Janssen, Daiichi Sankyo, Ora, Acix Therapeutics, Inc, Biogen Idec, Astellas Pharma, Inc., or Astellas, and Novartis Pharma A.G., or Novartis, collectively as our collaboration agreements.

#### Financial operations overview

##### Revenue

Our revenue to date has been generated primarily from collaboration and license revenue pursuant to our collaboration agreements. We have not generated any revenue from commercial product sales to date. Since inception, in connection with our agreements with Novartis, Merck, Biogen Idec, BMS and Pfizer, Bayer and Janssen, Lee's and Daiichi, we have received payments in the aggregate amount of \$219.7 million, as initial upfront payments, contingent consideration and a milestone payment of which \$6.5 million is subject to a 50% refund provision, pursuant to our Phase 3 clinical collaboration agreement with BMS and Pfizer.

We may also be entitled to additional milestone payments and other contingent payments upon the occurrence of specific events. Due to the nature of these collaboration agreements and the nonlinearity of the earnings process associated with certain payments and milestones, we expect that our revenue will continue to fluctuate in future periods.

The following table summarizes the sources of our collaboration and license revenue, in thousands:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
BMS and Pfizer	\$ 384	\$ 1,194	\$763	\$2,106
Bayer and Janssen	807	503	1,606	945
Lee's Pharmaceutical	52	63	104	125
Daiichi Sankyo	1,142	655	2,271	1,610
Total collaboration and license revenue	\$ 2,385	\$ 2,415	\$4,744	\$4,786

#### Research and development expenses

Research and development expenses represent costs incurred to conduct research, such as the discovery and development of our unpartnered product candidates, as well as discovery and development of clinical candidates pursuant to our collaboration agreements. We recognize all research and development costs as they are incurred.

Our research and development expenses may increase or decrease by amounts we may pay or receive under various cost-sharing provisions of our collaboration and license agreements.

We expect our research and development expenses to increase as we continue to advance our product candidates through clinical development. We intend to identify partnerships to further develop other product candidates that strengthen our pipeline, which may offset a portion of our research and development expenses through reimbursement from these partners.

The following table summarizes our research and development expenses incurred by product candidate:

	Phase of Development	Three Months Ended		Six Months Ended	
		June 30, 2015	June 30, 2014	June 31, 2015	June 31, 2014
Product candidate		(in thousands)	(in thousands)	(in thousands)	(in thousands)
Betrixaban	Phase 3	\$20,584	\$16,413	\$38,982	\$31,324
Andexanet alfa	Phase 2/3/4	28,251	10,760	46,650	22,759
Cerdulatinib	Phase 1/2a	2,822	1,575	5,678	2,647
Syk selective inhibitor	Pre-clinical	52	(52 )	94	(63 )
Other research and development expenses <sup>(1)</sup>		591	287	756	471
Total research and development expenses <sup>(2)</sup>		\$52,300	\$28,983	\$92,158	\$57,138

(1) Amounts in all periods include costs for other potential product candidates.

(2) Our research and development expenses have been reduced by reimbursements of certain research and development expenses pursuant to the cost-sharing provisions of our agreements with Biogen Idec commencing in the fourth quarter of 2011 and MyoKardia, Inc. and Global Blood Therapeutics, Inc. commencing in the fourth quarter of 2012.

The program-specific expenses summarized in the table above include costs directly attributable to our product candidates. We allocate research and development salaries, benefits, stock-based compensation and indirect costs to our product candidates on a program-specific basis, and we include these costs in the program-specific expenses. The largest component of our total operating expenses has historically been our investment in research and development activities, including the clinical development of our product candidates. We expect our research and development expenses to increase in the future. The process of conducting the necessary clinical research to obtain FDA approval is costly and time consuming. We consider the active management and development of our clinical pipeline to be crucial to our long-term success. The actual probability of success for each product candidate and clinical program may be affected by a variety of factors including: the quality of the product candidate, early clinical data, investment in the program, competition, manufacturing capability and commercial viability. Furthermore, in the past we have entered into collaborations with third parties to participate in the development and commercialization of our product candidates, and we may enter into additional collaborations in the future. In situations in which third parties have control over the preclinical development or clinical study process for a product candidate, the estimated completion dates are largely under the control of such third parties and not under our control. We cannot forecast with any degree of certainty which of our product candidates, if any, will be subject to future collaborations or how such arrangements

would affect our development plans or capital requirements. As a result of the uncertainties discussed above, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

#### Selling, general and administrative expenses

Selling, general and administrative expenses consist primarily of personnel costs, allocated facilities costs and other expenses for outside professional services, including legal, human resources, audit and accounting services and sales and marketing expenses related to commercial launch preparation. Personnel costs consist of salaries, benefits and stock-based compensation. We also incur additional expenses as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the SEC and those of The NASDAQ Global Market, additional insurance expenses, investor relations activities and other administration and professional services. In addition, if any of our product candidates receive regulatory approval for commercial sale, we expect to incur significant expenses associated with the establishment of a hospital-based sales force in the United States and possibly other major markets. Because of the numerous risks and uncertainties associated with drug development, we are unable to predict the timing or amount of expenses incurred or when, or if, we will be able to achieve sustained profitability.

## Interest and other income (expense), net

Interest and other income (expense), net consists primarily of interest received on our cash, cash equivalents and investments, unrealized gains and losses from the remeasurement of our foreign currency bank balances and foreign currency forward contracts.

## Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles, or U.S. GAAP. The preparation of these consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the reported revenue generated and expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. There have been no significant and material changes in our critical accounting policies during the six months ended June 30, 2015, as compared to those disclosed in "Management's Discussion and Analysis of Financial Condition and Results of Operations-Critical Accounting Policies and Significant Judgments and Estimates" in our Annual Report on Form 10-K for the year ended December 31, 2014 filed with the SEC on March 2, 2015 except related to stock based compensation as follows.

We estimate the fair value of restricted stock units (RSUs) based on the fair market values of the underlying stock on the dates of grant. The estimated fair value of performance-based RSUs is expensed using an accelerated method over the requisite service period based on management's best estimate as to whether it is probable that the shares awarded are expected to vest. We assess the probability of the performance indicators being met on a continuous basis.

We estimate fair value of market-based performance stock units (PSUs) based on Monte Carlo simulation models. The estimated fair value of PSUs is expensed using an accelerated method over the estimated requisite service period, with no adjustments in the future periods based upon the Company's actual stock price over the performance period. The Monte Carlo simulation models require the use of highly subjective and complex assumptions which determine the fair value of PSUs including price volatility of underlying stock and derived service period.

The assumptions used in calculating the fair value of PSUs and expected attainment of performance-based RSUs represent our best estimates, but these estimates involve inherent uncertainties and the application of management judgment.

## Results of operations

### Comparison of the three and six months ended June 30, 2015 and 2014

#### Revenue

Three Months Ended			Six Months Ended		
June 30,	Increase		June 30,	Increase	
	/	% Increase /		/	% Increase /

	2015	2014	(Decrease)	(Decrease)		2015	2014	(Decrease)	(Decrease)		
	(in thousands, except percentages)										
Collaboration and license revenue	\$2,385	\$2,415	\$ (30	)	(1	%)	\$4,744	\$4,786	\$ (42	) (1	%)

The decrease in collaboration and license revenue during the three months ended June 30, 2015 was due to a decrease of \$0.6 million associated with our Bayer and Janssen Phase 2 agreement, which was fully recognized by the third quarter of 2014, and a decrease of \$0.4 million associated with our Daiichi Sankyo Phase 2 agreement following a change in estimate. This decrease was partially offset by an increase of \$0.9 million associated with our Daiichi Sankyo Phase 3 agreement which we entered into in the third quarter of 2014, and an increase of \$0.2 million associated with our Phase 3 agreements with BMS and Pfizer and Bayer and Janssen which were entered into in the first quarter of 2014.

The decrease in collaboration and license revenue during the six months ended June 30, 2015 was due to a decrease of \$1.0 million associated with our Bayer and Janssen Phase 2 agreement, which was fully recognized in the third quarter of 2014, and a decrease of \$1.1 million associated with our Daiichi Sankyo Phase 2 agreement, which is expected to be completed in the fourth quarter of 2015. This decrease was partially offset by an increase of \$1.8 million associated with our Daiichi Sankyo Phase 3 agreement which we entered into in the third quarter of 2014, and an increase of \$0.6 million associated with our Phase 3 agreements with BMS and Pfizer and Bayer and Janssen which were entered into in the first quarter of 2014.



We regularly review the estimated periods of performance related to our collaborations based on the progress made under each arrangement. Our estimates of our performance period may change over the course of the collaboration term.

The total consideration received under our Phase 3 agreement with BMS and Pfizer of \$13.0 million, subject to a 50% refund provision, is being recognized on a straight-line basis over the estimated performance period through the first quarter of 2018. We are also eligible to receive additional payments up to \$12.0 million of contingent and milestones, subject to a 50% refund provision, due upon achievement of certain development and regulatory events.

The total consideration received under our Phase 3 agreement with Bayer and Janssen of \$10.0 million is being recognized on a straight-line basis over the estimated performance period through the first quarter of 2018. We are also eligible to receive additional payments up to \$15.0 million due upon achievement of certain development and regulatory events.

The total consideration received under our Phase 2 agreement with Bayer and Janssen of \$5.5 million was recognized as revenue on a straight-line basis through the fourth quarter of 2014.

The total consideration received under our Phase 2 agreement with Daiichi Sankyo of \$6.0 million includes contingent consideration of \$3.0 million and non-contingent consideration of \$3.0 million. The total non-contingent consideration under this agreement of \$3.0 million was fully recognized as revenue on a straight-line basis over the estimated non-contingent performance period through the first quarter 2014. The recognition of contingent consideration under this agreement of \$3.0 million commenced upon resolution of the contingency in the first quarter 2014 and is being recognized over the remaining estimated performance period, through the fourth quarter of 2015.

The total consideration received under our Phase 3 agreement with Daiichi Sankyo of \$15.0 million is being recognized as revenue on a straight-line basis over the estimated performance period through the third quarter of 2018. We are also eligible to receive additional payments up to \$25.0 million due upon achievement of certain milestones associated with the progress of our Phase 3 study.

We expect revenue recognized in future periods to fluctuate as we recognize revenue related to our existing collaboration agreements and enter into new collaboration agreements until such time as we achieve product commercialization.

#### Research and development expenses

	Three Months Ended				Six Months Ended				
	June 30, 2015	June 30, 2014	Increase / (Decrease)	% Increase / (Decrease)	June 30, 2015	June 30, 2014	Increase / (Decrease)	% Increase / (Decrease)	
(in thousands, except percentages)									
Research and development									
expenses	\$52,300	\$28,983	\$ 23,317	80	% \$92,158	\$57,138	\$ 35,020	61	%

The increase in research and development expenses during the three months ended June 30, 2015 was primarily due to increased program costs of \$2.8 million to advance Betrixaban, increased program costs of \$17.5 million to advance Andexanet alfa and increased program costs of \$1.3 million to advance Cerdulatinib.

The increase in research and development expenses during the six months ended June 30, 2015 was primarily due to increased program costs of \$6.3 million to advance Betrixaban, increased program costs of \$23.7 million to advance Andexanet alfa through commercial manufacturing validation to support a BLA submission and commercial launch and increased program costs of \$3.0 million to advance Cerdulatinib.

We expect our research and development expenses to increase in the future as we continue to advance our product candidates through clinical development. The timing and amount of expenses incurred will depend largely upon the outcomes of current or future clinical studies for our product candidates as well as the related regulatory requirements, manufacturing costs and any costs associated with the advancement of our preclinical programs.

## Selling, general and administrative expenses

	Three Months Ended				Six Months Ended			
	June 30, 2015	2014	Increase / (Decrease)	% Increase / (Decrease)	June 30, 2015	2014	Increase / (Decrease)	% Increase / (Decrease)
(in thousands, except percentages)								
Selling, general and administrative expenses	\$8,912	\$4,937	\$ 3,975	81	% \$17,917	\$10,177	\$ 7,740	76

The increase in selling, general and administrative expenses during the three months ended June 30, 2015 was primarily related to increased headcount-related costs including stock-based compensation expense of \$2.0 million, higher professional and legal fees to support general corporate activities of \$0.7 million, commercial launch preparation activities and business development related costs of \$1.0 million, and increased payroll expenses and other facilities-related costs of \$0.3 million.

The increase in selling, general and administrative expenses during the six months ended June 30, 2015 was primarily related to increased headcount-related costs including stock-based compensation expense of \$4.1 million, higher professional and legal fees to support general corporate activities of \$1.3 million, commercial launch preparation activities and business development related costs of \$1.7 million, and increased payroll expenses and other facilities-related costs of \$0.6 million.

We expect selling, general and administrative expenses to increase in order for us to continue to support our growing business and prepare for commercialization.

## Interest and other income (expense), net

	Three Months Ended				Six Months Ended			
	June 30, 2015	2014	Increase / (Decrease)	% Increase / (Decrease)	June 30, 2015	2014	Increase / (Decrease)	% Increase / (Decrease)
(in thousands, except percentages)								
Interest and other income, net	\$498	\$155	\$ 343	221	% \$89	\$453	\$ (364)	(80)

The increase in interest and other income (expense), net during the three months ended June 30, 2015 was primarily due to realized and unrealized foreign exchange gains on foreign currencies we hold of \$0.2 million compared to foreign currency exchange losses of \$0.1 million in the three months ended June 30, 2014. These gains and losses are primarily related to fluctuations in the Euro and British Pound compared to the U.S. dollar. This decrease was partially offset by an increase in interest income of \$0.1 million due to higher investment balances during the three months

ended June 30, 2015.

The decrease in interest and other income (expense), net during the six months ended June 30, 2015 was primarily due to realized and unrealized foreign exchange losses on foreign currencies we hold of \$0.6 million compared to foreign currency exchange gains of \$0.1 million in the six months ended June 30, 2014. These gains and losses are primarily related to fluctuations in the Euro and British Pound compared to the U.S. dollar. This decrease was partially offset by an increase in interest income of \$0.2 million due to higher investment balances during the six months ended June 30, 2015.

#### Liquidity and capital resources

Due to our significant research and development expenditures, we have generated significant operating losses since our inception. We have funded our operations primarily through the sale of equity securities and payments received from our collaboration partners. Our expenditures are primarily related to research and development activities. At June 30, 2015, we had available cash, cash equivalents and investments of \$407.5 million. Our cash, cash equivalents and investments are held in a variety of interest-bearing instruments, including investments backed by U.S. government agencies, corporate debt securities and money market accounts. Cash in excess of immediate requirements is invested with a view toward liquidity and capital preservation, and we seek to minimize the potential effects of concentration and degrees of risk.

In 2013, we closed public offerings of 16,052,684 shares of common stock that resulted in net proceeds of approximately \$245.7 million.

On October 8, 2014, we closed an underwritten public offering of 6,200,000 shares of our common stock, at a public offering price of \$26.00 per share. In addition, on October 3, 2014, the underwriters of the offering exercised their over-allotment option to purchase an additional 930,000 shares from us at the public offering price. The offer and sale of all of the shares in the offering were registered under the Securities Act pursuant to an automatically effective registration statement on Form S-3 ASR. The net proceeds from the offering to us including the over-allotment option, net of underwriting discounts and commissions and offering costs of approximately \$10.7 million, were approximately \$174.7 million.

On March 13, 2015, we closed an underwritten public offering of 2,495,652 shares of our common stock at a public offering price of \$40.00 per share. In addition, on March 11, 2015 the underwriters exercised their over-allotment option to purchase an additional 374,348 shares from us at the public offering price. The offer and sale of all of the shares in the offering were registered under the Securities Act pursuant to our automatically effective shelf registration statement on Form S-3 ASR (File No. 333-199094) previously filed with SEC. The net proceeds from the offering to us including the over-allotment option, net of underwriting discounts, commissions and offering expenses of approximately \$0.4 million were approximately \$108.4 million.

Since inception, in connection with our agreements with Novartis, Merck, Biogen Idec, BMS and Pfizer, Bayer and Janssen, Lee's, and Daiichi Sankyo we have received payments in the aggregate of \$219.7 million, as initial upfront payments, contingent consideration and a milestone payment. In addition, we have received proceeds of \$317.3 million from the sale of our convertible preferred stock.

The following table summarizes our cash flows for the periods indicated:

	Six Months Ended			% Increase	
	June 30, 2015	2014	Increase / (Decrease)	/ (Decrease)	
	(in thousands, except percentages)				
Cash used in operating activities	\$(95,915 )	\$(33,232 )	\$ 62,683	189	%
Cash used in investing activities	\$(8,097 )	\$(30,440 )	\$ (22,343 )	(73	%)
Cash provided by financing activities	\$ 115,409	\$ 2,967	\$ 112,442	3790	%
Net increase (decrease) in cash	\$ 11,397	\$(60,705 )	\$ 72,102	119	%

#### Cash used in operating activities

Cash used in operating activities was \$96 million for the six months ended June 30, 2015 reflecting a net loss of \$104.1 million, which was decreased by non-cash charges of \$9.9 million for stock-based compensation, \$1.8 million for amortization of premium on investments and \$0.6 million for depreciation and amortization. Cash used in operating activities also reflected a decrease in net operating assets of \$4.3 million primarily due to an increase in prepaid expenses and other current assets of \$10.2 million primarily reflecting clinical trial and manufacturing upfront fees, a decrease in deferred revenue of \$4.7 million due to the recognition of collaboration revenue earned from our collaboration agreements and a decrease in accrued compensation and employee benefits of \$0.5 million due to 2014 bonuses that were paid in the first quarter of 2015. Cash used in operating activities also reflected an increase in accounts payable and accrued and other liabilities of \$7.8 million due increased research and development and clinical study related costs and an increase in other long-term liabilities of \$1.5 million due to higher deferred rent following the lease renewal for our corporate headquarters in South San Francisco.

Cash used in operating activities was \$33.2 million for the six months ended June 30, 2014 reflecting a net loss of \$62.1 million, which was decreased by non-cash charges of \$4.7 million for stock-based compensation, \$1.8 million for amortization of premium on investments and \$0.7 million for depreciation and amortization. Cash used in operating activities also reflected an increase in net operating assets of \$21.6 million primarily due to an increase in deferred revenue of \$18.2 million related to upfront payments from BMS and Pfizer, subject to a 50% refund provision, and Bayer and Janssen in the six months ended June 30, 2014, partially offset by the recognition of collaboration revenue earned of \$4.8 million from our collaboration agreements, increases in accounts payable and accrued and other liabilities of \$4.3 million related to higher clinical study and related costs as we continue to increase our research and development activities. Cash used in operating activities also reflected an increase in prepaid expenses and other current assets of \$1.3 million primarily reflecting recognition of clinical trial upfront fees upon contract execution of \$0.3 million, withholding tax receivable on our Bayer collaboration payment of \$0.7 million, interest receivable on our investment portfolio of \$0.3 million, partially offset by a reduction in unrealized gains on our foreign currency forward contracts of \$0.3 million in the six months ended June 30, 2014. Also reflected in cash used in operating activities is an increase in receivables from collaborations of \$0.1 million primarily due to a receivable from Pfizer pursuant to our collaboration agreement with BMS and Pfizer, which was received in April 2014 and a decrease in accrued compensation and employee benefits of \$0.3 million due to 2013 bonuses that were paid during the six months ended June 30, 2014.

#### Cash used in investing activities

Cash used in investing activities of \$8.0 million for the six months ended June 30, 2015 was primarily related to proceeds from maturities of investments of \$162.1 million, partially offset by purchases of investments of \$167.7 million and capital equipment purchases of \$2.5 million.

Cash used in investing activities of \$30.4 million for the six months ended June 30, 2014 was primarily related to purchases of investments of \$143.5 million and capital equipment purchases of \$1.0 million, partially offset by proceeds from sales of investments of \$3.0 million and proceeds from maturities of investments of \$111.0 million.

#### Cash provided by financing activities

Cash provided by financing activities of \$115.4 million for the six months ended June 30, 2015 was related to proceeds from a public offering of our common stock of \$108.4 million and \$7.0 million in proceeds from the issuance of common stock pursuant to equity award plans.

Cash provided by financing activities of \$3.0 million for the six months ended June 30, 2014, was related to proceeds from the issuance of common stock pursuant to equity award plans.

We believe that our existing capital resources, together with interest thereon, will be sufficient to meet our projected operating requirements for at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Further, our operating plan may change, and we may need additional funds to meet operational needs and capital requirements for product development and commercialization sooner than planned. We currently have no credit facility or committed sources of capital other than potential milestones receivable under our current collaborations. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates and the extent to which we may enter into additional collaborations with third parties to participate in their development and commercialization, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical studies. Our future funding requirements will depend on many factors, including the following:

- the scope, rate of progress, results and cost of our clinical studies, preclinical testing and other related activities;
- the cost of manufacturing clinical supplies, and establishing commercial supplies, of our product candidates and any products that we may develop, including process improvements in order to manufacture Andexanet alfa at commercial scale;
- the receipt of any collaboration payments;
- the number and characteristics of product candidates that we pursue;
- the cost, timing and outcomes of regulatory approvals;
- the cost and timing of establishing sales, marketing and distribution capabilities;
- the terms and timing of any other collaborative, licensing and other arrangements that we may establish;
- the timing, receipt and amount of sales, profit sharing or royalties, if any, from our potential products;
- the cost of preparing, filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; and
- the extent to which we acquire or invest in businesses, products or technologies, although we currently have no commitments or agreements relating to any of these types of transactions.

If we need to raise additional capital to fund our operations, funding may not be available to us on acceptable terms, or at all. If we are unable to obtain adequate financing when needed, we may have to delay, reduce the scope of or suspend one or more of our clinical studies, research and development programs or commercialization efforts. We may seek to raise any necessary additional capital through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other marketing and distribution arrangements. To the extent that we raise additional capital through marketing and distribution arrangements or other collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we do raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

#### Off-balance sheet arrangements and contractual obligations

On July 1, 2014, we entered into a commercial supply agreement with CMC Biologics, pursuant to which CMC Biologics will manufacture clinical and commercial supply of Andexanet alfa and perform pre-validation and validation work on our behalf. Total fixed commitments under the agreement for the purchases of clinical and commercial batches, not taking into account possible price and batch adjustments, are \$293.9 million over the life of the agreement from 2015 through 2021.

Under the consolidation accounting guidance, we determined that CMC Biologics is a variable interest entity, or VIE, but that Portola is not CMC Biologics' primary beneficiary and therefore consolidation of CMC Biologics by us is not required. We based this determination on, among other factors, the upfront and reservation payment being akin to a form of subordinated financing, the fixed pricing terms of the arrangement creating variability that is absorbed by the Company, and that we do not have the power to direct the activities that most significantly affect the economic performance of CMC Biologics.

As of June 30, 2015, we have not provided financial, or other, support to CMC Biologics that was not previously contractually required. The upfront fee of \$14.6 million recorded in prepaid and other long-term assets and the batch initiation payments of \$6.4 million recorded in prepaid expenses and other current assets in the condensed consolidated balance sheet represents our maximum exposure to loss under this agreement at June 30, 2015. The upfront payment will be charged to research and development expense, or cost of sales upon regulatory approval of Andexanet alfa, as batches are delivered over the term of the agreement. We are currently not able to quantify the exposure to losses associated with the fixed pricing terms of this agreement.

We may terminate the agreement unilaterally if we discontinue the development and commercialization of Andexanet alfa for regulatory, safety, efficacy or other commercial reasons, or if the projected market demand or gross margin of Andexanet alfa is below a minimum threshold, in which case we will be obligated to pay CMC Biologics a termination payment ranging from between \$5.0 million and \$30.0 million, depending on the time of termination.

There were no other material changes during the six months ended June 30, 2015 outside the ordinary course of business in our specified contractual obligations as disclosed in our Annual Report on Form 10-K for the year ended December 31, 2014, filed with the SEC on March 2, 2015.



ITEM 3: QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

The primary objective of our investment activities is to preserve our capital to fund our operations. We also seek to maximize income from our investments without assuming significant risk. To achieve our objectives, we maintain a portfolio of cash equivalents and investments in a variety of securities of high credit quality. As of June 30, 2015, we had cash, cash equivalents and investments of \$407.6 million consisting of cash and liquid investments deposited in highly rated financial institutions in the United States. A portion of our investments may be subject to interest rate risk and could fall in value if market interest rates increase. However, because our investments are primarily short-term in duration, we believe that our exposure to interest rate risk is not significant and a 1% movement in market interest rates would not have a significant impact on the total value of our portfolio. We actively monitor changes in interest rates.

We contract for the conduct of certain clinical development and manufacturing activities with vendors in Europe. Beginning in 2012, we have utilized foreign currency forward contracts to mitigate our exposure to foreign currency gains and losses. We made payments in the aggregate amount of €5.0 million and €9.9 million to our European vendors during the three months ended June 30, 2015 and 2014, respectively. We are subject to exposure due to fluctuations in foreign exchange rates in connection with these agreements and with our cash balance denominated in Euros and British Pounds, to a lesser extent. For the three months ended June 30, 2015, the effect of the exposure to these fluctuations in foreign exchange rates was not material.

#### ITEM 4: CONTROLS AND PROCEDURES

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in our reports under the Securities Exchange Act of 1934, as amended, or the Exchange Act, and the rules and regulations thereunder, is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms and that such information is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow for timely decisions regarding required disclosure. In designing and evaluating the disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and management is required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

##### Evaluation of Disclosure Controls and Procedures

As required by Rule 13a-15(b) under the Exchange Act, our management, under the supervision and with the participation of our principal executive officer and principal financial officer, has evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of June 30, 2015. Based on such evaluation, our principal executive officer and principal financial officer have concluded that, as of June 30 2015, our disclosure controls and procedures were effective at the reasonable assurance level.

##### Changes in Internal Control Over Financial Reporting

There were no changes in our internal controls over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the quarter ended June 30, 2015 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

We are not currently a party to any material legal proceedings.

#### Item 1A. RISK FACTORS.

Investing in our common stock involves a high degree of risk. You should consider carefully the following risks, together with all the other information in this report, including our financial statements and notes thereto, before you invest in our common stock. If any of the following risks actually materializes, our operating results, financial condition and liquidity could be materially adversely affected. As a result, the trading price of our common stock could decline and you could lose part or all of your investment.

#### RISKS RELATED TO OUR FINANCIAL CONDITION AND NEED FOR ADDITIONAL CAPITAL

We have incurred significant losses since 2012, and expect to incur substantial and increasing losses for the foreseeable future.

We are a clinical-stage biopharmaceutical company. We do not currently have any products approved for sale, and we continue to incur significant research and development and general and administrative expenses related to our operations. We expect to incur substantial and increasing losses for the foreseeable future. As of June 30, 2015, we had an accumulated deficit of approximately \$526.5 million.

To date, we have financed our operations primarily through sales of our equity securities, collaborations, and to a lesser extent, government grants, equipment leases, venture debt and with the benefit of tax credits made available under a federal stimulus program supporting drug development. We have devoted substantially all of our efforts to research and development, including clinical studies, but have not completed development of any product candidates. We anticipate that we will continue to incur substantial expenses as we:

- initiate or continue clinical studies of our three most advanced product candidates;
- continue the research and development of our product candidates;
- seek to discover or in-license additional product candidates;
- seek regulatory approvals for our product candidates that successfully complete clinical studies;
- establish a sales, marketing and distribution infrastructure and scale-up manufacturing capabilities to commercialize products for which we may obtain regulatory approval, including process improvements in order to manufacture Andexanet alfa at commercial scale; and
- enhance operational, compliance, financial and information management systems and hire more personnel, including personnel to support development of our product candidates and support our commercialization efforts.

To be profitable in the future, we must succeed in developing and commercializing products with significant market potential. This will require us to be successful in a range of activities, including advancing our product candidates, completing clinical studies of our product candidates, obtaining regulatory approval for these product candidates and manufacturing, marketing and selling those products for which we may obtain regulatory approval. We are only in the preliminary stages of some of these activities. We may not succeed in these activities and may never generate revenue that is sufficient to be profitable in the future. Even if we are profitable, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to achieve sustained profitability would depress the value of our company and could impair our ability to raise capital, expand our business, diversify our product candidates,

market our product candidates, if approved, or continue our operations.

Our operating results may fluctuate significantly, which makes our future operating results difficult to predict and could cause our operating results to fall below expectations or our guidance.

Our quarterly and annual operating results may fluctuate significantly in the future, which makes it difficult for us to predict our future operating results. From time to time, we enter into collaboration agreements with other companies that include development funding and significant upfront and milestone payments, and we expect that amounts earned from our collaboration agreements will continue to be an important source of our revenue. Accordingly, our revenue will depend on development funding and the achievement of development and clinical milestones under our existing collaboration arrangements, as well as any potential future collaboration and license agreements and sales of our products, if approved. These upfront and milestone payments may vary significantly from period to period and any such variance could cause a significant fluctuation in our operating results from one period to the next. Furthermore, our operating results may fluctuate due to a variety of other factors, many of which are outside of our control and may be difficult to predict, including the following:

- the timing and cost of, and level of investment in, research and development activities relating to our product candidates, which may change from time to time;
- the cost of manufacturing our product candidates, which may vary depending on United States Food and Drug Administration, or FDA, guidelines and requirements, the quantity of production, technical challenges and the terms of our agreements with manufacturers;
- expenditures that we will or may incur to acquire or develop additional product candidates and technologies;
- the level of demand for our product candidates, should they receive approval, which may vary significantly;
- future accounting pronouncements or changes in our accounting policies;
- the timing and success or failure of clinical studies for our product candidates or competing product candidates, or any other change in the competitive landscape of our industry, including consolidation among our competitors or partners;
- the risk/benefit profile, cost and reimbursement policies with respect to our products candidates, if approved, and existing and potential future drugs that compete with our product candidates; and
- the changing and volatile global economic environment.

The cumulative effects of these factors could result in large fluctuations and unpredictability in our quarterly and annual operating results. As a result, comparing our operating results on a period-to-period basis may not be meaningful. Investors should not rely on our past results as an indication of our future performance. This variability and unpredictability could also result in our failing to meet the expectations of industry or financial analysts or investors for any period. If our revenue or operating results fall below the expectations of analysts or investors or below any forecasts we may provide to the market, or if the forecasts we provide to the market are below the expectations of analysts or investors, the price of our common stock could decline substantially. Such a stock price decline could occur even when we have met any previously publicly stated revenue or earnings guidance we may provide.

We will need additional funds to support our operations, and such funding may not be available to us on acceptable terms, or at all, which would force us to delay, reduce or suspend our research and development programs and other operations or commercialization efforts. Raising additional capital may subject us to unfavorable terms, cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our product candidates and technologies.

We are advancing multiple product candidates through the research and clinical development process. The completion of the development and the potential commercialization of our product candidates, should they receive approval, will require substantial funds. As of June 30, 2015, we had \$407.6 million in cash, cash equivalents and investments. We believe that our available cash, cash equivalents and investments will be sufficient to fund our anticipated level of operations for at least the next 12 months. Our future financing requirements will depend on many factors, some of

which are beyond our control, including the following:

- the rate of progress and cost of our clinical studies;
- the timing of, and costs involved in, seeking and obtaining approvals from the FDA and other regulatory authorities;
- the costs of commercialization activities if any of our product candidates is approved, including product sales, marketing, manufacturing and distribution;
- the possible development of additional product candidates, including through in-licensing and acquisitions;
- the degree and rate of market acceptance of any products launched by us or future partners;

33

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- our ability to enter into additional collaboration, licensing, commercialization or other financing arrangements and the terms and timing of such arrangements; and
- the emergence of competing technologies or other adverse market developments.

Until we can generate a sufficient amount of product revenue to finance our cash requirements, which we may never do, we expect to finance future cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic alliances, licensing arrangements and other financing, marketing and distribution arrangements. Additional financing may not be available to us when we need it or it may not be available on favorable terms.

If we raise additional capital through financing, marketing and distribution arrangements or other collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish certain valuable rights to our product candidates, technologies, future revenue streams or research programs or grant licenses on terms that may not be favorable to us. If we raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we are unable to obtain adequate financing when needed, we may have to delay, reduce the scope of, or suspend one or more of our clinical studies, research and development programs or commercialization efforts.

#### RISKS RELATED TO THE DEVELOPMENT AND COMMERCIALIZATION OF OUR PRODUCT CANDIDATES

Our success depends heavily on the approval and successful commercialization of our lead product candidates, Betrixaban and Andexanet alfa along with Cerdulatinib. Clinical studies of these product candidates may not be successful. If we are unable to commercialize one or more of our product candidates, or experience significant delays in doing so, our business will be materially harmed.

We have invested a significant portion of our efforts and financial resources into the development of Betrixaban, Andexanet alfa, and, to a lesser extent, Cerdulatinib and our selective Syk inhibitor program. 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. The success of our product candidates will depend on several factors, including the following:

- successful enrollment in, and completion of, clinical studies;
- our ability to reach agreement with the FDA and other regulatory authorities on the appropriate regulatory path for approval of our product candidates;
- receipt of marketing approvals from the FDA and similar regulatory authorities outside the United States for our product candidates;
- establishing commercial manufacturing arrangements with third parties;
- ability to manufacture product commercially at acceptable costs;
- commercializing any product candidate that may be approved, whether alone or in collaboration with others;
- acceptance of any approved product by the medical community, third-party payors and patients;
- effectively competing with other therapies;
- a continued acceptable safety profile of the product following approval; and
- obtaining, maintaining, enforcing and defending intellectual property rights and claims.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business.





If clinical studies of our product candidates fail to demonstrate safety and efficacy to the satisfaction of the FDA or similar regulatory authorities outside the United States or do not otherwise produce positive results, we may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

Before obtaining regulatory approval for the sale of our product candidates, we must conduct extensive clinical studies to demonstrate the safety and efficacy of our product candidates in humans. Clinical studies are expensive, difficult to design and implement, can take many years to complete and are uncertain as to outcome. A failure of one or more of our clinical studies could occur at any stage of testing. The outcome of preclinical testing and early clinical studies may not be predictive of the success of later clinical studies, and interim results of a clinical study do not necessarily predict final results.

For example, the favorable results from our Phase 2 clinical studies of Betrixaban, which involved the prophylaxis, or preventive treatment, against venous thromboembolism, or VTE, in patients receiving total knee replacements and the prevention of stroke in patients with atrial fibrillation, may not be predictive of success in our current Phase 3 APEX clinical study of Betrixaban for extended duration VTE prophylaxis for 35 days of in-hospital and post-discharge use in acute medically ill patients with elevated blood levels of D-dimer or over the age of 75, as the Phase 2 studies were not designed to demonstrate statistically significant effectiveness, were in different medical conditions, involved different patient populations or dosing regimens, were of different duration or had different comparators. Any of these factors and other factors could result in Betrixaban showing decreased activity or increased safety risks in our APEX study as compared to the Phase 2 studies.

Moreover, the probability of our APEX study succeeding is highly dependent on the adequacy of its design. Two other Factor Xa inhibitors have failed in Phase 3 trials for the indication that we are pursuing for Betrixaban. We have reviewed publicly available data from those studies and incorporated the results of our analysis into the design of our APEX study, but we could have misinterpreted the data or performed a flawed analysis. Furthermore, relevant information from the studies may not be publicly available or, if available, may not have been obtained by us. As a result, there could be flaws in the design of our APEX study that could cause it to fail. For example, our patient inclusion criteria for the APEX study selects for patients with a higher risk of VTE, and these patients may be more likely to experience a severe bleeding event, even though we attempt to exclude certain patients at higher risk of bleeding. If patients in the APEX study experience a higher than expected rate of severe bleeding events, the APEX study may fail to demonstrate a sufficient safety profile for Betrixaban. In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain regulatory approval for the marketing of their products.

Similarly, the favorable results from our Phase 2 proof-of concept studies of Andexanet alfa, evaluating the effect of Andexanet alfa in healthy volunteers taking apixaban, rivaroxaban, edoxaban or enoxaparin, may not be predictive of success in our other Phase 3 or other later studies. In addition, although parts 1 of our Phase 3 ANNEXA-A (apixaban) and ANNEXA-R (rivaroxaban) studies demonstrated that, for the primary efficacy endpoint, an intravenous bolus of Andexanet alfa immediately and significantly reversed the anticoagulation activity of apixaban and rivaroxaban, and part 2 of our ANNEXA-A study demonstrated that, for all the primary and secondary endpoints, an intravenous bolus of Andexanet alfa followed by a continuous two-hour infusion sustained the reversal of anticoagulation activity of apixaban, these positive results may not be predictive of success in our ANNEXA-4 confirmatory study in patients receiving apixaban, rivaroxaban, edoxaban or enoxaparin who present with acute major bleeding, or in part 2 of our bolus plus continuous infusion ANNEXA-R study. We also do not yet know how the results from our clinical studies of Andexanet alfa in healthy volunteers who have received a Factor Xa inhibitor followed by Andexanet alfa will translate into clinical outcomes in patients. Moreover, the results from our studies to date of Andexanet alfa may not address the effect of repeat doses or allow a determination of the optimal therapeutic

dose of Andexanet alfa for our intended target patient population.

We may experience numerous unforeseen events during, or as a result of, clinical studies that could delay or prevent our ability to receive regulatory approval or commercialize our product candidates, including the following:

- clinical studies of our product candidates may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional clinical studies or abandon product development programs;
- the number of patients required for clinical studies of our product candidates may be larger than we anticipate, enrollment in these clinical studies may be insufficient or slower than we anticipate or patients may drop out of these clinical studies at a higher rate than we anticipate;
- the cost of clinical studies or the manufacturing of our product candidates may be greater than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we might have to suspend or terminate clinical studies of our product candidates for various reasons, including unanticipated serious side effects, other unexpected characteristics or unacceptable health risks;

35

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- regulators may not approve our proposed clinical development plans;
- regulators or institutional review boards may not authorize us or our investigators to commence a clinical study or conduct a clinical study at a prospective study site;
- regulators or institutional review boards may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements; and
- the supply or quality of our product candidates or other materials necessary to conduct clinical studies of our product candidates may be insufficient or inadequate.

If we are required to conduct additional clinical studies or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical studies of our product candidates or other testing, if the results of these studies or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications that are not as broad as intended;
- have the product removed from the market after obtaining marketing approval;
- be subject to additional post-marketing testing requirements; or
- be subject to restrictions on how the product is distributed or used.

Our product development costs will also increase if we experience delays in testing or approvals. We do not know whether any clinical studies will begin as planned, will need to be restructured or will be completed on schedule, or at all. Significant clinical study delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which would impair our ability to commercialize our product candidates and harm our business and results of operations.

If serious adverse side effects are identified during the development of any of our product candidates, we may need to abandon our development of that product candidate.

None of our product candidates has completed clinical development. The risk of failure of clinical development is high. It is impossible to guarantee when or if any of our product candidates will prove safe enough to receive regulatory approval. For example, our product candidate Betrixaban, like all currently marketed inhibitors of Factor Xa, carries some risk of life-threatening bleeding. In addition, patients taking Betrixaban in our Phase 2 studies had an increased rate of gastrointestinal issues, such as diarrhea, nausea and vomiting, and other side effects such as back pain, dizziness, headaches, rashes and insomnia as compared to subjects taking a placebo or an active comparator. There can be no assurance that our APEX study or other clinical studies will not fail due to safety issues. In such an event, we might need to abandon development of that product candidate or enter into a partnership to continue development.

The failure of two of our competitors' clinical trials evaluating Factor Xa inhibitors for VTE prophylaxis in acute medically ill patients may suggest an increased risk that our APEX trial for Betrixaban will also fail.

Two of our competitors' clinical trials evaluating Factor Xa inhibitors for VTE prophylaxis in acute medically ill patients have failed. The MAGELLAN trial sponsored by Bayer Pharma AG, or Bayer, and Janssen Pharmaceuticals, Inc., or Janssen, which evaluated rivaroxaban, demonstrated efficacy but failed to demonstrate an acceptable benefit to risk profile due to increased bleeding. The ADOPT trial sponsored by Bristol-Myers Squibb Company, which evaluated apixaban, showed a reduction in VTE events, but failed to demonstrate statistically significant efficacy and also showed an increase in bleeding. Betrixaban, like rivaroxaban and apixaban, may fail in clinical trials if it does not show a statistically significant level of efficacy or if the resulting bleeding risk is too high compared to its benefits.

Delays in the enrollment of patients in any of our clinical studies could increase our development costs and delay completion of our clinical studies and associated regulatory submissions.

We may not be able to initiate or continue clinical studies for our product candidates if we are unable to locate and enroll a sufficient number of eligible patients to participate in these studies as required by the FDA or other regulatory authorities. Even if we are able to enroll a sufficient number of patients in our clinical studies, if the pace of enrollment is slower than we expect, the development costs for our product candidates may increase, and the completion of our studies may be delayed or our studies could become too expensive to complete.

For example, our Phase 3 Betrixaban APEX study has been expanded and is now expected to enroll approximately 7,500 patients from over 450 study sites throughout the world. We have never previously conducted a study of this magnitude and can provide no assurance that we will be able to enroll patients to complete the study within our projected time frame. The first patient was enrolled in APEX in March 2012, and, based on current enrollment, we expect patient enrollment, as expanded, to be completed by the end of 2015, which will require us to continue to enroll patients at established rates. If we experience delays in enrollment, our ability to complete our APEX study could be materially adversely affected. If we are unable to continue to enroll the patients at the established rate, the completion of the study could be delayed and the costs of conducting the study could increase, either of which could have a material adverse effect on our business.

As another example, the ANNEXA-4 study of Andexanet alfa is our first experience in patients with major bleeding who are receiving a factor Xa inhibitor. Because we have no first-hand enrollment experience in this patient population, our enrollment forecasts are estimated based on our understanding of enrollment experience of similar studies conducted by others in similar patient populations. Our current forecasts suggest that enrolling up to 270 patients should ensure that a sufficient number are able to be included in the primary analysis. However, if after enrolling 270 patients, the true number of evaluable patients is less than required, it may be necessary to continue enrolling additional patients beyond the planned 270. Enrollment of additional patients (or slower than anticipated enrollment of the currently planned 270 patients) could increase the cost and duration of the study, and could result in alterations of the clinical plan including, but not limited to, opening of additional sites or geographic regions, both of which would result in increased costs. In addition, our current enrollment forecasts indicate that enrollment of enough patients to satisfy requests from regulators will occur in time to support an Andexanet alfa BLA submission in 2015. However, if the actual enrollment rate differs from our predicted rate, we may not meet these enrollment targets which could delay the BLA submission.

Even if Andexanet alfa is approved by the FDA, this approval may be limited to certain indications, additional clinical studies and regulatory applications may be required to expand Andexanet alfa indications and we can provide no assurances that such additional clinical studies or regulatory applications will be successful.

We are developing Andexanet alfa as a universal antidote for patients receiving a Factor Xa inhibitor anticoagulant who suffer a major bleeding episode or who may require emergency surgery. Our ANNEXA-4 Phase 4 study is being conducted in patients receiving either a direct or indirect Factor Xa inhibitor who present with an acute major bleed, and our ANNEXA Phase 3 registration-enabling studies have been conducted on healthy volunteers. It is not certain at this time which indications, if any, the FDA will approve based on this data. It is possible that additional clinical studies will be required to support our targeted indications, which would require additional time and expense and may not prove successful. Limitations in our label for Andexanet alfa would reduce the number of patients for whom Andexanet alfa is indicated and could reduce the size of the anticipated market and our financial prospects.

Even if our APEX study demonstrates statistically significant efficacy and safety of Betrixaban for extended duration VTE prophylaxis in acute medically ill patients for 35 days of in-hospital and post-discharge use, the FDA or similar regulatory authorities outside the United States may not approve Betrixaban for marketing or may approve it with restrictions on the label, which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

Assuming the success of our APEX study, we anticipate seeking regulatory approval for Betrixaban in the United States for extended duration VTE prophylaxis in acute medically ill patients for 35 days of in-hospital and post-discharge use. It is possible that the FDA may not consider the results of our APEX study to be sufficient for approval of Betrixaban for this indication. In general, the FDA suggests that sponsors complete two adequate and well-controlled clinical studies to demonstrate effectiveness because a conclusion based on two persuasive studies will be more compelling than a conclusion based on a single study. Although the FDA has informed us that our APEX

study, plus supportive Phase 2 data obtained to date, could potentially provide sufficient safety and efficacy data for extended duration VTE prophylaxis in acute medically ill patients for 35 days of in-hospital and post-discharge use, the FDA has further advised us that whether one or two adequate and well-controlled clinical studies are required will be a review issue in connection with a new drug application, or NDA, submission. Even if we achieve favorable results in our APEX study, the FDA may nonetheless require that we conduct additional clinical studies, possibly using a different clinical study design.

Even if the FDA or other regulatory authorities approve Betrixaban for VTE prophylaxis in acute medically ill patients, the approval may include additional restrictions on the label that could make Betrixaban less attractive to physicians and patients than other products that may be approved for broader indications, which could reduce the potential market for Betrixaban.

We are seeking regulatory approval of Andexanet alfa in the United States through an Accelerated Approval process, and since we have limited experience with this process, the development or commercialization of Andexanet alfa could be delayed or abandoned.

In November 2013, the FDA granted breakthrough therapy designation for Andexanet alfa which allows for an Accelerated Approval process. In addition, we have reached agreement with the FDA on an Accelerated Approval strategy. The Accelerated Approval regulations allow drugs that are being developed to treat an unmet medical need to be approved substantially based on evidence of an effect on a surrogate biomarker endpoint that is considered reasonably likely to predict clinical benefit rather than a clinical endpoint such as survival or irreversible morbidity. Use of an Accelerated Approval process provides a shortened timetable to approval, but a Phase 4 clinical study with clinical endpoints that will confirm the validity of the surrogate endpoint(s) must be ongoing at the time our BLA is submitted and some early patient data will be required by the FDA to support the BLA. We expect that this study will continue into commercialization. Because of the accelerated timelines required for accelerated approval, we may require more time and incur greater costs than anticipated and may not succeed in timely manufacture of drug supply or in obtaining regulatory approval of Andexanet alfa. In addition, the FDA may subsequently determine that the studies conducted by us were insufficient to support approval or require us to conduct extensive post-approval studies.

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

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

- the prevalence and severity of any side effects;
- efficacy and potential advantages compared to alternative treatments;
- the price we charge for our product candidates;
- 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;
- convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the strength of marketing and distribution support; and
- the availability of third-party coverage or reimbursement.

For example, while there are no approved therapies for VTE prophylaxis in acute medically ill patients approved for use beyond the typical hospitalization period, there are therapies available for in-hospital use and physicians may not be willing to change their current in-hospital treatment practices in favor of Betrixaban. If our product candidates, if approved, 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.

There are risks associated with scaling up manufacturing to commercial scale. Our commercial manufacturing strategy for Andexanet alfa is particularly complex and challenging. If our manufacturers are unable to manufacture our products on a commercial scale or scale to increased production, this could potentially delay regulatory approval and commercialization or materially adversely affect our results of operations.

There are risks associated with scaling up manufacturing to commercial volumes including, among others, cost overruns, technical problems with process scale-up, process reproducibility, stability issues, lot consistency and timely availability of raw materials. Even if we could otherwise obtain regulatory approval for any product candidate, there is no assurance that our manufacturer will be able to manufacture the approved product to specifications acceptable to

the FDA or other regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential launch of the product or to meet potential future demand. If our manufacturers are unable to produce sufficient quantities of the approved product for commercialization, either on a timely basis or at all, our commercialization efforts would be impaired, which would have a material adverse effect on our business, financial condition, results of operations and growth prospects.



In particular, we face uncertainties and risks associated with scaling up the manufacturing for Andexanet alfa. Andexanet alfa is a recombinant biological molecule, or biologic, rather than a small molecule chemical compound like our other product candidates. The manufacture of biologics involves complex processes, typically including developing cell lines or cell systems to produce the biologic, growing large quantities of such cells and harvesting and purifying the biologic produced by them. The cost to manufacture biologics is generally far higher than traditional small molecule chemical compounds, and the manufacturing process is more complex and can be difficult to reproduce. There is no guarantee we will be successful in establishing a larger-scale commercial manufacturing process for Andexanet alfa which achieves our objectives for manufacturing capacity and cost of goods. Due, in part, to the high cost to manufacture Andexanet alfa and the inherent uncertainty related to manufacturing costs, if the effective dose of Andexanet alfa is higher than we anticipate or the obtainable sales price is lower than we anticipate, there is a greater risk that Andexanet alfa may not be commercially viable.

Andexanet alfa used in our clinical studies is currently produced for us by a third-party contract manufacturer, CMC ICOS Biologics, Inc., or CMC Biologics, who will also support our initial BLA submission and initial commercial launch in the U.S. However, to support broader U.S. and worldwide supply with a lower cost, we must also increase production capacity at CMC Biologics, add production from Lonza or another larger-scale manufacturer, and improve the manufacturing process to increase the yield and lower the manufacturing costs. Developing a commercial manufacturing process with two separate commercial manufacturing organizations increases the cost and complexity of commercial manufacturing which could increase the risk of successful implementation of our commercial manufacturing supply strategy.

We do not anticipate that supply from CMC Biologics, even as expanded, will be sufficient to meet projected worldwide demand for Andexanet alfa, therefore, we must also develop an improved and more cost-effective process at Lonza. However, the first commercial material from Lonza will not become available until after our expected U.S. launch. There is significant technical and regulatory work which we will need to complete before Lonza is able to produce commercial quantities of Andexanet alfa and there remains substantial uncertainty whether Lonza will be able to produce commercial supply of Andexanet alfa at the quantities and cost of goods necessary for commercial success. Previous scale-up work was unsuccessful in achieving acceptable product yield within the desired timeline.

In addition, in order to obtain FDA approval of material produced by a new vendor or using a new process, we will need to demonstrate that such material is comparable to the clinical material we previously used and material produced by CMC Biologics. Demonstrating comparability can require significant pre-clinical and clinical studies. If we are not able to demonstrate comparability, then the material may be considered a new biological entity and a new clinical program, possibly commencing with Phase 1, and a full BLA submission may be required for approval, resulting in additional time and expense. If we are not able to establish targeted capacity at CMC Biologics and Lonza on a timely basis, implement the proposed transitions in a timely manner, or establish comparability of the new material, or obtain the anticipated improvements in efficiency, our business, financial condition, results of operations and growth prospects would be materially adversely affected.

We currently have limited sales and distribution personnel and are in the initial stages of developing marketing capabilities. If we are unable to develop a sales and marketing and distribution capability on our own or through collaborations or other marketing partners, we will not be successful in commercializing Betrixaban, Andexanet alfa or other future products.

We do not currently have a significant sales or marketing infrastructure and have never sold, marketed or distributed therapeutic products. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties. We plan to establish a hospital-based sales force in the United States and possibly other major markets and work with partners in other parts of the world to commercialize both Betrixaban and Andexanet alfa globally, if they are approved. There are risks involved with both

establishing our own sales and marketing capabilities and entering into arrangements with third parties to perform these services. For example, recruiting and training a sales force is expensive and time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

We also may not be successful entering into arrangements with third parties to sell and market 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 our products 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.

We face substantial competition, which may result in others discovering, developing or commercializing competing products before or more successfully than we do.

The development and commercialization of new therapeutic products is highly competitive. We face competition with respect to our current product candidates, and will face competition with respect to any products that we may seek to develop or commercialize in the future, from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide. For example, several large pharmaceutical and biotechnology companies currently market and sell direct or indirect Factor Xa inhibitors for use in various disease states, including injectable Factor Xa inhibitors for the prevention of VTE in acute medically ill patients. Potential competitors also include academic institutions, government agencies and other public and private research organizations that conduct research, seek patent protection and establish collaborative arrangements for research, development, manufacturing and commercialization. Many of these competitors are attempting to develop therapeutics for our target indications.

In addition, many of our competitors are large pharmaceutical companies that will have a greater ability to reduce prices for their competing drugs in an effort to gain market share and undermine the value proposition that we might otherwise be able to offer to payors. We are developing our product candidate Betrixaban for extended duration VTE prophylaxis in acute medically ill patients for 35 days of in-hospital and post-discharge use. The current standard of care for VTE prophylaxis in acute medically ill patients in the United States is a 6- to 14-day hospital administration of enoxaparin, marketed as Lovenox® and also available in generic form, an indirect Factor Xa inhibitor. Enoxaparin is widely accepted by physicians, patients and third-party payors. As a result, we may face difficulties in marketing Betrixaban as a substitute therapy in the hospital for the current standard of care, enoxaparin.

Furthermore, the FDA has already approved a number of therapies that, like Betrixaban, are oral direct Factor Xa inhibitors and that have already achieved substantial market acceptance. Although these products have not been approved for VTE prophylaxis in acute medically ill patients, the owners of the products may decide to seek such approval or physicians may decide to prescribe these products for the treatment of VTE in acute medically ill patients absent such approval, known as prescribing “off-label.” Further, our competitors may have the financial and other resources to conduct additional clinical studies in an effort to obtain regulatory approval for use of their drugs for VTE prophylaxis in acute medically ill patients, even in cases where they have previously run clinical trials that have failed. For example, in March 2014, Bayer and Janssen announced the initiation of a new Phase 3 clinical trial to evaluate the safety and efficacy of rivaroxaban to reduce the risk of post-hospital discharge symptomatic VTE in patients hospitalized for acute medical illness.

While there are no therapies approved specifically as antidotes for Factor Xa inhibitors, we are aware of at least one drug candidate being studied in early stage clinical trials as a potential antidote to Factor Xa inhibitors. In addition, in December 2014, Bristol-Myers Squibb Company and Pfizer Inc. announced that a clinical trial of 15 healthy human subjects demonstrated that 4-factor prothrombin complex concentrate may affect the steady-state pharmacodynamics effects of Eliquis (apixaban). Andexanet alfa, if approved, may compete with other currently approved treatments designed to enhance coagulation, such as fresh frozen plasma, prothrombin complex concentrates, recombinant Factor VIIa or whole blood. Although there is no clinical evidence supporting the use of such treatments in patients taking Factor Xa inhibitors, physicians may choose to use them because of familiarity, cost or other reasons. In addition, we are aware that several companies have conducted preclinical research on compounds intended to be antidotes for Factor Xa inhibitors.

There are also a number of products in clinical development for hematologic cancer, ophthalmological diseases, allergic rhinitis, allergic asthma and other inflammatory diseases that are potential indications for Cerdulatinib or selective Syk inhibitors. Our competitors may develop products that are more effective, safer, more convenient or less costly than any that we are developing or that would render our product candidates obsolete or noncompetitive. Many competing products are in later stages of development than our products and are, therefore, likely to obtain FDA or

other regulatory approval for their products before we obtain approval for ours. Many of our competitors, including a number of large pharmaceutical companies that compete directly with us, have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical, biotechnology and diagnostic industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical study sites and patient registration for clinical studies, as well as in acquiring technologies complementary to, or necessary for, our programs.

## RISKS RELATED TO OUR RELIANCE ON THIRD PARTIES

We rely on third parties to conduct our clinical studies, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such studies.

We do not independently conduct clinical studies of our product candidates. We rely on third parties, such as contract research organizations, or CROs, clinical data management organizations, medical institutions and clinical investigators, to perform this function. For example, we rely on PPD Development, LP and other CROs to oversee and manage our APEX study. Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. Furthermore, most of the clinical study sites for our APEX study are outside the United States, including several developing countries. The performance of these sites may be adversely affected by various issues, including less advanced medical infrastructure, lack of familiarity with conducting clinical studies using U.S. standards, insufficient training of personnel, communication difficulties and geopolitical risk. We remain responsible for ensuring that each of our clinical studies is conducted in accordance with the general investigational plan and protocols for the study.

Moreover, the FDA requires us to comply with standards, commonly referred to as good clinical practices, for conducting, recording and reporting the results of clinical studies to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of patients in clinical studies are protected. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical studies in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, regulatory approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates.

We also rely on other third parties to store and distribute supplies for our clinical studies. Any performance failure on the part of our existing or future distributors could delay clinical development or regulatory approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

We rely on third-party contract manufacturing organizations to manufacture and supply our product candidates for us. If one of our suppliers or manufacturers fails to perform adequately or fulfill our needs, we may be required to incur significant costs and devote significant efforts to find new suppliers or manufacturers. We may also face significant delays in the development and commercialization of our product candidates.

We do not own facilities for, clinical-scale or commercial manufacturing of our product candidates. We currently rely critically on individual suppliers for each of our product candidates. For example, we rely on Hovione Inter Limited to produce the active pharmaceutical ingredient for Betrixaban for our APEX study, we have contracted with CMC Biologics to expand its production capacity of Andexanet alfa bulk drug substance to support our potential U.S. commercial launch, and we have engaged Lonza to develop a new, higher-capacity and lower cost process for Andexanet alfa bulk drug substance in order to support our broader, worldwide commercialization strategy. We have not yet entered into a commercial supply agreement for the manufacture of Betrixaban. We also rely or expect to rely on other third party providers for lyophilization, packaging, labeling and supply chain distribution. If we and our suppliers cannot agree to the terms and conditions for them to provide the drug product necessary for our clinical and commercial supply needs, or if any single source supplier terminates the agreement in response to a breach by us or otherwise becomes unable to fulfill its supply obligations, we would not be able to manufacture and distribute the product candidate until a qualified alternative supplier is identified, which could also significantly delay the development of, and impair our ability to commercialize, our product candidates.

The manufacture of pharmaceutical products in compliance with the FDA's current good manufacturing practices, or cGMPs, requires significant expertise and capital investment, including the development of advanced manufacturing techniques and process controls. Manufacturers of pharmaceutical products often encounter difficulties in production, including difficulties with production costs and yields, quality assurance, including stability of the product candidate and quality control testing, shortages of qualified personnel, as well as compliance with strictly enforced cGMP requirements, other federal and state regulatory requirements and foreign regulations. If our manufacturers were to encounter any of these difficulties or otherwise fail to comply with their obligations to us or under applicable regulations, our ability to supply our clinical studies or commercial demand would be jeopardized. Any delay or interruption in the supply of clinical study materials could delay the completion of our clinical studies, increase the costs associated with maintaining our clinical study programs and, depending upon the period of delay, require us to commence new studies at significant additional expense or terminate the studies completely.

All manufacturers of our product candidates must comply with cGMP requirements enforced by the FDA through its facilities inspection program. These requirements include, among other things, quality control, quality assurance and the maintenance of records and documentation. Manufacturers of our product candidates may be unable to comply with these cGMP requirements and with other FDA, state and foreign regulatory requirements. The FDA or similar foreign regulatory agencies may also implement new standards at any time, or change their interpretation and enforcement of existing standards for manufacturing, packaging or testing of products. We have limited control over our manufacturers' compliance with these regulations and standards. A failure to comply with these requirements may result in fines and civil penalties, suspension of production, suspension or delay in product approval, product seizure or recall or withdrawal of product approval. If the safety of any product supplied is compromised due to our manufacturers' failure to adhere to applicable laws or for other reasons, we may not be able to obtain regulatory approval for or successfully commercialize our products and we may be held liable for any injuries sustained as a result. Any of these factors could cause a delay of clinical studies, regulatory submissions, approvals or commercialization of our product candidates, entail higher costs or impair our reputation.

Although alternative sources of supply exist, the number of third-party suppliers with the necessary manufacturing and regulatory expertise and facilities to manufacture biologics is limited, and it could be expensive and take a significant amount of time to arrange for alternative suppliers, which could have a material adverse effect on our business. New suppliers of any product candidate would be required to qualify under applicable regulatory requirements and would need to have sufficient rights under applicable intellectual property laws to the method of manufacturing the product candidate. Obtaining the necessary FDA approvals or other qualifications under applicable regulatory requirements and ensuring non-infringement of third-party intellectual property rights could result in a significant interruption of supply and could require the new manufacturer to bear significant additional costs which may be passed on to us.

We may enter into collaborations that place the development of our product candidates outside our control, require us to relinquish important rights or may otherwise be on terms unfavorable to us, and if our collaborations are not successful, our product candidates may not reach their full market potential.

We may enter into additional collaboration agreements with third parties with respect to our product candidates for the commercialization of the candidates outside the U.S., or for other purposes. In addition, depending on our capital requirements, development and commercialization costs, need for additional therapeutic expertise and other factors, it is possible that we will enter into broader development and commercialization arrangements with respect to our product candidates. Our likely collaborators for any distribution, marketing, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. We will have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenue from these arrangements will depend in part on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

Collaborations involving our product candidates are subject to numerous risks, which may include the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to any such collaborations;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical study results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators may delay clinical studies, provide insufficient funding for a clinical study program, stop a clinical study, abandon a product candidate, repeat or conduct new clinical studies or require a new formulation of a product

- candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to their marketing and distribution;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
- disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our product candidates or that results in costly litigation or arbitration that diverts management attention and resources;



- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates; and
- collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to commercialize such intellectual property.

Any termination or disruption of our collaboration with potential collaborators could result in delays in the development and commercialization of our product candidates, increases in our costs to develop and commercialize the product candidate or the termination of development of a product candidate.

## RISKS RELATED TO THE OPERATION OF OUR BUSINESS

Our future success depends on our ability to retain our chief executive officer and other key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on William Lis, our Chief Executive Officer, and the other principal members of our executive and scientific teams. Under the terms of their employment, our executives may terminate their employment with us at any time. The loss of the services of any of these people could impede the achievement of our research, development and commercialization objectives. We maintain “key person” insurance for Mr. Lis but not for any other executives or employees. Any insurance proceeds we may receive under our “key person” insurance on Mr. Lis would not adequately compensate us for the loss of his services.

Recruiting and retaining qualified scientific, clinical, manufacturing and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us.

We expect to expand our development, regulatory and sales and marketing capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

Over the next several years, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

We are incurring significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, we incur significant legal, accounting and other expenses. For example, the Sarbanes-Oxley Act, and rules of the SEC and those of The NASDAQ Stock Market, or the NASDAQ, have imposed various requirements on public companies including requiring establishment and maintenance of effective disclosure and financial controls. Our management and other personnel have and will need to continue to devote a substantial amount

of time to these compliance initiatives. Moreover, these rules and regulations have increased and will continue to increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal control over financial reporting and disclosure controls and procedures. In particular, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. In addition, we are required to have our independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting. Our compliance with Section 404 of the Sarbanes-Oxley Act, as applicable, requires us to incur substantial accounting expense and expend significant management efforts. We currently do not have an internal audit group, and we will need to continue to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge. If we are not able to comply with the requirements of Section 404, as applicable, in a timely manner, or if we or our independent registered public accounting firm identify deficiencies in our internal control over financial reporting that are deemed to be material weaknesses, the market price of our stock could decline and we could be subject to sanctions or investigations by the NASDAQ, the SEC or other regulatory authorities, which would require additional financial and management resources.

Our ability to successfully implement our business plan and comply with Section 404, as applicable, requires us to be able to prepare timely and accurate financial statements. We expect that we will need to continue to improve existing, and implement new operational and financial systems, procedures and controls to manage our business effectively. Any delay in the implementation of, or disruption in the transition to, new or enhanced systems, procedures or controls, may cause our operations to suffer and we may be unable to conclude that our internal control over financial reporting is effective and to obtain an unqualified report on internal controls from our auditors as required under Section 404 of the Sarbanes-Oxley Act. If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results, and current and potential stockholders may lose confidence in our financial reporting. This, in turn, could have an adverse impact on trading prices for our common stock, and could adversely affect our ability to access the capital markets.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of any products that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical studies and will face an even greater risk if we commercially sell any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of patients from clinical studies or cancellation of studies;
- significant costs to defend the related litigation;
- substantial monetary awards to patients;
- loss of revenue; and
- the inability to commercialize any products that we may develop.

We currently hold \$10.0 million in product liability insurance coverage, which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or

other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products.

If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been advantageous for us to retain sole development and commercialization rights.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological or hazardous materials. In addition, we may be required to incur substantial costs to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.

Our operations could be subject to earthquakes, power shortages, telecommunications failures, floods, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or manmade disasters or business interruptions. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. Our corporate headquarters is located in California near major earthquake faults. Our operations and financial condition could suffer in the event of a major earthquake, fire or other natural or manmade disaster.

If we obtain approval to commercialize any approved products outside of the United States, a variety of risks associated with international operations could materially adversely affect our business. If any product candidates that we may develop are approved for commercialization outside the United States, we will be subject to additional risks related to entering into international business relationships, including:

- different regulatory requirements for drug approvals in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

In connection with our Betrixaban and Andexanet alfa studies, we are currently utilizing certain suppliers outside of the United States, which subjects us to certain of the above risks. For example, a significant number of our APEX trial sites and enrolled patients are in Russia and Ukraine, and the recent political unrest in Ukraine has disrupted activities at some of our trial sites in this region. Continued or worsening political unrest in this region and the effect of international sanctions could adversely affect patient enrollment or other activities at our sites in Ukraine and Russia.

Our internal computer systems, or those of our CROs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our drug development programs.

Despite the implementation of security measures, our internal computer systems and those of our CROs 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 material disruption of our drug development programs. For example, the loss of clinical study data from completed or ongoing clinical studies for any of our product candidates 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 was 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 our product candidates could be delayed.

#### RISKS RELATED TO INTELLECTUAL PROPERTY

If we fail to comply with our obligations in our intellectual property licenses with third parties, we could lose license rights that are important to our business.

We are a party to intellectual property license agreements with third parties, including with respect to Betrixaban, Cerdulatinib and one of our selective Syk inhibitors, and expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that our future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, our licensors may have the right to terminate these agreements, in which event we may not be able to develop and market any product that is covered by these agreements. Termination of these licenses or reduction or elimination of our licensed rights may result in our having to negotiate new or reinstated licenses with less favorable terms or our not having sufficient intellectual property rights to operate our business. The occurrence of such events could materially harm our business.

Our ability to successfully commercialize our technology and products may be materially adversely affected if we are unable to obtain and maintain effective intellectual property rights for our technologies and product candidates.

Our success depends in large part on our and our licensors' ability to obtain and maintain patent and other intellectual property protection in the United States and in other countries with respect to our proprietary technology and products. In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology or products that we license from third parties. Therefore, we cannot be certain that these patents and applications will be prosecuted and enforced in a manner consistent with the best interests of our business. In addition, if third parties who license patents to us fail to maintain such patents, or lose rights to those patents, the rights we have licensed may be reduced or eliminated.

We have sought to protect our proprietary position by filing patent applications in the United States and abroad related to our novel technologies and products that are important to our business. This process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technologies or from developing competing products and technologies.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain and involves complex legal and factual questions for which legal principles remain unresolved. In recent years patent rights have been the subject of significant litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our and our licensors' patent rights are highly uncertain. Our and our licensors' pending and future patent applications may not result in patents being issued which protect our technology or products or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. The laws of foreign countries may not protect our rights to the same extent as the laws of the United States. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we or our licensors were the first to make the inventions claimed in our owned and licensed patents or pending patent applications, or that we or our licensors were the first to file for patent protection of such inventions. Assuming the other requirements for patentability are met, prior to March 16, 2013, in the United States, the first to make the claimed invention is entitled to the patent, while outside the United States, the first to file a patent application is entitled to the patent. On March 16, 2013, under the recently enacted America Invents Act, the United States moved to a first to file system.



The effects of these changes are currently unclear as the United States Patent and Trademark Office, or USPTO, has only recently implemented various regulations, the courts have only just begun to issue decisions addressing these provisions and the applicability of the act and new regulations on specific patents discussed herein have not been determined and would need to be reviewed. We may become involved in opposition or interference proceedings challenging our patent rights or the patent rights of others, and the outcome of any proceedings are highly uncertain. For example, in November 2013, Zentiva k.s. and Günter SÖLCH separately filed papers with the European Patent Office opposing European Patent 2101760, assigned to Millennium Pharmaceuticals, Inc., to which we have an exclusive license. This patent is related to a formulation of Betrixaban. The opposition proceedings are still pending. An adverse determination in this or any other such proceeding could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or products and compete directly with us, without payment to us, or result in our inability to manufacture or commercialize products without infringing third-party patent rights.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner. The issuance of a patent is not conclusive as to its scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop or prevent us from stopping others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours or otherwise provide us with a competitive advantage.

We may become involved in lawsuits to protect or enforce our patents, which could be expensive, time-consuming and unsuccessful.

Competitors may infringe our patents. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours is invalid or unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation proceeding could put one or more of our patents at risk of being invalidated or interpreted narrowly. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the proprietary rights or intellectual property of third parties. We may become party to, or be threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference proceedings before the USPTO. An interference proceeding is a proceeding before the USPTO to determine the priority among multiple patents or patent applications. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third-party's intellectual property rights, we could be required to obtain a license from such third-party to

continue developing and marketing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all.

Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties can have a similar negative impact on our business.

We may be unable to protect the confidentiality of our trade secrets, thus harming our business and competitive position.

In addition to our patented technology and products, we rely upon trade secrets, including unpatented know-how, technology and other proprietary information to develop and maintain our competitive position, which we seek to protect, in part, by confidentiality agreements with our employees and our collaborators and consultants. We also have agreements with our employees and selected consultants that obligate them to assign their inventions to us. However, it is possible that technology relevant to our business will be independently developed by a person that is not a party to such an agreement. Furthermore, if the employees, consultants or collaborators that are parties to these agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets through such breaches or violations. Further, our trade secrets could be disclosed, misappropriated or otherwise become known or be independently discovered by our competitors. In addition, intellectual property laws in foreign countries may not protect our intellectual property to the same extent as the laws of the United States. If our trade secrets are disclosed or misappropriated, it would harm our ability to protect our rights and have a material adverse effect on our business.

We may be subject to claims that our employees have wrongfully used or disclosed intellectual property of their former employers. Intellectual property litigation or proceeding could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Many of our employees were previously employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce our resources available for development activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their substantially greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other intellectual property related proceedings could have a material adverse effect on our ability to compete in the marketplace.

## RISKS RELATED TO GOVERNMENT REGULATION

The regulatory approval process is expensive, time consuming and uncertain and may prevent us from obtaining approvals for the commercialization of some or all of our product candidates.

The research, testing, manufacturing, labeling, approval, selling, import, export, marketing and distribution of drug products are subject to extensive regulation by the FDA and other regulatory authorities in the United States and other countries, which regulations differ from country to country. We will not be permitted to market our product candidates in the United States until we receive approval of an NDA or a BLA, from the FDA. We have not submitted an application or received marketing approval for any of our product candidates. Obtaining approval of an NDA or BLA can be a lengthy, expensive and uncertain process. In addition, failure to comply with FDA and other applicable U.S.

and foreign regulatory requirements may subject us to administrative or judicially imposed sanctions, including the following:

- warning letters;
- civil or criminal penalties and fines;
- injunctions;
- suspension or withdrawal of regulatory approval;
- suspension of any ongoing clinical studies;
- voluntary or mandatory product recalls and publicity requirements;
- refusal to accept or approve applications for marketing approval of new drugs or biologics or supplements to approved applications submitted by us;

48

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- restrictions on operations, including costly new manufacturing requirements; or
- seizure or detention of our products or import bans.

Prior to receiving approval to commercialize any of our product candidates in the United States or abroad, we must demonstrate with substantial evidence from well-controlled clinical studies, and to the satisfaction of the FDA and other regulatory authorities abroad, that such product candidates are safe and effective for their intended uses. Results from preclinical studies and clinical studies can be interpreted in different ways. Even if we and our collaboration partners believe the preclinical or clinical data for our product candidates are promising, such data may not be sufficient to support approval by the FDA and other regulatory authorities. Administering any of our product candidates to humans may produce undesirable side effects, which could interrupt, delay or cause suspension of clinical studies of our product candidates and result in the FDA or other regulatory authorities denying approval of our product candidates for any or all targeted indications.

Regulatory approval of an NDA or BLA is not guaranteed, and the approval process is expensive and may take several years. The FDA also has substantial discretion in the approval process. Despite the time and expense exerted, failure can occur at any stage, and we could encounter problems that cause us to abandon or repeat clinical studies, or perform additional preclinical studies and clinical studies. The number of preclinical studies and clinical studies that will be required for FDA approval varies depending on the product candidate, the disease or condition that the product candidate is designed to address and the regulations applicable to any particular product candidate. The FDA can delay, limit or deny approval of a product candidate for many reasons, including, but not limited to, the following:

- a product candidate may not be deemed safe or effective;
- FDA officials may not find the data from preclinical studies and clinical studies sufficient;
- the FDA may find our manufacturing data insufficient to support approval
- the FDA might not approve our or our third-party manufacturer's processes or facilities; or
- the FDA may change its approval policies or adopt new regulations.

If any of our product candidates fails to demonstrate safety and efficacy in clinical studies or does not gain regulatory approval, our business and results of operations will be materially and adversely harmed.

Even if we receive regulatory approval for a product candidate, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and subject us to penalties if we fail to comply with applicable regulatory requirements.

Once regulatory approval has been granted, the approved product and its manufacturer are subject to continual review by the FDA and non-U.S. regulatory authorities. Any regulatory approval that we or our collaboration partners receive for our product candidates may be subject to limitations on the indicated uses for which the product may be marketed or contain requirements for potentially costly post-marketing follow-up studies to monitor the safety and efficacy of the product. In addition, if the FDA or non-U.S. regulatory authorities approve any of our product candidates, we will be subject to extensive and ongoing regulatory requirements by the FDA and other regulatory authorities with regard to the labeling, packaging, adverse event reporting, storage, advertising, promotion and recordkeeping for our products. In addition, manufacturers of our drug products are required to comply with cGMP regulations, which include requirements related to quality control and quality assurance as well as the corresponding maintenance of records and documentation. Further, regulatory authorities must approve these manufacturing facilities before they can be used to manufacture our drug products, and these facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. If we or a third party discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory authority may impose restrictions on that product, the manufacturer or us, including requiring withdrawal of the product from the market or suspension of manufacturing.

The regulatory requirements and policies may change and additional government regulations may be enacted for which we may also be required to comply. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or in other countries. If we are not able to maintain regulatory compliance, we may not be permitted to market our future products and our business may suffer.

Unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives could harm our business.

There is increasing pressure on biotechnology companies to reduce healthcare costs. In the U.S., these pressures come from a variety of sources, such as managed care groups, institutional, and government purchasers. Increased purchasing power of entities that negotiate on behalf of federal healthcare programs and private sector beneficiaries could increase pricing pressures in the future. Such pressures may also increase the risk of litigation or investigation by the government regarding pricing calculations. The biotechnology industry will likely face greater regulation and political and legal action in the future.

The regulations that govern marketing approvals, pricing and reimbursement for new therapeutic products vary widely from country to country. Some countries require approval of the sale price of a product 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 might obtain regulatory approval for a product in a particular country, but then be subject to price regulations that delay our commercial launch of the product and negatively impact the revenue we are able to generate from the sale of the product in that country.

Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain regulatory approval. Adverse pricing limitations prior to approval will also adversely affect us by reducing our commercial potential. Our ability to commercialize any products successfully also will depend in part on the extent to which reimbursement for these products and related treatments becomes available from government health administration authorities, private health insurers and other organizations. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels.

A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and these third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. Increasingly, third-party payors are requiring that companies provide them with predetermined discounts from list prices and are challenging the prices charged for medical products. We cannot be sure that coverage and reimbursement will be available for any product that we commercialize and, if reimbursement is available, what the level of reimbursement will be. Reimbursement may impact the demand for, or the price of, any product for which we obtain marketing approval. Obtaining reimbursement for our products may be particularly difficult because of the higher prices often associated with products administered under the supervision of a physician. If reimbursement is not available or is available only to limited levels, we may not be able to successfully commercialize any product candidate that we successfully develop.

There may be significant delays in obtaining reimbursement for approved products, and coverage may be more limited than the purposes for which the product is approved by the FDA or regulatory authorities in other countries. Moreover, eligibility for reimbursement does not imply that any product will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Payment rates may vary according to the use of the product and the clinical setting in which it is used, may be based on payments allowed for lower cost products that are already reimbursed and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of products from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government funded and private payors for new

products that we develop could have a material adverse effect on our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Failure to obtain regulatory approvals in foreign jurisdictions will prevent us from marketing our products internationally.

We may pursue commercialization of our future products in international markets, either through distribution and marketing partners or our own commercial organization. In order to market our future products in the European Economic Area, or EEA, and many other foreign jurisdictions, we must obtain separate regulatory approvals. Specifically, in the EEA, medicinal products can only be commercialized after obtaining a Marketing Authorization, or MA. Before granting the MA, the European Medicines Agency or the competent authorities of the member states of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.



We have had limited interactions with foreign regulatory authorities, and the approval procedures vary among countries and can involve additional clinical testing, and the time required to obtain approval may differ from that required to obtain FDA approval. Clinical studies conducted in one country may not be accepted by regulatory authorities in other countries. Approval by the FDA does not ensure approval by regulatory authorities in other countries, and approval by one or more foreign regulatory authorities does not ensure approval by regulatory authorities in other foreign countries or by the FDA. However, a failure or delay in obtaining regulatory approval in one country may have a negative effect on the regulatory process in others. The foreign regulatory approval process may include all of the risks associated with obtaining FDA approval. We may not obtain foreign regulatory approvals on a timely basis, if at all. We may not be able to submit for regulatory approvals and even if we submit we may not receive necessary approvals to commercialize our products in any market.

Healthcare reform measures could hinder or prevent our product candidates' commercial success.

In the United States, there have been and we expect there will continue to be a number of legislative and regulatory changes to the healthcare system in ways that could affect our future revenue and profitability and the future revenue and profitability of our potential customers. Federal and state lawmakers regularly propose and, at times, enact legislation that would result in significant changes to the healthcare system, some of which are intended to contain or reduce the costs of medical products and services. For example, one of the most significant healthcare reform measures in decades, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, collectively, the Affordable Care Act, was enacted in 2010. The Affordable Care Act contains a number of provisions, including those governing enrollment in federal healthcare programs, reimbursement changes and fraud and abuse measures, all of which will impact existing government healthcare programs and will result in the development of new programs. The Affordable Care Act, among other things:

- imposes a non-deductible annual fee on pharmaceutical manufacturers or importers who sell "branded prescription drugs," effective 2011;
- increases the minimum level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1%, effective 2011;
- could result in the imposition of injunctions;
- expanded Medicaid drug rebates to cover drugs paid by Medicaid managed care organizations;
- changes the Medicaid rebate rates for line extensions or new formulations of oral solid dosage form;
- expands the types of entities eligible for the "Section 340B discounts" for outpatient drugs;
- requires manufacturers to participate in a coverage gap discount program, under which they must agree to offer 50% point-of-sale discounts off negotiated prices of applicable branded 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; and
- creates a process for approval of biologic therapies that are similar or identical to approved biologics.

While the U.S. Supreme Court upheld the constitutionality of most elements of the Affordable Care Act in June 2012, other legal challenges are still pending final adjudication in several jurisdictions. In addition, Congress has in the past proposed and likely will continue to propose a number of legislative initiatives, including possible repeal of the Affordable Care Act. At this time, it remains unclear whether there will be any changes made to the Affordable Care Act, whether to certain provisions or its entirety. We cannot assure that the Affordable Care Act, as currently enacted or as amended in the future, will not adversely affect our business and financial results and we cannot predict how future federal or state legislative or administrative changes relating to healthcare reform will affect our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, the Budget Control Act of 2011, or Budget Control Act, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee did not achieve a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, which triggered the legislation's automatic reduction to several government programs, including aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in 2013. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which delayed for another two months the budget cuts mandated by the sequestration provisions of the Budget Control Act. The ATRA, among other things, also reduced Medicare payments to several providers, including hospitals, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. In March 2013, the President signed an executive order implementing sequestration, and in April 2013, the 2% Medicare reductions went into effect. In December 2013, Congress amended the Budget Control Act to provide greater discretionary spending in 2014 and 2015 than originally budgeted and provide relief from the FDA user fee for two years. This amendment also extended the prohibition against reducing payments to Medicare providers by more than 2% until 2023. In December 2014, Congress passed the Consolidated and Further Continuing Appropriations Act, 2015 and a tax extenders bill, both of which may negatively impact coverage and reimbursement of healthcare items and services.

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

- our ability to set a price we believe is fair for our products;
- our ability to generate revenue and achieve or maintain profitability; and
- the availability of capital.

Further, changes in regulatory requirements and guidance may occur and we may need to amend clinical study protocols to reflect these changes. Amendments may require us to resubmit our clinical study protocols to Institutional Review Boards for reexamination, which may impact the costs, timing or successful completion of a clinical study. In light of widely publicized events concerning the safety risk of certain drug products, regulatory authorities, members of Congress, the Governmental Accounting Office, medical professionals and the general public have raised concerns about potential drug safety issues. These events have resulted in the recall and withdrawal of drug products, revisions to drug labeling that further limit use of the drug products and establishment of risk management programs that may, for instance, restrict distribution of drug products or require safety surveillance and/or patient education. The increased attention to drug safety issues may result in a more cautious approach by the FDA to clinical studies and the drug approval process. Data from clinical studies may receive greater scrutiny with respect to safety, which may make the FDA or other regulatory authorities more likely to terminate or suspend clinical studies before completion, or require longer or additional clinical studies that may result in substantial additional expense and a delay or failure in obtaining approval or approval for a more limited indication than originally sought.

Given the serious public health risks of high profile adverse safety events with certain drug products, the FDA may require, as a condition of approval, costly risk evaluation and mitigation strategies, which may include safety surveillance, restricted distribution and use, patient education, enhanced labeling, special packaging or labeling, expedited reporting of certain adverse events, preapproval of promotional materials and restrictions on direct-to-consumer advertising.

If we fail to comply with healthcare regulations, we could face substantial penalties and our business, operations and financial condition could be adversely affected.

Pharmaceutical companies are heavily regulated by federal, state and local regulations in the countries in which business activities occur. Even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. We could be subject to laws and regulations governing healthcare fraud and abuse, advertising and other promotional activities, data privacy and patient rights by both the federal government and the states in which we conduct our business. The regulations that may affect our ability to operate include, without limitation:

- the federal Anti-Kickback Statute, which prohibits, among other things, any person from knowingly and willfully offering, soliciting, receiving or providing remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs, such as the Medicare and Medicaid programs;
- the federal Physician Payment Sunshine Act or Open Payments Program provisions and the implementing regulations which will require extensive tracking of physician and teaching hospital payments, maintenance of a payments database, and public reporting of the payment data;

52

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- the federal False Claims Act, which prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, false claims, or knowingly using false statements, to obtain payment from the federal government;
- federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the Foreign Corrupt Practices Act and similar statutes and regulations in foreign jurisdictions, which makes it unlawful for certain classes of persons and entities to make payments to foreign government officials to assist in obtaining or retaining business;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information; and
- state law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers.

The Affordable Care Act, among other things, amends the intent requirement of the Federal Anti-Kickback Statute and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the Affordable Care Act provides that the government may assert that a claim including items or services resulting from a violation of the Federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

If our operations are found to be in violation of any of the laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security and fraud laws may prove costly.

## RISKS RELATED TO OWNERSHIP OF OUR COMMON STOCK

Our stock price may be volatile, and investors in our common stock could incur substantial losses.

Our stock price has fluctuated in the past and may be volatile in the future. The stock market in general, and the market for biotechnology companies in particular, have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may experience losses on their investment in our stock. The market price for our common stock may be influenced by many factors, including the following:

- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;
- market conditions in the pharmaceutical and biotechnology sectors;
- actual or anticipated changes in earnings estimates or changes in stock market analyst recommendations regarding our common stock, other comparable companies or our industry generally;
- trading volume of our common stock;
- sales of our common stock by us or our stockholders;
- general economic, industry and market conditions; and
- the other risks described in this "Risk factors" section.

These broad market and industry factors may seriously harm the market price of our common stock, regardless of our operating performance. In the past, following periods of volatility in the market, securities class-action litigation has often been instituted against companies. Such litigation, if instituted against us, could result in substantial costs and

diversion of management's attention and resources, which could materially and adversely affect our business, financial condition, results of operations and growth prospects.

Our executive officers, directors and principal stockholders have the ability to significantly influence all matters submitted to stockholders for approval.

Based, in part, on a review of SEC filings, we believe that our executive officers, directors and stockholders who own more than 5% of our outstanding common stock beneficially own close to half of our outstanding shares of common stock, based on shares of common stock outstanding as of June 30, 2015. As a result, if these stockholders were to choose to act together, they would be able to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these stockholders, if they choose to act together, will significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire.

If securities or industry analysts do not publish research, or publish inaccurate or unfavorable research, about our business, our stock price and trading volume could decline.

The trading market for our common stock depends, in part, on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts may cease to publish research on our company at any time in their discretion. If one or more of these analysts cease coverage of our company or fail to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline. In addition, if one or more of the analysts who cover us downgrade our stock or publish inaccurate or unfavorable research about our business, our stock price would likely decline. If our operating results fail to meet the forecast of analysts, our stock price will likely decline.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Because our board of directors is responsible for appointing the members of our management team, these provisions could in turn affect any attempt by our stockholders to replace current members of our management team. Among others, these provisions include the following:

- our board of directors is divided into three classes with staggered three-year terms which may delay or prevent a change of our management or a change in control;
- our board of directors has the right to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death or removal of a director, which prevents stockholders from being able to fill vacancies on our board of directors;
- our stockholders may not act by written consent or call special stockholders' meetings; as a result, a holder, or holders, controlling a majority of our capital stock would not be able to take certain actions other than at annual stockholders' meetings or special stockholders' meetings called by the board of directors, the chairman of the board, the chief executive officer or the president;
- our certificate of incorporation prohibits cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;
- stockholders must provide advance notice and additional disclosures in order to nominate individuals for election to the board of directors or to propose matters that can be acted upon at a stockholders' meeting, which may discourage

or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of our company; and

- our board of directors may issue, without stockholder approval, shares of undesignated preferred stock; the ability to issue undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to acquire us.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Our employment agreements with our executive officers may require us to pay severance benefits to any of those persons who are terminated in connection with a change in control of us, which could harm our financial condition or results.

Certain of our executive officers are parties to employment agreements that contain change in control and severance provisions providing for aggregate cash payments of up to approximately \$2.5 million for severance and other benefits and acceleration of vesting of stock options with a value of approximately \$45.8 million as of June 30, 2015, based on the closing price of our common stock of \$45.55 on such date in the event of a termination of employment in connection with a change in control of us. The accelerated vesting of options could result in dilution to our existing stockholders and harm the market price of our common stock. The payment of these severance benefits could harm our financial condition and results. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be our stockholders' sole source of gain.

We have never declared or paid cash dividends on our common stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of existing or any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be our stockholders' sole source of gain for the foreseeable future.

## 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.

## ITEM 6. EXHIBITS



A list of exhibits filed with this Quarterly Report on Form 10-Q or incorporated herein by reference is found in the Index to Exhibits immediately following the signature page of this report and is incorporated into this Item 6 by reference.

SIGNATURES

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

PORTOLA  
PHARMACEUTICALS, INC.

August 4, 2015 By: /s/ William Lis  
William Lis  
Chief Executive Officer

August 4, 2015 By: /s/ Mardi C. Dier  
Mardi C. Dier  
Chief Financial Officer

## EXHIBIT INDEX

		Incorporation By Reference			
Exhibit					
Number	Exhibit Description	Form	SEC File No.	Exhibit	Filing Date
3.1	Amended and Restated Certificate of Incorporation of Portola Pharmaceuticals, Inc.	8-K	001-35935	3.1	5/28/2013
3.2	Amended and Restated Bylaws of Portola Pharmaceuticals, Inc.	8-K	001-35935	3.2	5/28/2013
4.1	Reference is made to Exhibits 3.1 through 3.2.				
4.2	Form of Common Stock Certificate of Portola Pharmaceuticals, Inc.	S-1	333-187901	4.1	5/17/2013
4.3	Third Amended and Restated Investor Rights Agreement, dated as of November 11, 2011, by and among Portola Pharmaceuticals, Inc., and certain of its stockholders.	S-1	333-187901	10.6	4/12/2013
4.4	Warrant to Purchase Shares of Series A Preferred Stock by and between the registrant and General Electric Capital Corporation, dated January 21, 2005.	10-Q	001-35935	4.4	11/06/2013
4.6	Warrant to Purchase Shares of Series B Preferred Stock by and between Portola Pharmaceuticals, Inc., and Comerica Incorporated, dated September 29, 2006.	10-Q	001-35935	4.6	11/06/2013
4.7	Warrant to Purchase Shares of Common Stock by and between the registrant and Laurence Shushan and Magdalena Shushan Acosta, Trustees, The Laurence and Magdalena Shushan Family Trust, Under Agreement Dated October 8, 1997, dated December 15, 2006.	10-Q	001-35935	4.7	11/06/2013
4.8	Warrant to Purchase Shares of Common Stock by and between Portola Pharmaceuticals, Inc., and HCP Life Science Assets TRS, LLC, dated December 15, 2006.	10-Q	001-35935	4.8	11/06/2013
4.9	Warrant to Purchase Shares of Common Stock by and between Portola Pharmaceuticals, Inc., and Bristow Investments, L.P., dated December 15, 2006.	10-Q	001-35935	4.9	11/06/2013
10.27+*	Offer Letter by and between Portola Pharmaceuticals, Inc. and Tao Fu, dated as of May 8, 2015				

31.1\* Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended.

31.2\* Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended.

32.1\* Certification of Principal Executive Officer and Principal Financial Officer pursuant to Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.<sup>(1)</sup>

101.INS\* XBRL Instance Document

101.SCH\* XBRL Taxonomy Extension Schema Document

101.CAL\* XBRL Taxonomy Extension Calculation Linkbase Document

101.DEF\* XBRL Taxonomy Extension Definition Linkbase Document

101.LAB\* XBRL Taxonomy Extension Label Linkbase Document

101.PRE\* XBRL Taxonomy Extension Presentation Linkbase Document

+Management contract or compensatory plan

\*Filed herewith

(1) This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.