

PORTOLA PHARMACEUTICALS INC

Form 10-Q

November 06, 2013

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 September 30, 2013

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)

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Delaware  
(State or Other Jurisdiction of Incorporation or Organization)

20-0216859  
(I.R.S. Employer Identification No.)

270 E. Grand Avenue  
South San Francisco, California  
(Address of Principal Executive Offices)  
(650) 246-7000

94080  
(Zip Code)

(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 October 31, 2013, the number of outstanding shares of the registrant's common stock, par value \$0.001 per share, was 39,709,658.

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## PART I FINANCIAL INFORMATION

## ITEM 1. FINANCIAL STATEMENTS

## PORTOLA PHARMACEUTICALS, INC.

## Condensed Balance Sheets

(In thousands, except share and per share data)

	September 30, 2013 (Unaudited)	December 31, 2012 (Note 2)
<b>ASSETS</b>		
<b>CURRENT ASSETS:</b>		
Cash and cash equivalents	\$ 58,710	\$ 53,613
Short-term investments	116,066	77,656
Receivables from collaborations	227	662
Prepaid expenses and other current assets	3,669	2,982
Total current assets	178,672	134,913
Property and equipment, net	2,428	2,861
Long-term investments	44,151	6,115
Other assets	116	2,112
<b>TOTAL ASSETS</b>	<b>\$ 225,367</b>	<b>\$ 146,001</b>
<b>LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' EQUITY (DEFICIT)</b>		
<b>CURRENT LIABILITIES:</b>		
Accounts payable	\$ 1,517	\$ 4,840
Accrued compensation and employee benefits	1,863	1,860
Accrued and other liabilities	15,960	7,399
Deferred revenue - current portion	3,930	4,042
Convertible preferred stock warrant liability		683
Total current liabilities	23,270	18,824
Deferred revenue - noncurrent portion	3,337	
Other long-term liabilities	811	1,466
<b>TOTAL LIABILITIES</b>	<b>27,418</b>	<b>20,290</b>
<b>Commitments and contingencies</b>		
Convertible preferred stock, \$0.001 par value. 0 and 243,258,300 shares authorized at September 30, 2013 and December 31, 2012; 0 shares and 24,026,797 shares issued and outstanding at September 30, 2013 and December 31, 2012		317,280
<b>STOCKHOLDERS' EQUITY (DEFICIT):</b>		
Preferred stock, \$0.001 par value. 5,000,000 shares authorized at September 30, 2013; 0 issued and outstanding at September 30, 2013; 0 authorized, issued or outstanding at December 31, 2012		

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Common stock, \$0.001 par value. 100,000,000 and 300,000,000 shares authorized; 35,229,352 and 1,385,508 shares issued and outstanding at September 30, 2013 and December 31, 2012

	35	1
Additional paid-in capital	458,460	10,717
Accumulated deficit	(260,610)	(202,320)
Accumulated other comprehensive income (loss)	64	33
Total stockholders' equity (deficit)	197,949	(191,569)
<b>TOTAL LIABILITIES, CONVERTIBLE PREFERRED STOCK, AND STOCKHOLDERS' EQUITY</b>	<b>\$ 225,367</b>	<b>\$ 146,001</b>

See accompanying notes.

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## PORTOLA PHARMACEUTICALS, INC.

## Condensed Statements of Operations

(Unaudited)

(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Collaboration and license revenue	\$ 2,766	\$ 738	\$ 8,474	\$ 70,084
Operating expenses:				
Research and development	18,088	9,954	56,642	36,004
General and administrative	3,907	2,879	10,654	8,744
Total operating expenses	21,995	12,833	67,296	44,748
Income (loss) from operations	(19,229)	(12,095)	(58,822)	25,336
Interest and other income (expense), net	679	607	532	(188)
Net income (loss)	\$ (18,550)	\$ (11,488)	\$ (58,290)	\$ 25,148
Net income (loss) attributable to common stockholders:				
Basic	\$ (18,550)	\$ (11,488)	\$ (58,290)	\$ 322
Diluted	\$ (18,550)	\$ (11,488)	\$ (58,290)	\$ 462
Shares used to compute net income (loss) per share attributable to common stockholders:				
Basic	35,200,761	1,370,190	17,218,475	1,342,919
Diluted	35,200,761	1,370,190	17,218,475	1,976,618
Net income (loss) per share attributable to common stockholders:				
Basic	\$ (0.53)	\$ (8.38)	\$ (3.39)	\$ 0.24
Diluted	\$ (0.53)	\$ (8.38)	\$ (3.39)	\$ 0.23



See accompanying notes.

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PORTOLA PHARMACEUTICALS, INC.

Condensed Statements of Comprehensive Income (Loss)

(Unaudited)

(In thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Net income (loss)	\$ (18,550)	\$ (11,488)	\$ (58,290)	\$ 25,148
Other comprehensive income:				
Unrealized gain on available-for-sale securities, net of tax	100	10	31	56
Total comprehensive income (loss)	\$ (18,450)	\$ (11,478)	\$ (58,259)	\$ 25,204

See accompanying notes.

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## PORTOLA PHARMACEUTICALS, INC.

## Condensed Statements of Cash Flows

(Unaudited)

(In thousands)

	Nine Months Ended September 30	
	2013	2012
<b>Operating activities</b>		
Net income (loss)	\$ (58,290)	\$ 25,148
Adjustments to reconcile net income (loss) to cash used in operating activities:		
Depreciation and amortization	1,015	1,053
Amortization of premium on investment securities	1,523	1,018
Stock-based compensation expense	3,457	2,017
Revaluation of convertible preferred stock warrant liability	(24)	(144)
Unrealized (gain) loss on foreign currency forward contracts	(97)	450
Changes in operating assets and liabilities:		
Receivables from collaborations	435	(2,347)
Prepaid expenses and other current assets	(540)	(2,155)
Other assets	361	(368)
Accounts payable	(3,323)	711
Accrued compensation and employee benefits	3	(918)
Accrued and other liabilities	10,004	1,299
Deferred revenue	3,225	(69,468)
Other long-term liabilities	(655)	(620)
Net cash used in operating activities	(42,906)	(44,324)
<b>Investing activities</b>		
Purchases of property and equipment	(582)	(362)
Purchases of investments	(152,793)	(133,073)
Proceeds from sales of investments	6,644	42,767
Proceeds from maturities of investments	68,211	18,637
Net cash used in investing activities	(78,520)	(72,031)
<b>Financing activities</b>		
Proceeds from initial public offering, net of underwriters discount	131,026	
Payment of initial public offering costs	(5,025)	
Proceeds from issuance of common stock, including early exercise of stock options	522	292
Net cash provided by financing activities	126,523	292
Net increase (decrease) in cash and cash equivalents	5,097	(116,063)
Cash and cash equivalents at beginning of period	53,613	170,323

Cash and cash equivalents at end of period	\$ 58,710	\$ 54,350
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See accompanying notes.

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PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed 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 disorders 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 two lead programs address the area of thrombosis, or blood clots. Our lead compound Betrixaban is a novel oral once-daily inhibitor of Factor Xa in Phase 3 development for extended duration prophylaxis, or preventive treatment, of a form of thrombosis known as venous thromboembolism, in acute medically ill patients. Our second lead development candidate (pINN) Andexanet alfa, formerly PRT4445, is a recombinant protein designed to reverse the anticoagulant activity in patients treated with a Factor Xa inhibitor who suffer an uncontrolled bleeding episode or undergo emergency surgery. Our third product candidate, PRT2070, is an orally available kinase inhibitor being developed for hematologic, or blood, cancers and inflammatory disorders. Our fourth program, PRT2607 and other selective Syk inhibitors, is being developed in partnership with Biogen Idec Inc.

### Initial Public Offering

In May 2013, we closed our initial public offering ( IPO ) of 9,686,171 shares of our common stock, which included 1,263,413 shares of common stock issued pursuant to the over-allotment option granted to our underwriters. The public offering price of the shares sold in the offering was \$14.50 per share. The total proceeds from the offering to us, net of underwriting discounts and commissions of approximately \$9.4 million, were approximately \$131.0 million. After deducting offering expenses payable by us of approximately \$5.2 million, net proceeds to us were \$125.8 million. Upon the closing of the IPO, all shares of convertible preferred stock then outstanding converted into 24,026,797 shares of common stock. In addition, all of our convertible preferred stock warrants were converted into warrants to purchase common stock.

## 2. Summary of Significant Accounting Policies

### Basis of Presentation

The accompanying unaudited condensed financial statements have been prepared in accordance with U.S. generally accepted accounting principles ( U.S. GAAP ), and following 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 financial statements have been prepared on the same basis as the Company's annual 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 the Company's financial information. The results of operations for the nine months ended September 30, 2013 are not necessarily indicative of the results to be expected for the year ending December 31, 2013. The condensed balance sheet as of December 31, 2012 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 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, 2012 included in the Company's Prospectus filed pursuant to Rule 424(b)(4) as filed on May 21, 2013 with the SEC.

#### Reverse Stock Split

On May 17, 2013, the Company effected a 1-for-10 reverse split of our preferred stock and common stock. Upon the effectiveness of the reverse stock split every 10 shares of outstanding preferred stock and common stock was decreased to one share of preferred stock or common stock, as applicable, the number of shares of common stock into which each outstanding option to purchase common stock is exercisable was proportionately decreased on a 1-for-10 basis, and the exercise price of each outstanding option to purchase common stock was proportionately increased on a 1-for-10 basis. All the shares numbers, share prices and exercise prices have been adjusted within the condensed financial statements, on a retroactive basis, to reflect the 1-for-10 reverse stock split.

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

## Use of Estimates

The preparation of condensed 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 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.

## 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 were reported as a component of accumulated comprehensive income. 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 whose collaborative research and development revenue accounted for 10% or more of total revenues were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Novartis AG		100%		100%
Bristol-Myers Squibb Company and Pfizer Inc.	28%		46%	
Bayer Pharma, AG and Janssen Pharmaceuticals, Inc.	37%		41%	
Daiichi Sankyo, Inc.	32%		12%	



#### Deferred Offering Costs

Deferred offering costs, which primarily consist of direct incremental legal, accounting and printing fees incurred in the preparation of the IPO, were capitalized. The deferred offering costs were offset against IPO proceeds upon completion of the offering in May 2013. As of December 31, 2012, \$1.6 million of deferred offering costs were capitalized in other assets on the balance sheets. There were no remaining amounts deferred at September 30, 2013 pertaining to the IPO.

#### 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 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 materials and obligations to participate on certain development and/or commercialization committees with the collaborators.

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. Milestones are 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.

Amounts from sales of licenses are recognized as revenue, as licensing of intellectual property is one of our principal or major ongoing activities. Amounts received as funding of research and development activities are recognized as revenue if the collaboration arrangement involves the sale of our research or development 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 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, 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 currencies and our foreign currency forward contracts are marked to market at the end of each period and recorded as gains and losses in the condensed statements of operations.

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 Income (Loss) per Share Attributable to Common Stockholders

Basic and diluted net income (loss) per share attributable to common stockholders is calculated in conformity with the two-class method required for companies with participating securities. Under the two-class method, in periods when we have net income, basic net income attributable to common stockholders is determined by allocating undistributed earnings, calculated as net income less current period convertible preferred stock noncumulative dividends, between the common stock and the convertible preferred stock. In computing diluted net income attributable to common stockholders, undistributed earnings are re-allocated to reflect the potential impact of dilutive securities. 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. The diluted net income per share attributable to common stockholders is computed by giving effect to all potential dilutive common stock equivalents outstanding for the period. In periods when we have incurred a net loss, convertible preferred stock, options and warrants to purchase common stock and convertible preferred stock warrants are considered common stock equivalents but have been excluded from the calculation of diluted net loss per share attributable to common stockholders as their effect is antidilutive.

### 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 and accounts payable, approximate their fair value due to their short maturities. 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.

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

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 was measured on a recurring basis (in thousands):

	September 30, 2013			
	Level 1	Level 2	Level 3	Total
<b>Financial Assets:</b>				
Money market funds	\$ 54,930	\$	\$	\$ 54,930
Corporate notes and commercial paper		103,065		103,065
U.S. government agency securities		57,150		57,150
Foreign currency forward contracts		266		266
Total financial assets	\$ 54,930	\$ 160,481	\$	\$ 215,411
	December 31, 2012			
	Level 1	Level 2	Level 3	Total
<b>Financial Assets:</b>				
Money market funds	\$ 43,303	\$	\$	\$ 43,303
Corporate notes and commercial paper		64,425		64,425
U.S. government agency securities		19,346		19,346
Foreign currency forward contracts		51		51
Total financial assets	\$ 43,303	\$ 83,822	\$	\$ 127,125
<b>Financial Liabilities:</b>				
Convertible preferred stock warrant liability	\$	\$	\$ 683	\$ 683

We have elected to use the income approach to value the derivatives (foreign currency forward contracts), using observable Level 2 market expectations at the measurement date and standard valuation techniques to convert future amounts to a single present amount assuming that participants are motivated, but not compelled to transact. Level 2 inputs for the valuations are limited to quoted prices for similar assets or liabilities in active markets and inputs other than quoted prices that are observable for the asset or liability (specifically foreign currency spot and forward rates, and credit risk at commonly quoted intervals). Mid-market pricing is used as a practical expedient for fair value measurements. The fair value measurement of any asset or liability must reflect the non-performance risk of the entity and the counterparty to the transaction. Therefore, the impact of the counterparty's creditworthiness, when in an asset position, and our creditworthiness, when in a liability position, has also been factored into the fair value measurement of the derivative instruments and did not have a material impact on the fair value of these derivative instruments. Both we and the counterparty are expected to continue to perform under the contractual terms of the instruments. There were no transfers between Level 1 and Level 2 during the periods presented.

Our convertible preferred stock warrant liability was classified as a Level 3 liability. The fair values of the outstanding convertible preferred stock warrants were measured using the Black-Scholes option-pricing model. Inputs used to determine estimated fair value included the estimated fair value of the underlying convertible preferred stock at the valuation measurement date, the remaining contractual term of the warrants, risk-free interest rates, expected dividends and estimated volatility. The significant unobservable input used in the fair value measurement of the convertible preferred stock warrant liability was the fair value of the underlying convertible preferred stock at the valuation remeasurement date. Generally, increases (decreases) in the fair value of the underlying convertible preferred stock would result in a directionally similar impact to the fair value measurement. The preferred stock warrants were converted to common stock warrants upon the completion of the IPO and are no longer subject to remeasurement.

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

The following table sets forth a summary of the changes in the estimated fair value of our convertible preferred stock warrants, which were measured at fair value on a recurring basis until their conversion to common stock warrants and related reclassification to additional paid-in capital (in thousands):

Balance as of December 31, 2012	\$ 683
Recognized gain upon final remeasurement	(24)
Reclassification of warrant liability to additional paid-in capital	(659)
Balance as of September 30, 2013	\$

The recognized gain was included in interest and other income (expense), net.

The estimated fair value of the convertible preferred stock warrants was determined as of May 22, 2013, the date remeasurement was no longer applicable, and December 31, 2012 using the Black-Scholes option-pricing model using the following assumptions:

	May 22, 2013		December 31, 2012	
Risk free interest rate	0.11	0.91%	0.26	0.57%
Estimated term equal to the remaining contractual term	1.7	3.8 years	2.1	4.2 years
Volatility		79%		82%
Dividend yield				

## 4. Financial Instruments

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

	September 30, 2013		December 31, 2012
Cost		Cost	

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		Unrealized Gains	Unrealized Losses	Estimated Fair Value		Unrealized Gains	Unrealized Losses	Estimated Fair Value
Money market funds	\$ 54,929	\$	\$	\$ 54,929	\$ 43,303	\$	\$	\$ 43,303
Corporate notes and commercial paper	103,034	40	(7)	103,067	64,403	25	(3)	64,425
U.S. government agency securities	57,119	34	(3)	57,150	19,335	11		19,346
	\$ 215,082	\$ 74	\$ (10)	\$ 215,146	\$ 127,041	\$ 36	\$ (3)	\$ 127,074

Classified as:

Cash equivalents		\$ 54,929		\$ 43,303
Short-term investments		116,066		77,656
Long-term investments		44,151		6,115
Total cash equivalents and investments		\$ 215,146		\$ 127,074

At September 30, 2013 and December 31, 2012, 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. Derivative Instruments

We are exposed to foreign currency exchange rates related to our business operations. To reduce our risks related to these exposures, we utilize certain derivative instruments, namely foreign currency forward contracts. We do not use derivatives for speculative trading purposes.

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

We enter into foreign currency forward contracts, none of which are designated as hedging transactions for accounting purposes, to reduce our exposure to foreign currency fluctuations of certain liabilities denominated in foreign currencies. These exposures are hedged on a quarterly basis. As of September 30, 2013 and December 31, 2012, we had foreign currency forward contracts with notional amounts of 9.5 million (\$12.9 million based on the exchange rate as of September 30, 2013) and 16.8million (\$22.2 million based on the exchange rate as of December 31, 2012), respectively, that were not designated as hedges. As of September 30, 2013, we recorded a derivative asset within prepaid expenses and other current assets and other long term assets of \$239,000 and \$27,000, respectively, related to these foreign currency forward contracts. As of December 31, 2012, we recorded a derivative asset within prepaid expenses and other current assets and other long-term assets of \$30,000 and \$21,000, respectively, related to these foreign currency forward contracts.

We recorded an unrealized gain of \$450,000 and an unrealized gain of \$97,000 in interest and other income (expense), net on our condensed statements of operations related to these foreign currency forward contracts for the three and nine months ended September 30, 2013, respectively. During the three and nine months ended September 30, 2013, the Company settled foreign currency forward contracts and recognized a realized gain of \$45,000 and \$118,000, respectively, in interest and other income (expense), net. During the three and nine months ended September 30, 2012 we recognized an unrealized gain of \$243,000 and an unrealized loss of \$450,000, respectively, and no realized gains or losses.

Our derivative financial instruments present certain market and counterparty risks. In general, the market risk related to these contracts is offset by corresponding gains and losses on the hedged transactions. The credit risk associated with these contracts is driven by changes in interest and currency exchange rates and, as a result, varies over time.

## 6. Balance Sheet Components

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

	September 30, 2013	December 31, 2012
Research and development related \$	14,407	\$ 4,217
Legal and accounting fees	323	507
Deferred rent	866	831
Accrued offering costs	66	1,506
Other	298	338
Total accrued and other liabilities \$	15,960	\$ 7,399



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## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

## 7. 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 September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
<b>Novartis:</b>				
Recognition of upfront license fee	\$	\$ 738	\$	\$ 53,846
Reimbursement of research and development expense				16,238
Novartis total		738		70,084
<b>BMS and Pfizer:</b>				
Recognition of research and development services	772		3,865	
BMS and Pfizer total	772		3,865	
<b>Bayer and Janssen:</b>				
Recognition of research and development services	1,028		3,466	
Bayer and Janssen total	1,028		3,466	
<b>Lee s:</b>				
Recognition of research and development services	78		130	
Lee s total	78		130	
<b>Daiichi Sankyo:</b>				
Recognition of research and development services	888		1,013	
Daiichi Sankyo total	888		1,013	
Total collaboration and license revenue	\$ 2,766	\$ 738	\$ 8,474	\$ 70,084
Novartis AG ( Novartis )				

In February 2009, we entered into a license agreement with Novartis to develop and commercialize Elinogrel. We estimated the term of our obligation to participate in the Joint Steering Committee and Joint Development Committee (collectively, the Committees ) to extend through December 31, 2018. In April 2012, we and Novartis agreed to a plan to return all rights to Elinogrel to Portola and to terminate the exclusive worldwide license agreement effective July 1, 2012. In connection with this plan, the expected term of our obligation to participate in the Committees changed from December 31, 2018 to July 1, 2012. The change in term of the obligation to participate in the Committees was accounted for as a change in accounting estimate on a prospective basis effective April 1, 2012. All remaining deferred revenue was recognized as revenue through July 2012, as no further performance obligations remained upon termination. As of the time of termination, no milestones had been achieved and no royalties had been triggered under our agreement with Novartis.

Biogen Idec, Inc. ( Biogen Idec )

In October 2011, we entered into an exclusive, worldwide license and collaboration agreement with Biogen Idec, under which Portola and Biogen Idec were to jointly develop and commercialize selective, novel oral Syk inhibitors for the treatment of autoimmune and inflammatory diseases.

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PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

In November 2012, we elected to exercise our option under our agreement with Biogen Idec to convert the agreement to a fully out-licensed agreement. After such election, we relinquished our right to share profits from sales of products related to PRT2607 and other selective Syk inhibitors, but are entitled to receive royalties from sales of these products by Biogen Idec. We no longer have the responsibility to fund the program under the agreement. The out-licensed agreement now provides for future payments to us of up to approximately \$370.0 million based on the occurrence of certain development and regulatory events. As all contingent consideration payments are based solely on the performance of Biogen Idec, the milestone method of accounting will not be applied to such amounts. Biogen Idec has elected to assume all future development work for Syk inhibitors, including the major indications, such as allergic asthma. This agreement will continue in force until either party terminates the agreement pursuant to the agreement or until the expiration of Biogen Idec's royalty obligations pursuant to the agreement. Biogen Idec may terminate the agreement without cause upon 120 days' notice. In such event, we would regain all development rights and Biogen Idec would have no further payment obligations pursuant to the agreement.

During the three and nine months ended September 30, 2013 and 2012, we recorded a reduction in research and development expense of \$227,000 and \$721,000, and \$1.0 million and \$5.9 million, respectively, owed by Biogen Idec to us under the cost-sharing terms of the agreement.

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

In October 2012, we entered into a three-way agreement with BMS and Pfizer to include subjects dosed with apixaban, their jointly owned product candidate, in one of our Phase 2 proof-of-concept studies of Andexanet alfa. We are responsible for the cost of conducting this clinical study. BMS and Pfizer will work closely with us on both development and regulatory aspects of Andexanet alfa in connection with our Phase 2 proof-of-concept studies to the extent such matters relate to apixaban. Pursuant to our agreement with BMS and Pfizer we are obligated to provide research and development services and participate on various committees. We originally estimated the period of performance of our obligations to extend through June 2013. In March 2013, we revised our estimated period of performance to be through July 2013. In June 2013, we revised our estimated period of performance to be through September 2013. In September 2013, we revised our estimated period of performance to be through October 2013. The effects of these changes in estimates were not significant.

The total consideration under this agreement of \$6.0 million is being recognized as revenue on a straight-line basis over the estimated performance period through October 2013.

During the three and nine months ended September 30, 2013, we recognized \$772,000 and \$3.9 million in collaboration revenue, respectively. The deferred revenue balance as of September 30 2013 was \$176,000.

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 the 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 the 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 ratably 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 nine months ended September 30, 2013, we recognized \$78,000 and \$130,000, respectively, of collaboration revenue. The deferred revenue balance as of September 30, 2013 was \$570,000.

PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

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 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. 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 one-half 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. We have not incurred any such costs during the three and nine months ended September 30, 2013 related to this agreement.

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 Joint Collaboration Committee ( 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 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. We originally estimated the period of performance to be through November 2013. In June 2013, we revised our estimated period of performance to be through January 2014. In September 2013, we revised our estimated period of performance to be through March 2014. The effect of this change was not significant. The total consideration under this agreement is being recognized as revenue ratably over the estimated performance period through March 2014.

During the three and nine months ended September 30, 2013, we recognized \$1.0 million and \$3.5 million in collaboration revenue, respectively. The deferred revenue balance as of September 30, 2013 was \$1.5 million.

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 will provide us with an upfront fee of \$6.0 million, \$3.0 million of which is subject to refund should Daiichi Sankyo decide 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 through May 2014. The total non-contingent consideration under this agreement of \$3.0 million is being recognized as

revenue ratably over the estimated non-contingent performance period through May 2014. The contingent consideration under this agreement of \$3.0 million will be recognized after the contingency is resolved over the remaining performance period, which is currently estimated to begin in May 2014 and conclude in October 2014.

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Financial Statements

(continued)

During the three and nine months ended September 30, 2013, we recognized \$888,000 and \$1.0 million, respectively, in collaboration revenue associated with the non-contingent element of the arrangement. The contingent element of the arrangement of \$3.0 million and the unearned portion of the non-contingent element of the arrangement of \$2.0 million was recorded as deferred revenue as of September 30, 2013.

## 8. Restructuring Charge

In November 2012, as part of our strategy to better align our capital resources with our clinical development plan, we reduced our workforce by 23 employees, 16 of whom were immediately terminated, five of whom were terminated on January 31, 2013, two of whom were terminated on April 30, 2013. The final restructuring charge of \$698,000 includes severance and related costs associated with the termination of the employees. For the nine months ended September 30, 2013, we recorded a net restructuring charge of \$79,000, of which \$66,000 is included within research and development expense and \$13,000 is included within general and administrative expense on our condensed statements of operations. During the nine months ended September 30, 2013, we paid \$223,000 of severance costs. At December 31, 2012, the accrued restructuring liability, which is included within accrued and other liabilities on the balance sheet was \$143,000. There were no remaining amounts accrued for the restructuring liability at September 30, 2013.

## 9. 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 IPO in May 2013. As of September 30, 2013 there are 4,903,323 shares reserved under the 2013 Plan for the issuance of options and restricted stock.

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 Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Risk-free interest rate	1.8%	1.2%	1.3%	1.2%
Expected life	6.0 years	6.0 years	6.0 years	6.0 years
Volatility	80%	70%	79%	70%
Dividend yield	0%	0%	0%	0%



The following table summarizes option activity under our 2003 Equity Incentive Plan and our 2013 Equity Incentive Plan and related information during the nine months ended September 30, 2013:

	Shares Available for Grant	Shares Subject to Outstanding Options	Weighted-Average Exercise Price Per Share
Balance at December 31, 2012	593,011	3,451,178	\$ 6.35
Options authorized	150,000		
Options granted	(785,241)	785,241	17.29
Options exercised		(129,787)	22.67
Options cancelled	326,274	(326,274)	7.81
Balance at September 30, 2013	284,044	3,780,358	\$ 7.94

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## 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 September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Research and development	\$ 787	\$ 353	\$ 1,732	\$ 1,102
General and administrative	747	303	1,725	965
Total stock-based compensation	\$ 1,534	\$ 656	\$ 3,457	\$ 2,067

## 10. Net Income (Loss) per Share Attributable to Common Stockholders

The following outstanding shares of common stock equivalents were excluded from the computation of diluted net income (loss) per share attributable to common stockholders for the periods presented because including them would have been antidilutive:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Convertible preferred stock		24,026,797		24,026,797
Common stock subject to repurchase	750		750	
Stock options to purchase common stock	3,780,358	1,678,800	3,780,358	1,605,305
Preferred stock warrants		81,075		81,075
Common stock warrants	82,575	1,500	82,575	1,500

The following table sets forth the computation of our unaudited basic and diluted net income (loss) per share attributable to common stockholders (in thousands, except share and per share data):

Three Months Ended September 30,		Nine Months Ended September 30,	
2013	2012	2013	2012

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Net income (loss)	\$	(18,550)	\$	(11,488)	\$	(58,290)	\$	25,148
Noncumulative dividends on convertible preferred stock								(19,065)
Undistributed earnings allocated to participating securities								(5,761)
Net income (loss) attributable to common stockholders, basic		(18,550)		(11,488)		(58,290)		322
Adjustment to undistributed earnings allocated to participating securities								140
Net income (loss) attributable to common stockholders, diluted	\$	(18,550)	\$	(11,488)	\$	(58,290)	\$	462
Basic shares:								
Weighted average common shares outstanding		35,200,761		1,370,190		17,218,475		1,342,919
Diluted shares:								
Weighted average common shares outstanding		35,200,761		1,370,190		17,218,475		1,342,919
Weighted average effect of dilutive stock options								633,699
		35,200,761		1,370,190		17,218,475		1,976,618
Net income (loss) per share attributable to common stockholders:								
Basic	\$	(0.53)	\$	(8.38)	\$	(3.39)	\$	0.24
Diluted	\$	(0.53)	\$	(8.38)	\$	(3.39)	\$	0.23

PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Financial Statements

(continued)

11. Income Taxes

For the nine months ended September 30, 2012, we did not record an income tax provision on pre-tax income because we incurred a current taxable loss for federal income tax purposes and had available tax credits to offset all state income tax. Tax credits were used in lieu of net operating losses because in 2012 California state law suspended their use. We maintain deferred tax assets that reflect the net tax effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. These deferred tax assets include net operating loss carryforwards, research credits and capitalized research and development. Realization of deferred tax assets is dependent upon future earnings, if any, the timing and amount of which are uncertain based on our history of losses. Accordingly, our deferred tax assets have been fully offset by a valuation allowance. Utilization of operating losses and credits may be subject to substantial annual limitation due to ownership change provisions of the Internal Revenue Code of 1986, as amended and similar state provisions. The annual limitation may result in the expiration of net operating losses and credits before utilization.

12. Subsequent Events

In October 2013, we completed a follow-on offering of 6,366,513 shares of our common stock, which included 1,908,803 shares of common stock sold by certain existing stockholders, at a public offering price of \$23.75 per share. In addition, the underwriters of the offering have been granted a 30-day option to purchase up to an additional 954,976 shares from us at the public offering price. This option to purchase additional shares is not yet exercised. The total proceeds from the offering to us, net of underwriting discounts and commissions of approximately \$6.4 million and excluding the underwriters' option, were approximately \$99.5 million. After deducting estimated offering expenses payable by us of approximately \$800,000, net proceeds to us were \$98.7 million.

## 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 together with the section of this report entitled "Selected financial data" and our financial statements and related notes included elsewhere in this report. This discussion and other parts of this report contain forward-looking statements that involve risk and uncertainties, such as statements of our plans, objectives, expectations and intentions. Our actual results could differ materially from those discussed in these forward-looking statements. Factors that could cause or contribute to such differences include, but are not limited to, those discussed in the section of this report entitled "Risk factors."

### 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. Since our inception in 2003, we have advanced several innovative compounds into clinical development. Our lead product candidate Betrixaban is in a pivotal Phase 3 clinical study, and our second lead development candidate (pINN) Andexanet alfa, formerly PRT4445, has completed the first of a series of Phase 2 proof-of-concept studies. We initiated a Phase 1/2 proof-of-concept study of PRT2070, one of our other product candidates, in October 2013. We also have completed multiple Phase 1 studies for PRT2607.

#### Our product candidates and collaboration agreements

##### Betrixaban

Betrixaban is a novel oral once-daily inhibitor of Factor Xa in development for extended duration prophylaxis, or preventive treatment, of a form of thrombosis, or blood clots, known as venous thromboembolism, or VTE, in acute medically ill patients for up to 35 days. In March 2012, we initiated a pivotal Phase 3 study to evaluate oral once-daily Betrixaban for superiority as compared to subcutaneous injection of enoxaparin for extended VTE prophylaxis in acute medically ill patients with restricted mobility and other risk factors. This study is anticipated to enroll approximately 6,850 patients. Based on current enrollment, we expect our current Phase 3 study of Betrixaban, or APEX, to be completed in mid-2015.

We entered into an asset purchase agreement with Millennium Pharmaceuticals, Inc., or Millennium, in November 2003 to acquire patent rights and intellectual property to a platelet research program, and a license agreement with Millennium in August 2004, to obtain certain exclusive rights to research, develop and commercialize certain compounds that inhibit Factor Xa, including Betrixaban. Both of these agreements were amended in December 2005.

In July 2009, we entered into an exclusive worldwide license and collaboration agreement with Merck & Co., Inc., or Merck, to develop and commercialize Betrixaban, which was terminated effective September 2011.

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 is a recombinant protein designed to reverse the anticoagulant activity in patients treated with a Factor Xa inhibitor who suffer an uncontrolled bleeding episode or undergo emergency surgery. In May 2013, we completed the first of a series of Phase 2 proof-of-concept studies of Andexanet alfa in healthy volunteers who were

administered a Factor Xa inhibitor, in this case Eliquis® (apixaban) manufactured by Bristol-Myers Squibb and Pfizer. Andexanet alfa is the first therapy to demonstrate reversal of a Factor Xa inhibitor in a clinical study. Based on the results of our initial Phase 2 study, we participated in an End of Phase 2 meeting with the FDA in August 2013 to discuss the remaining clinical studies needed for approval of Andexanet alfa. Based on discussions with the FDA, we believe that the FDA supports our pursuit of an accelerated approval process.

In October 2012, we entered into a three-way agreement with Bristol-Myers Squibb Company, or BMS, and Pfizer Inc., or Pfizer, to include subjects dosed with apixaban, their jointly owned Factor Xa inhibitor product, in one of our proof-of-concept studies of Andexanet alfa. 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 and development services and participate on various committees. We originally estimated the period of performance of our obligations to extend through June 2013. In March 2013, we revised our estimated period of performance to be through July 2013, in June 2013 we revised our estimated period of performance to be through September 2013 and in September 2013 we revised our estimated period of performance to be through October 2013. The total consideration under this agreement of \$6.0 million is being recognized as revenue on a straight-line basis over the estimated performance period through October 2013.

In February 2013, we entered into a three-way agreement with Bayer Pharma AG, or Bayer, and Janssen Pharmaceuticals, Inc., or Janssen, to include subjects dosed with rivaroxaban, their jointly owned Factor Xa inhibitor product, in one of our 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 Joint Collaboration Committee, or JCC, with Bayer and Janssen to oversee the collaboration activities under the agreement. We originally estimated the period of performance of our obligations to extend through November 2013. In June 2013, we revised our estimated period of performance to be through January 2014. In September 2013, we revised our estimated period of performance to be through March 2014. The total consideration under this agreement of \$5.5 million is being recognized as revenue ratably over the estimated performance period through March 2014.

In June 2013, we entered into an agreement with Daiichi Sankyo, Inc., or Daiichi Sankyo, to include subjects dosed with edoxaban, Daiichi Sankyo's Factor Xa inhibitor product, in one of our 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. Daiichi Sankyo may terminate the agreement at any time. Should Daiichi Sankyo terminate the agreement prior to the first patient dosing in the clinical trial, it is entitled to a refund of \$3.0 million. The total consideration under this agreement of \$6.0 million was received in July 2013, although only the non-contingent consideration of \$3.0 million was recorded as receivables from collaborations at September 30, 2013. We are obligated to perform preclinical proof-of-concept studies and participate on a JCC with Daiichi Sankyo to oversee the collaboration activities under the agreement. The total non-contingent consideration under this agreement of \$3.0 million is being recognized as revenue ratably over the estimated performance period through May 2014. The contingent consideration under this agreement of \$3.0 million will be recognized after the contingency is resolved over the remaining performance period, which is currently estimated to begin in May 2014 and conclude in October 2014.

In anticipation of a potential Biologics License Application, or BLA, filing and subsequent commercialization, we signed an agreement in June 2013 with Lonza Group Ltd, or Lonza, to develop a commercial-scale manufacturing process for Andexanet alfa. We have transferred manufacturing of Andexanet alfa to Lonza and are making process improvements in order to increase scale and efficiency. We plan to implement proposed changes at Lonza to initiate BLA-enabling studies with a manufacturing process that will allow us to launch Andexanet alfa pursuant to an accelerated approval. After recent discussions with the FDA, we determined that the additional process changes needed to further reduce cost of goods will be incorporated into the commercial production later in the development of Andexanet alfa or as a supplemental BLA, if supporting studies are required.

#### PRT2070

In addition to our thrombosis products, we have discovered two novel orally available kinase inhibitors to treat hematologic disorders and inflammation. The first, PRT2070, 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 PRT2070 for the treatment of certain B-cell hematologic cancers. We have completed preclinical testing for PRT2070, and initiated a Phase 1/2 proof-of-concept study in non-Hodgkin's lymphoma and chronic lymphocytic leukemia in October 2013.

In February 2013, we entered into an agreement with Aciex Therapeutics, Inc., or Aciex, for topical and intranasal co-development and co-commercialization of 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. We retain rights to other non-systemic indications, including dermatologic disorders. 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. Third-party related development costs incurred pursuant to this agreement will be shared by Aciex and us 60% and 40%, respectively, until the end of the Phase 2 clinical study, and shared equally thereafter. 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. No costs related to this agreement were incurred during the three and nine months ended September 30, 2013.

#### PRT2607

Our second kinase inhibitor, PRT2607, is an orally available, potent and selective inhibitor of Syk. Syk is an important mediator of immune response in a number of different types of immune cells. PRT2607 has been successfully evaluated in 131 subjects in several Phase 1 clinical studies. Biogen Idec Inc., or Biogen Idec, is leading the pre-clinical study of PRT2607 and other highly selective Syk inhibitors for allergic asthma and other inflammatory disorders and is responsible for all development-related expenses.

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.



In June 2005, we entered into a license agreement with Astellas Pharma, Inc., or Astellas, pursuant to which we licensed from Astellas certain rights to research, develop and commercialize Syk kinase inhibitors, including PRT2070 and PRT2607. This agreement was amended in December 2010.

#### Other

Prior to 2012, we were developing Elinogrel, a novel anti-platelet agent. In February 2009, we entered into a worldwide collaboration and license agreement with Novartis Pharma A.G., or Novartis, to develop and commercialize Elinogrel. In April 2012, we and Novartis agreed to a plan for Novartis to return all rights to Elinogrel to us and to terminate our agreement, effective July 1, 2012. Although we may resume development of Elinogrel in the future, we currently do not plan to do so.

For purposes of this discussion and analysis of our financial condition and results of operations, we refer to our agreements with Millennium, Merck, Lee's, BMS and Pfizer, Bayer and Janssen, Daiichi Sankyo, Acix, Biogen Idec, Astellas and 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. Under our agreements with Biogen Idec, Merck, Novartis, BMS and Pfizer, Bayer and Janssen, Daiichi Sankyo and Lee's, we received payments including non-refundable upfront license fees, a refundable contingent payment and a milestone payment in the aggregate amount of \$178.7 million, of which \$6.0 million was received in July 2013 pursuant to our agreement with Daiichi Sankyo.

We may also be entitled to additional milestone payments and other contingent payments upon the occurrence of specific events. We have achieved one milestone and received a milestone payment for its achievement and we have received one contingent refundable payment under our collaboration agreements as of September 30, 2013. Due to the nature of these collaboration agreements and the nonlinearity of the related revenue recognition, we expect that our revenue will continue to fluctuate in future periods.

The following table summarizes the sources of our revenue for the three and nine months ended September 30, 2012 and 2013:

	Three Months Ended September 30, 2013		Nine Months Ended September 30, 2013	
		2012	2013	2012
			(in thousands) (unaudited)	
Novartis:				
Recognition of upfront license fee	\$	\$ 564	\$	\$ 53,846
Reimbursement of research and development expenses		174		16,238
Novartis total		738		70,084
BMS and Pfizer:				
Recognition of research and development services		772		3,866
BMS and Pfizer total		772		3,866
Bayer and Janssen:				
Recognition of research and development services		1,028		3,466
Bayer and Janssen total		1,028		3,466
Lee s				
Recognition of research and development services		78		130
Lee s total		78		130
Daiichi Sankyo:				
Recognition of research and development services		888		1013
Daiichi Sankyo total		888		1013
Total collaboration and license revenue	\$ 2,766	\$ 738	\$ 8,475	\$ 70,084
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. 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.

The following table summarizes our research and development expenses incurred by product candidate during the three and nine months ended September 30, 2012 and 2013:

	Phase of development	Three Months Ended September 30,		Nine Months Ended September 30,	
		2013	2012	2013	2012
(in thousands)					
(unaudited)					
Product candidate					
Betrixaban	Phase 3	\$ 7,310	\$ 4,652	\$ 30,007	\$ 18,745
Andexanet alfa	Phase 2	8,906	3,386	22,683	11,243
PRT2070	Phase 1/2	1,768	110	3,913	318
PRT2607	Pre-clinical	93	971	(113)	3,256
Elinogrel <sup>(1)</sup>	Phase 3 ready	13	81	59	182
Other research and development expenses <sup>(2)</sup>		(1)	755	93	2,259
Total research and development expenses <sup>(3)</sup>		\$ 18,088	\$ 9,955	\$ 56,642	\$ 36,003

(1) Although we may resume development of Elinogrel in the future, we currently do not plan to do so.

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

(3) 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. Reimbursement of research and development expenses related to the cost-sharing provisions of our agreements with Merck and Novartis were recognized as revenue pursuant to the revenue recognition accounting policy applicable to these agreements.

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.

#### General and administrative expenses

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. Personnel costs consist of salaries, benefits and stock-based compensation. We expect to incur additional expenses as a result of operating as a public company, including expenses related to compliance with the rules and regulations of the Securities and Exchange Commission, or SEC, and those of The NASDAQ Global Market, additional insurance expenses, investor relations activities and other administration and professional services.

#### 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 and gains and losses resulting from the remeasurement of our convertible preferred stock warrant liability. We recorded adjustments to the estimated fair value of the convertible preferred stock warrants until they were converted into warrants to purchase shares of our common stock upon the closing of our initial public offering, or IPO. At that time, we reclassified the convertible preferred stock warrant liability to additional paid-in capital and we will no longer record any related periodic fair value adjustments.

## 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 financial statements, which have been prepared in accordance with United States generally accepted accounting principles, or U.S. GAAP. The preparation of these 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 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 three and nine months ended September 30, 2013, 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 prospectus dated May 21, 2013 filed with the SEC pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, or Securities Act.

## Results of operations

Comparison of the three and nine months ended September 30, 2013 and 2012

## Revenue

	Three Months Ended		Increase / (Decrease)	% Increase / (Decrease)	Nine Months Ended		Increase / (Decrease)	% Increase / (Decrease)
	September 30, 2013	September 30, 2012			September 30, 2013	September 30, 2012		

(dollars in thousands)

Collaboration and license revenue	\$ 2,766	\$ 738	\$ 2,028	275%	\$ 8,475	\$ 70,084	\$ (61,610)	(88)%
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The increase in collaboration and license revenue during the three months ended September 30, 2013 was due to the increase in collaboration and license revenue with respect to our agreements with BMS and Pfizer, Bayer and Janssen, Lee's and Daiichi Sankyo, which we entered into in the fourth quarter of 2012, the first quarter of 2013, the first quarter of 2013, and the second quarter of 2013, respectively. This increase in collaboration and license revenue was partially offset by the decrease in revenue recognized from Novartis. We recognized no revenue from our agreement with Novartis during the three months ended September 30, 2013, compared to revenue of \$0.7 million recognized from our agreement with Novartis during the three months ended September 30, 2012 following the termination of our agreement with Novartis effective July 1, 2012.

The decrease in collaboration and license revenue during the nine months ended September 30, 2013 was due to the decrease in collaboration and license revenue from Novartis following the termination of our agreement with Novartis effective July 1, 2012. We recognized no revenue from our agreement with Novartis during the nine months ended September 30, 2013, compared to revenue of \$70.1 million recognized from our agreement with Novartis during the nine months ended September 30, 2012. This decrease in collaboration and license revenue was partially offset by revenue recognized during the nine months ended September 30, 2013 with respect to our agreements with BMS and

Pfizer of \$3.9 million, Bayer and Janssen of \$3.5 million, Lee's of \$0.1 million and Daiichi Sankyo of \$1.0 million.

Pursuant to our agreement with BMS and Pfizer, we are obligated to provide research and development services and participate on various committees. We originally estimated the period of performance of our obligations to extend through June 2013. In March 2013, we revised our estimated period of performance to be through July 2013, in June 2013 we revised our estimated period of performance to be through September 2013 and in September 2013 we revised our estimated period of performance to be through October 2013. The total consideration under this agreement of \$6.0 million was recognized as revenue on a straight-line basis over the estimated performance period through October 2013.

Pursuant to our agreement with Bayer and Janssen, we are obligated to participate on a JCC with Bayer and Janssen to oversee the collaboration activities under the agreement. We originally estimated the period of performance of our obligations to extend through November 2013. In June 2013 we revised our estimated period of performance to be through January 2014 and in September 2013 we revised our estimated period of performance to be through March 2014. The total consideration under this agreement of \$5.5 million is being recognized as revenue ratably over the estimated performance period through March 2014.

Pursuant to our agreement with Daiichi Sankyo, we are obligated to perform preclinical proof-of-concept studies and participate on a JCC with Daiichi Sankyo to oversee the collaboration activities under the agreement. The total consideration under this agreement is \$6.0 million, of which the total non-contingent consideration of \$3.0 million is being recognized as revenue ratably over the estimated performance period through May 2014.

We expect revenue recognized in future periods, until we achieve product commercialization, to be lower than that in 2012 primarily due to no further revenue being recognized in connection with our terminated agreement with Novartis, which may be partially offset by an increase in revenue recognized in connection with any new collaboration agreements.

#### Research and development expenses

	Three Months Ended September 30,		Increase /	% Increase /	Nine Months Ended September 30		Increase /	% Increase /
	2013	2012	(Decrease)	(Decrease)	2013	2012	(Decrease)	(Decrease)
	(dollars in thousands)							
Research and development expenses	\$ 18,088	\$ 9,955	\$ 8,133	82%	\$ 56,642	\$ 36,003	\$ 20,639	57%

The increase in research and development expenses during the three months ended September 30, 2013 was primarily due to the following:

- increased program costs of \$2.7 million to advance Betrixaban;

- increased program costs of \$5.4 million to advance Andexanet alfa; and

- increased program costs of \$1.7 million to advance PRT2070.

These increases were partially offset by:

- decreased net program costs of \$0.9 million related to PRT2607, primarily due to reimbursements received from Biogen Idec to fund clinical and manufacturing costs pursuant to the cost-sharing provisions of our agreement with Biogen Idec; and

- decreased development costs of \$0.8 million as we reduced costs for programs that are not related to or in support of our primary programs of development; Betrixaban, Andexanet alfa and PRT2070.

The increase in research and development expenses during the nine months ended September 30, 2013 was primarily due to the following:

- increased program costs of \$11.3 million to advance Betrixaban;



- increased program costs of \$11.4 million to advance Andexanet alfa; and

- increased program costs of \$3.6 million to advance PRT2070.

These increases were partially offset by:

- decreased net program costs of \$3.4 million related to PRT2607, primarily due to reimbursements received from Biogen Idec to fund clinical and manufacturing costs pursuant to the cost-sharing provisions of our agreement with Biogen Idec; and

- decreased development costs of \$2.3 million as we reduced costs for programs that are not related to or in support of our primary programs of development; Betrixaban, Andexanet alfa and PRT2070.

We expect our research and development expenses to increase in the future as we 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.

## General and administrative expenses

	Three Months Ended September 30,		Increase % Increase / /		Nine Months Ended September 30		Increase % Increase / /	
	2013	2012	(Decrease)	(Decrease)	2013	2012	(Decrease)	(Decrease)
(dollars in thousands)								
General and administrative expenses	\$ 3,907	\$ 2,879	\$ 1,028	36%	\$ 10,654	\$ 8,744	\$ 1,911	22%

The increase in general and administrative expenses during the three months ended September 30, 2013 was primarily related to increased headcount related costs including stock based compensation expense resulting from the increased value of the underlying stock options following our IPO in May 2013 of \$1.0 million, and increased costs associated with being a public company including directors and officer's insurance and director fees of \$0.2 million, partially offset by lower professional and legal fees to support business development and collaboration arrangements of \$0.1 million.

The increase in general and administrative expenses during the nine months ended September 30, 2013 was primarily related to increased headcount related costs including an increase in stock based compensation expense resulting from the increased fair value of new stock options following our IPO in May 2013 of \$2.0 million, and increased costs associated with being a public company including directors and officer's insurance and director fees of \$0.3 million, partially offset by lower professional and legal fees to support business development and collaboration arrangements of \$0.4 million.

We expect general and administrative expenses to increase in order for us to continue to support the costs of being a public company.

## Interest and other income (expense), net

	Three Months Ended September 30,		Increase % Increase / /		Nine Months Ended September 30		Increase % Increase / /	
	2013	2012	(Decrease)	(Decrease)	2013	2012	(Decrease)	(Decrease)
(dollars in thousands)								
Interest and other income (expense), net	\$ 679	\$ 607	\$ 72	12%	\$ 532	\$ (188)	\$ 720	(382)%

The increase in interest and other income (expense), net during the three months ended September 30, 2013 of \$0.1 million is primarily due to foreign currency exchange gains of \$0.6 million in the three months ended September 30, 2013, compared to foreign currency exchange gains of \$0.5 million in the three months ended September 30, 2012. These gains and losses are primarily related to fluctuations in the Euro compared to the U.S. dollar and unrealized gains and losses related to our foreign currency forward contracts.

The increase in interest and other income (expense), net during the nine months ended September 30, 2013 of \$0.7 million is primarily due foreign currency exchange gains of \$0.5 million in the nine months ended September 30,

2013, compared to foreign currency exchange losses of \$0.2 million in the three months ended September 30, 2012, related to fluctuations in the Euro compared to the U.S. dollar and unrealized gains and losses related to our foreign currency forward contracts.

#### 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 sales of our common stock as part of our IPO, and of our convertible preferred stock and payments from our collaboration partners. Our expenditures are primarily related to research and development activities. We have received additional funding from long-term debt and interest earned on investments. At September 30, 2013, we had available cash, cash equivalents and investments of \$218.9 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.

On May 28, 2013, we closed our IPO, of 9,686,171 shares of our common stock, which included 1,263,413 shares of common stock issued pursuant to the over-allotment option granted to the underwriters. The public offering price of the shares sold in the offering was \$14.50 per share. The offer and sale of all of the shares in the IPO were registered under the Securities Act pursuant to a registration statement on Form S-1 (File No. 333-187901), which was declared effective by the SEC on May 21, 2013. The total proceeds from the offering to us, net of underwriting discounts and commissions of approximately \$9.4 million, were approximately \$131.0 million. After deducting offering expenses payable by us of approximately \$5.1 million, our net proceeds were approximately \$125.8 million. As of September 30, 2013, no accrued offering costs remained unpaid. Upon the closing of the IPO, 24,026,797 shares of convertible preferred stock then outstanding automatically converted into 24,026,797 shares of our common stock.

On October 22, 2013, we closed a follow-on public offering of 6,366,513 shares of our common stock, which included 4,457,710 shares issued and sold by us and 1,908,803 shares of common stock sold by certain existing stockholders of Portola. The public offering price of the shares sold in the offering was \$23.75 per share. In addition, the underwriters of the offering have been granted a 30-day option to purchase up to an additional 954,976 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 a registration statement on Form S-1 (File No. 333-191609), which was declared effective by the SEC on October 16, 2013. The total proceeds from the offering to us, net of underwriting discounts and commissions of approximately \$6.4 million and excluding the underwriters' option, were approximately \$99.5 million. After deducting estimated offering expenses payable by us of approximately \$0.8 million, our net proceeds are estimated to be approximately \$98.8 million.

In February 2009, July 2009, November 2011, December 2012, February 2013, March 2013 and April 2013, in connection with our agreements with Novartis, Merck and Biogen Idec, BMS and Pfizer, Bayer and Janssen, and Lee's we received payments of \$75.0 million, \$50.0 million, \$36.0 million, \$6.0 million, \$5.0 million, and \$0.7 million, respectively, as initial upfront payments and a milestone payment of \$2.0 million from BMS and Pfizer. These payments are reflected as deferred revenue and included within cash used in operating activities. In addition, in July 2013, we received \$6.0 million pursuant to our agreement with Daiichi Sankyo, of which \$3.0 million was recorded as receivables from collaborations and deferred revenue at September 30, 2013. In addition, in November 2011, we received proceeds of \$98.0 million from the sale of our convertible preferred stock.

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

	Nine Months ended September 30, 2013                      2012 (in thousands)	
Cash used in operating activities	\$ (42,906)	\$ (44,324)
Cash used in investing activities	(78,520)	(72,031)
Cash provided by financing activities	126,523	292
Net increase (decrease) in cash	\$ 58,710	\$ 54,350

#### Cash used in operating activities

Cash used in operating activities was \$42.9 million for the nine months ended September 30, 2013 reflecting a net loss of \$58.2 million, which was decreased by non-cash charges of \$3.5 million for stock-based compensation, \$1.5 million for amortization of premium on investments and \$1.0 million for depreciation and amortization. Cash used in

operating activities also reflected an increase in net operating assets of \$9.4 million primarily due to increases in accounts payable and accrued and other liabilities of \$6.6 million related to higher clinical study and related costs as we continue to increase our research and development activities, an increase in deferred revenue of \$3.2 million due to an increase in deferred revenue of \$5.0 million related to the upfront payments received from Bayer and Janssen, \$6.0 million related to the upfront payments received from Daiichi Sankyo and \$0.7 million related to the upfront payments received from Lee's in the nine months ended September 30, 2013, partially offset by the recognition of collaboration revenue earned of \$8.5 million from our collaboration agreements. Cash used in operating activities also reflected a decrease in prepaid expenses and other current assets of \$0.5 million primarily reflecting payment and classification of deferred offering costs of \$1.6 million and recognition of clinical trial upfront fees upon contract execution of \$0.8 million partially offset by higher prepaid premiums for corporate director's and officer's insurance of \$0.4 million following the renewal of our corporate insurance program and placement of our public company policies, interest receivable on our investment portfolio of \$0.4 million, unrealized gains on our foreign currency forward contracts of \$0.2 million and prepaid rent of \$0.2 million in the nine months ended September 30, 2013. Also reflected in cash used in operating activities is a decrease in receivables from collaborations of \$0.4 million due to the receipt of research and development expenses reimbursable from Biogen Idec pursuant to our agreement with Biogen Idec.

Cash used in operating activities was \$44.3 million for the nine months ended September 30, 2012 reflecting net income of \$25.1 million, which was increased by non-cash charges of \$2.0 million for stock-based compensation, \$1.1 million for depreciation and amortization and \$1.0 million for amortization of premium on investments, unrealized losses on foreign currency forward contracts of \$0.5 million and decreased by \$0.1 million for revaluation of preferred stock warrant liabilities. Cash used in operating activities also reflected a decrease in net operating assets of \$73.9 million primarily due to a decrease in deferred revenue of \$69.5 million following the termination of our agreement with Novartis effective July 1, 2012, the decrease in receivables from collaborations of \$2.3 million due to payments related to research and development expenses reimbursable from Biogen Idec pursuant to our agreements with Biogen Idec a decrease in accrued compensation and employee benefits of \$0.9 million due to 2011 bonuses that were paid in the nine months ended September 30, 2012, and a decrease in prepaid expenses and other current assets of \$2.2 million primarily for clinical study costs expensed which were paid in advance to our CRO and prepaid clinical study insurance. Also reflected in cash used in operating activities is an increase in accounts payable and accrued and other liabilities of \$2.0 million related to higher clinical study and related costs as we continue to increase our research and development activities.

#### Cash used in investing activities

Cash used in investing activities of \$78.5 million for the nine months ended September 30, 2013 was primarily related to purchases of investments of \$152.8 million and capital equipment purchases of \$0.6 million, partially offset by proceeds from sales of investments of \$6.6 million and proceeds from maturities of investments of \$68.2 million.

Cash used in investing activities of \$72.0 million for the nine months ended September 30, 2012 was primarily related to capital equipment purchases of \$0.4 million and purchases of investments of \$133.1 million, partially offset by proceeds from sales of investments of \$42.8 million and proceeds from maturities of investments of \$18.6 million.

#### Cash provided by financing activities

Cash provided by financing activities of \$126.5 million for the nine months ended September 30, 2013, was primarily related to proceeds from our IPO, net of underwriting discounts and commissions of \$131.0 million, partially offset by payments of deferred offering costs of \$5.0 million and proceeds from the exercise of stock options of \$0.5 million.

Cash provided by financing activities of \$0.3 million for the nine months ended September 30, 2012, was related to proceeds from the exercise of stock options.

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 collaboration. 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;
- 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

Since our inception, we have not engaged in any off-balance sheet arrangements, including the use of structured finance, special purpose entities or variable interest entities.

### 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 September 30, 2013, we had cash, cash equivalents and investments of \$218.9 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 \$6.7 million to our European vendors during the nine months ended September 30, 2013. 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. For the nine months ended September 30, 2013, the effect of the exposure to these fluctuations in foreign exchange rates was not material. A 10% change in the exchange rates upward or downward in our portfolio of foreign currency forward contracts would have increased unrealized gain by \$1.6 million or decreased unrealized gain by \$6.0 million, respectively, at September 30, 2013. We hedge our foreign currency exposures but we have not used derivative financial instruments for speculation or trading purposes.

### 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 September 30, 2013. Based on such evaluation, our principal executive officer and principal financial officer have concluded that, as of September 30, 2013, our disclosure controls and procedures were effective at the reasonable assurance level.

## 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 prospectus, 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

Although we reported net income for the years ended December 31, 2012 and December 31, 2011, we have incurred significant losses prior to 2011 and for the nine months ended September 30, 2013 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. Although we reported net income for the years ended December 31, 2012 and December 31, 2011, this was primarily due to the recognition of all remaining deferred revenue following the termination of two of our collaboration agreements. We have incurred significant operating losses prior to 2011 and for the nine months ended September 30, 2013 and expect to incur substantial and increasing losses for the foreseeable future. As of September 30, 2013, we had an accumulated deficit of \$260.5 million.

To date, we have financed our operations primarily through private placements of our convertible preferred stock, sale of our common stock in our initial public offering and follow-on offering, 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 our expenses will increase substantially 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, formerly PRT4445, at commercial scale; and
  - enhance operational, financial and information management systems and hire more personnel, including personnel to support development of our product candidates and, if a product candidate is approved, our commercialization efforts.
- To be profitable in the future, we must succeed in developing and eventually 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. For example, in the year ended December 31, 2011, we recognized all remaining deferred revenue of approximately \$8.3 million following the termination of our exclusive worldwide license and collaboration agreement with Merck & Co., Inc., or Merck, and in the year ended December 31, 2012, we recognized all remaining deferred revenue of approximately \$65.1 million following the termination of our worldwide license agreement with Novartis Pharma A.G., or Novartis. In addition, we measure compensation cost for stock-based awards made to employees at the grant date of the award, based on the fair value of the award as determined by our board of directors, and recognize the cost as an expense over the employee's requisite service period. As the variables that we use as a basis for valuing these awards change over time, including our underlying stock price and stock price volatility, the magnitude of the expense that we must recognize may vary significantly. 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 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 and/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 September 30, 2013, we had approximately \$218.9 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 degree and rate of market acceptance of any products launched by us or future partners;
- our ability to enter into additional collaboration, licensing, commercialization or other arrangements and the terms and timing of such arrangements; and

- the emergence of competing technologies or other adverse market developments.

We do not have any material committed external source of funds or other support for our development efforts other than our exclusive worldwide license and collaboration agreement with Biogen Idec Inc., or Biogen Idec, for the development and commercialization of PRT2607 and other highly selective Syk inhibitors, which is terminable by Biogen Idec without cause upon 120 days' notice. 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 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 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 or research and development programs or our 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 PRT2070 and our selective Syk inhibitor program. 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, a novel oral once-daily inhibitor of Factor Xa, an enzyme involved in the body's coagulation system, that seeks to inhibit the blood coagulation process, and Andexanet alfa, a recombinant protein designed to reverse the anticoagulant activity in patients treated with a Factor Xa inhibitor who suffer an uncontrolled bleeding episode or undergo emergency surgery, and, to a lesser extent, PRT2070 and our selective Syk inhibitor program. Our ability to generate

product revenue, which we do not expect to occur for at least the next several years, if ever, will depend heavily 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, particularly Andexanet alfa;
- 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 at commercially acceptable costs;
- launching commercial sales of 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 clinical study of Betrixaban, which we refer to as APEX, for extended duration VTE prophylaxis for up to 35 days in acute medically ill patients with restricted mobility and other risk factors, 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 first Phase 2 proof-of concept study of Andexanet alfa, evaluating the effect of Andexanet alfa in healthy volunteers taking apixaban, may not be predictive of success in our other Phase 2 proof-of-concept studies or other later studies. We do not yet know how the results from our Phase 1 studies of Andexanet alfa or our Phase 2 study in healthy volunteers taking Andexanet alfa will translate into clinical outcomes



in our intended target population of patients treated with a Factor Xa inhibitor who suffer an uncontrolled bleeding episode or undergo emergency surgery. Moreover, the results from our study 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 a finding that our product candidates have unanticipated serious side effects or other unexpected characteristics or that the patients are being exposed to unacceptable health risks;

- 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. For example, in 2010, we suspended our Phase 1 clinical study of PRT2607 in order to investigate potentially adverse toxicology findings in an animal study that was being conducted concurrently. A follow-up study determined

that there was not a significant safety risk, but the completion of the study was delayed by approximately nine months.

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 leading product candidates has completed clinical development. The risk of failure of clinical development is high. It is impossible to predict when or if any of our product candidates will prove safe enough to receive regulatory approval. For example, our lead product candidate Betrixaban, like all currently marketed inhibitors of Factor Xa, carries some risk of life-threatening bleeding. In addition, subjects taking Betrixaban 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 will not fail due to safety issues. In such an event, we might need to abandon development of Betrixaban 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 its 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 the study.

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 APEX study is expected to enroll approximately 6,850 patients at approximately 400 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 at a sufficient pace 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 the study to be completed by mid-2015. Completing the study by that date will require us to continue to activate new clinical study sites and to enroll patients at forecasted rates at both new and existing clinical study sites. Our forecasts regarding the rates of clinical site activation and patient enrollment at those sites are based on a number of assumptions including assumptions based on past experience with our APEX study. However, there can be no assurance that those forecasts will be accurate or that we will complete our APEX study by the currently anticipated date. During the initial months of the APEX study, the number of clinical sites activated and the number of patients enrolled at each clinical site per month was lower than we had anticipated and, as a result, we made a number of adjustments to the clinical study plan, including increasing the number of clinical study sites. We can provide no assurance that those adjustments will be sufficient to enable us to complete the APEX study within our anticipated time frame. If we experience delays in enrollment, our ability to complete our APEX study could be materially adversely affected.

If we are unable to enroll the patients at the projected 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. For example, in October 2012, we decided to increase the number of study sites for our APEX study and make certain changes to the management of the study in order to increase the enrollment rate, which had been slower than originally anticipated. These adjustments increased the cost of the study.

Even if our APEX study demonstrates statistically significant safety and efficacy of Betrixaban for extended duration VTE prophylaxis in acute medically ill patients for up to 35 days, 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 up to 35 days. 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 up to 35 days, 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 limit potential sales of Betrixaban.

If we fail to obtain FDA or other regulatory approval of Betrixaban or if the approval is for an indication that is narrower than what we seek, it could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We anticipate seeking regulatory approval of Andexanet alfa in the United States through an accelerated approval process, and if the FDA does not determine that such a process is available for Andexanet alfa, then the development or commercialization of Andexanet alfa could be delayed or abandoned.

We currently plan to seek FDA approval of Andexanet alfa through an accelerated approval process, such as

Accelerated Approval, which is a process allowing drugs that treat serious diseases for which there is an unmet medical need to be approved on a shortened timetable based on use of a surrogate endpoint in clinical studies or with restrictions to promote safe use. However, we have not reached agreement with the FDA on a development plan for Andexanet alfa under accelerated approval or any other expedited process. If the FDA does not allow us to pursue an accelerated approval process for Andexanet alfa or determines that a study based on a surrogate endpoint will not be acceptable, the time and expense associated with developing Andexanet alfa would be significantly greater than we currently anticipate, and we might be required to enter into a partnership in order to develop Andexanet alfa or delay or abandon development of Andexanet alfa. Even if we are able to pursue an accelerated approval process, 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. If the FDA determines that a randomized, placebo-controlled study demonstrating superior efficacy of Andexanet alfa in Factor Xa inhibitor treated patients experiencing a severe bleeding event is required for approval of Andexanet alfa, it may not be feasible to conduct such a trial or may take many years to complete at substantially greater cost.

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.

Our product candidates have never been manufactured on a commercial scale, and there are risks associated with scaling up manufacturing to commercial scale. In particular, we will need to develop a larger scale manufacturing process that is more efficient and cost-effective to commercialize Andexanet alfa, which may not be successful, and which will require us to transfer our production to another manufacturer, potentially delaying regulatory approval and commercialization.

Our product candidates have never been manufactured on a commercial scale, and there are risks associated with scaling up manufacturing to commercial scale including, among others, cost overruns, potential 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 manufacturer is unable to produce sufficient quantities of the approved product for commercialization, our commercialization efforts would be impaired, which would have an 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 biological molecule, or biologic, rather than a small molecule chemical compound like our other product candidates. The manufacture of biologics involves complex processes, including developing cells or cell systems to produce the biologic, growing large quantities of such cells and harvesting and purifying the biologic produced by them. As a result, the cost to manufacture biologics is generally far higher than traditional small molecule chemical compounds, and the manufacturing process is less reliable and is difficult to reproduce. Andexanet alfa is currently produced for us by a third-party contract manufacturer using a small-scale process that is too expensive and inefficient to support the commercialization of Andexanet alfa in the dosages and at the sales volumes and price that would be necessary for a commercially viable drug. We have entered into an agreement with a third party manufacturer to develop a more efficient, larger-scale commercial manufacturing process. However, scaling up and improving a biologic manufacturing process is a difficult and uncertain task, and we can give no assurance that we will be successful in developing and implementing this new process. In particular, we will need to demonstrate that the new process produces material that is comparable to the material we previously used. Demonstrating comparability can require significant pre-clinical and clinical studies. If we are not able to demonstrate comparability, then the material would be considered a new biological entity and a full Biologics License Application, or BLA, submission would be required for approval. Additionally, if the therapeutically effective dosage of Andexanet alfa is higher than we anticipate or the obtainable sales price is lower than we anticipate, we may not be able to successfully commercialize Andexanet alfa.

We currently have no sales or distribution personnel and only limited 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 have a significant sales or marketing infrastructure and have no experience in the sale, marketing or distribution of 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 and 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 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 the treatment of 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 lead product candidate Betrixaban for extended duration VTE prophylaxis in acute medically ill patients for up to 35 days. 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 for the current standard of care, enoxaparin. Furthermore, the FDA has already approved a number of therapies that, like Betrixaban, are 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.

While there are no therapies approved specifically as antidotes for Factor Xa inhibitors, 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 and that at least one company has initiated a Phase 1 clinical trial of an antidote.

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 PRT2070 or PRT2607. 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 non-competitive. 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.

If we are able to commercialize any product candidates, the products may become subject to unfavorable pricing regulations, third-party reimbursement practices or healthcare reform initiatives, thereby harming our business.

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.

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 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.

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.

#### 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 and communication difficulties. 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, particularly with respect to Andexanet alfa, to find new suppliers or manufacturers. We may also face delays in the development and commercialization of our product candidates.

We currently have limited experience in, and we do not own facilities for, clinical-scale manufacturing of our product candidates and we rely upon third-party contract manufacturing organizations to manufacture and supply drug product for our clinical studies. 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 control, including stability of the product candidate and quality assurance 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 provide study drugs in our clinical studies 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 manufacture, packaging or testing of products. We have little 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.

We currently rely on a single source supplier 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, and we have recently engaged Lonza Group Ltd., or Lonza, to be the sole manufacturer of Andexanet alfa. Our current agreements with our suppliers do not provide for the entire supply of the drug product necessary for all anticipated clinical studies or for full scale commercialization. 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 the product candidate until a qualified alternative supplier is identified, which could also delay the development of, and impair our ability to commercialize, our product candidates. One of our leading product candidates, Andexanet alfa, is a biologic and therefore requires a complex production process. We have transferred production of Andexanet alfa to a new manufacturer, Lonza, and are also engaging a new sole-source vendor to perform lyophilization and packaging. In connection with the transfer of production, we intend to make certain changes to the manufacturing process in order to increase its scale and efficiency. There can be no assurance that we will be able to successfully implement these transitions or implement the proposed improvements to the manufacturing process. In particular, 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 material we previously used.

Demonstrating comparability can require significant pre-clinical and clinical studies. If we are not able to demonstrate comparability, then the material would be considered a new biological entity and a full BLA submission would be required for approval, resulting in additional time and expense. If we are not able to implement the proposed transitions in a timely manner, or establish comparability of the new material, or obtain the anticipated improvements in efficiency, our business, results of operations and growth prospects would be materially adversely affected.

Although alternative sources of supply exist, the number of third-party suppliers with the necessary manufacturing and regulatory expertise and facilities 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 have entered into a collaboration agreement with each of Lee's, BMS and Pfizer, Bayer and Janssen, Daiichi Sankyo, Biogen Idec and Acix with respect to our product candidates. These collaborations may place the development of these product candidates outside our control, may require us to relinquish important rights or may otherwise be on terms unfavorable to us, and if our collaborations are not successful, these product candidates may not reach their full market potential.

In January 2013, we entered into a clinical collaboration agreement with Lee's Pharmaceutical (HK) Ltd, or Lee's, to jointly expand our 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. In October 2012, we entered into a three-way agreement with Bristol-Myers Squibb Company, or BMS, and Pfizer Inc., or Pfizer, to include subjects dosed with apixaban, their jointly owned Factor Xa inhibitor product, in one of our proof-of-concept studies of Andexanet alfa. 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 proof-of-concept studies of Andexanet alfa. In June 2013, we entered into an agreement with Daiichi Sankyo, Inc., or Daiichi Sankyo, to include subjects dosed with edoxaban, their Factor Xa inhibitor product, in one of our proof-of-concept studies of Andexanet alfa. In February 2013, we entered into a license and collaboration agreement with Acix Therapeutics, Inc., or Acix, pursuant to which we granted Acix an exclusive license to co-develop and co-commercialize 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.

We retain rights to other non-systemic indications including dermatologic disorders. In October 2011, we entered into a collaboration agreement with Biogen Idec pursuant to which Biogen Idec has ultimate decision-making authority with respect to the research, development and commercialization of PRT2607 and other highly selective Syk inhibitors. We may enter into additional collaboration agreements with third parties with respect to our other product candidates for the commercialization of the candidates outside the United States. 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 other 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, such as our collaboration with Biogen Idec, 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. For example, we previously had an exclusive worldwide license and collaboration agreement with Merck for the development and commercialization of Betrixaban and an exclusive worldwide license agreement with Novartis for the development and commercialization of Elinogrel, a novel anti-platelet agent. In each case, the collaborator chose to terminate the collaboration for internal business reasons. As a result of these terminations, we were required to revise the development plan for Betrixaban and raise additional financing to support that plan, and we also decided to

halt our development efforts with respect to Elinogrel. Any termination or disruption of our collaboration with Biogen Idec or other potential collaborators could result in delays in the development of product candidates, increases in our costs to develop 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.

As of September 30, 2013, we had 58 employees. 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.

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 radioactive 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 also store certain low level radioactive waste at our facilities until the materials can be properly disposed of. 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, hazardous or radioactive 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.

Requirements associated with being a public company will increase our costs significantly, as well as divert significant company resources and management attention.

Prior to the completion of our initial public offering in May 2013, we were not subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, or Securities Exchange Act, or the other rules and regulations of the Securities and Exchange Commission, or SEC, or any securities exchange relating to public companies. With the assistance of our legal, independent accounting and financial advisors we have identified those areas in which changes should be made to our financial and management control systems to manage our growth and our obligations as a public company. These areas include corporate governance, corporate control, internal audit, disclosure controls and procedures and financial reporting and accounting systems. Making those changes has resulted, and will continue to result in our incurring significant expenses. In addition, compliance with the various reporting and other requirements applicable to public companies requires considerable time and attention of management. There can be no assurance that the changes we have made and will make will be sufficient to allow us to satisfy our obligations as a public

company on a timely basis.

In addition, as a public company, it may be more difficult or more costly for us to obtain certain types of insurance, including directors' and officers' liability insurance, and we may be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified personnel to serve on our board of directors, our board committees or as executive officers.

We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, which was enacted in April 2012. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years, although circumstances could cause us to lose that status earlier. We will remain an emerging growth company until the earlier of (1) December 31, 2018, (2) the last day of the fiscal year (a) in which we have total annual gross revenue of at least \$1.0 billion, or (b) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th, and (3) the date on which we have issued more than \$1.0 billion in non-convertible debt securities during the prior three-year period. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may suffer or be more volatile.

Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards, and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

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 and certain clinical sites for our product candidates, operations of our existing and future partners and suppliers are or will be located in California near major earthquake faults and fire zones. The ultimate impact on us, our significant partners, suppliers and our general infrastructure of being located near major earthquake faults and fire zones and being consolidated in certain geographical areas is unknown, but 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 studies, we are currently utilizing certain suppliers outside of the United States, which subjects us to certain of the above risks, but our risks will be significantly increased if we establish operations internationally.

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 were to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of 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, PRT2070 and PRT2607, 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. Under our collaboration agreement with Biogen Idec, we are obligated to use commercially reasonable efforts to file and prosecute patent applications, and maintain patents, covering PRT2607 and other highly selective Syk inhibitors in specified jurisdictions, and these patent rights are licensed to Biogen Idec.

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, must still implement various regulations, the courts have yet to address any of 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. An adverse determination in any 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.

For example, in August 2011, the USPTO declared an interference proceeding involving U.S. Patent No. 7,727,982 assigned to Millennium Pharmaceuticals, Inc., to which we have an exclusive license, and U.S. Application No. 12/203,640 assigned to Yamanouchi Pharmaceuticals Co., Ltd. Both of these patent applications potentially covered a Factor Xa inhibitor being developed by a competitor, but not Betrixaban or its lead backup compounds. As the competitor had ceased clinical development of its compound, we decided against contesting the interference proceeding and priority was given to U.S. Application No. 12/203,640. We do not believe this result will have a material 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 or our collaboration partners 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. Neither we nor our collaboration partners are permitted to market our product candidates in the United States until we receive approval of an NDA or a BLA, from the FDA. Neither we nor our collaboration partners have 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 filed by us;
- 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 and our collaboration partners 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 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/or 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 and/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. If we, our product candidates or the manufacturing facilities for our product candidates fail to comply with regulatory requirements of the FDA and/or other non-U.S. regulatory authorities, we could be subject 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 filed by us;
- restrictions on operations, including costly new manufacturing requirements; or



· seizure or detention of our products or import bans.

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.

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

We intend to seek a distribution and marketing partner for Betrixaban outside the United States and may market future products in international markets. 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 file for regulatory approvals and even if we file 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 PPACA, was enacted in 2010. The PPACA 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 PPACA, 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;
  - requires collection of rebates for drugs paid by Medicaid managed care organizations;
  - 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 PPACA in June 2012, other legal challenges are still pending final adjudication in several jurisdictions. In addition, Congress has also proposed a number of legislative initiatives, including possible repeal of the PPACA. At this time, it remains unclear whether

there will be any changes made to the PPACA, whether to certain provisions or its entirety. We cannot assure that the PPACA, 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 PPACA was enacted. For example, the Budget Control Act of 2011, 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. On January 2, 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 of 2011. 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. On March 1, 2013, the President signed an executive order implementing sequestration, and on April 1, 2013, the 2% Medicare reductions went into effect.

There likely will continue to be legislative and regulatory proposals at the federal and state levels directed at containing or lowering the cost of health care. 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 health care 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.

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 healthcare fraud and abuse and patient privacy regulation 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 healthcare program 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;

- indirectly, to induce either the referral of an individual, for an item or service or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs, such as the Medicare and Medicaid programs;

- 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, and which may apply to entities like us which provide coding and billing advice to customers;
  - federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
  - the federal transparency requirements under the Health Care Reform Law requires manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests;
  - 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 PPACA, 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 PPACA 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:

- the success of competitive products or technologies;
- results of clinical studies of our product candidates or those of our competitors;
- regulatory or legal developments in the United States and other countries, especially changes in laws or regulations applicable to our products;
- introductions and announcements of new products by us, our commercialization partners, or our competitors, and the timing of these introductions or announcements;
- actions taken by regulatory agencies with respect to our products, clinical studies, manufacturing process or sales and marketing terms;
- variations in our financial results or those of companies that are perceived to be similar to us;
- the success of our efforts to acquire or in-license additional products or product candidates;
- developments concerning our collaborations, including but not limited to those with our sources of manufacturing supply and our commercialization partners;
- developments concerning our ability to bring our manufacturing processes to scale in a cost-effective manner;

- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments
- developments or disputes concerning patents or other proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our products;
- our ability or inability to raise additional capital and the terms on which we raise it;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- 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.

A significant portion of our total outstanding shares are restricted from immediate resale but may be sold into the market in the near future. This could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. As of September 30, 2013, we had outstanding 35,229,352 shares of common stock. 26,985,598 of these shares are currently restricted as a result of securities laws or lock-up agreements. Moreover, holders of an aggregate of up to 24,107,872 shares of our common stock, including shares of our common stock issuable upon exercise of outstanding warrants, have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also intend to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements.

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

Our executive officers, directors and stockholders who own more than 5% of our outstanding common stock beneficially own shares representing approximately 30% of our common stock. As a result, if these stockholders were to choose to act together, they would be able to control or 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 control or 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.

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 newly public company, we are incurring significant legal, accounting and other expenses that we did not incur as a private company. In addition, 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 will need 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, beginning with our annual report on Form 10-K for the fiscal year ended December 31, 2013. In addition, we will be required to have our independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting



beginning with our annual report on Form 10-K following the date on which we are no longer an emerging growth company. Our compliance with Section 404 of the Sarbanes-Oxley Act will require that we incur substantial accounting expense and expend significant management efforts. We currently do not have an internal audit group, and we will need 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 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 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. 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.

An active trading market for our common stock may not be maintained

Our stock is currently traded on the NASDAQ, but we can provide no assurance that we will be able to maintain an active trading market for our shares on the NASDAQ or any other exchange in the future. If an active market for our common stock is not maintained, it may be difficult for our stockholders to sell shares without depressing the market price for the shares or at all.

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 acquiror from conducting a solicitation of proxies to elect the acquiror'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 \$2.8 million for severance and other benefits and acceleration of vesting of stock options with a value of approximately \$34.1 million as of September 30, 2013, based on the closing price of our common stock of \$26.75 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

### Unregistered Sales of Equity Securities

During the nine months ended September 30, 2013, we granted options in unregistered transactions to purchase an aggregate of 785,201 shares of common stock at a weighted average exercise price of \$16.97 per share to our employees. During such period, options were exercised in unregistered transactions to purchase 129,787 shares for cash consideration in the aggregate amount of \$0.2 million. The sales of the above securities were exempt from registration under Rule 701 promulgated under the Securities Act of 1933, as amended, or the Securities Act, as transactions pursuant to a compensatory benefit plan or a written contract relating to compensation. Shares of common stock to be issued pursuant to awards (including options) under our equity incentive plans were registered on a Registration Statement on Form S-8, filed with the SEC on May 31, 2013.

### Use of Proceeds

On May 21, 2013, our registration statement on Form S-1 (File No. 333-187901) was declared effective for our initial public offering. As a result of our initial public offering and the exercise of the overallotment option, both of which closed on May 28, 2013, we received net proceeds of approximately \$131.0 million, after underwriting discounts and commissions of approximately \$9.4 million. In addition, we incurred other expenses associated with our initial public offering of approximately \$5.2 million.

On October 16, 2013, our registration statement on Form S-1 (File No. 333-191609) was declared effective for our follow-on public offering. As a result of our follow-on public offering which closed on October 22, 2013, we received net proceeds of approximately \$99.5 million, after underwriting discounts and commissions of approximately \$6.4 million. We did not receive any proceeds from the sale of common stock by certain of our existing stockholders in the follow-on public offering. We estimate the expenses associated with our follow-on public offering to be

approximately \$0.8 million.

The net proceeds from the offerings described above were to be used and will be used, together with our cash, cash equivalents and investments, to fund continued advancement of our Betrixaban, Andexanet alfa and PRT2070 programs, anticipated to be approximately \$255.0 million, with the balance to be used to fund working capital, capital expenditures and other general corporate purposes, which may include the acquisition or licensing of other products, businesses or technologies.

There has been no material change in the planned use of proceeds from our initial public offering as described in our prospectus dated May 22, 2013, filed with the SEC pursuant to Rule 424(b) of the Securities Act or the planned use of proceeds from our public offering as described in our prospectus dated October 17, 2013, filed with the SEC pursuant to Rule 424(b) of the Securities Act.

### ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

ITEM 5. OTHER INFORMATION

None.

## ITEM 6. EXHIBITS

The following exhibits are filed as part of this report:

Exhibit Number	Exhibit Description	Incorporation By Reference			
		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 the registrant 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.				
4.5	Warrant to Purchase Shares of Series B Preferred Stock by and between the registrant and Hercules Technology Growth Capital, Inc., dated September 29, 2006.				
4.6	Warrant to Purchase Shares of Series B Preferred Stock by and between the registrant and Comerica Incorporated, dated September 29, 2006.				
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.				
4.8	Warrant to Purchase Shares of Common Stock by and between the registrant and HCP Life Science Assets TRS, LLC, dated December 15, 2006.				

- 4.9 Warrant to Purchase Shares of Common Stock by and between the registrant and Bristow Investments, L.P., dated December 15, 2006.
- 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<sup>(2)</sup>

101.SCH XBRL Taxonomy Extension Schema Document<sup>(2)</sup>

101.CAL XBRL Taxonomy Extension Calculation Linkbase Document<sup>(2)</sup>

101.DEF XBRL Taxonomy Extension Definition Linkbase Document<sup>(2)</sup>

101.LAB XBRL Taxonomy Extension Label Linkbase Document<sup>(2)</sup>

101.PRE XBRL Taxonomy Extension Presentation Linkbase Document<sup>(2)</sup>

(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.

(2) Pursuant to applicable securities laws and regulations, the Registrant is deemed to have complied with the reporting obligation relating to the submission of interactive data files in such exhibits and is not subject to liability under any anti-fraud provisions of the federal securities laws as long as the Registrant has made a good faith attempt to comply with the submission requirements and promptly amends the interactive data files after becoming aware that the interactive data files fail to comply with the submission requirements. These interactive data files are deemed not filed or part of a registration statement or prospectus for purposes of sections 11 or 12 of the Securities Act of 1933, as amended, are deemed not filed for purposes of section 18 of the Securities Exchange Act of 1934, as amended, and otherwise are not subject to liability under these sections.





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.

November 6, 2013 By: /s/ William Lis  
William Lis  
Chief Executive Officer

November 6, 2013 By: /s/ Mardi C. Dier  
Mardi C. Dier  
Chief Financial Officer

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