

PORTOLA PHARMACEUTICALS INC  
Form 10-Q  
May 10, 2018

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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended March 31, 2018

OR

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

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

Commission file number: 001-35935

PORTOLA PHARMACEUTICALS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware

(State or Other Jurisdiction of Incorporation or Organization)

270 E. Grand Avenue

20-0216859

(I.R.S. Employer Identification No.)

94080

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South San Francisco, California

(Address of Principal Executive Offices)

(Zip Code)

(650) 246-7000

(Registrant's Telephone Number, Including Area Code)

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

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

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

Large accelerated filer

Accelerated filer

Non-accelerated filer

(Do not check if a smaller reporting company) Smaller reporting company

Emerging growth company

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

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

As of May 7, 2018, the number of outstanding shares of the registrant's common stock, par value \$0.001 per share, was 65,825,191.



PORTOLA PHARMACEUTICALS, INC.

FORM 10-Q FOR THE QUARTER ENDED MARCH 31, 2018

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## PART I. FINANCIAL INFORMATION

ITEM 1. FINANCIAL STATEMENTS  
PORTOLA PHARMACEUTICALS, INC.

## Condensed Consolidated Balance Sheets

(Unaudited)

(In thousands, except share and per share data)

	March 31, 2018	December 31, 2017
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 168,414	\$ 181,568
Short-term investments	240,157	281,589
Restricted cash (SRX Cardio)	47	173
Trade and other receivables, net	1,693	3,750
Unbilled - collaboration and license revenue	4,660	—
Inventory	3,248	1,099
Prepaid research and development	3,449	734
Prepaid expenses and other current assets	14,569	9,010
<b>Total current assets</b>	<b>436,237</b>	<b>477,923</b>
Property and equipment, net	5,393	5,217
Intangible assets	7,710	7,851
Long-term investments	42,514	71,076
Prepaid and other long-term assets	4,812	9,609
<b>Total assets</b>	<b>\$496,666</b>	<b>\$ 571,676</b>
<b>Liabilities and stockholders' equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$20,788	\$ 9,304
Accrued compensation and employee benefits	5,113	11,526
Accrued research and development	22,105	44,973
Accrued and other liabilities	5,641	3,552
Deferred revenue, current portion	3,861	11,169
<b>Total current liabilities</b>	<b>57,508</b>	<b>80,524</b>
Notes payable, long-term	51,370	50,565
Long term debt	56,027	54,251
Long term obligation to collaborator	8,000	8,000
Deferred revenue, long-term	5,412	18,798
Other long-term liabilities	8,181	10,045
<b>Total liabilities</b>	<b>186,498</b>	<b>222,183</b>
<b>Stockholders' equity:</b>		
Preferred stock, \$0.001 par value, 5,000,000 shares authorized; no shares issued		
and outstanding	—	—

Common stock, \$0.001 par value, 100,000,000 shares authorized at March 31, 2018 and December 31, 2017; 65,811,082 shares and 65,296,643 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively	67	66
Additional paid-in capital	1,568,386	1,551,728
Accumulated deficit	(1,259,660)	(1,204,519 )
Accumulated other comprehensive loss	(801 )	(409 )
Total Portola stockholders' equity	307,992	346,866
Noncontrolling interest (SRX Cardio)	2,176	2,627
Total stockholders' equity	310,168	349,493
Total liabilities and stockholders' equity	\$496,666	\$ 571,676

Amounts include the assets and liabilities of SRX Cardio, LLC, a consolidated variable interest entity ("VIE"). Portola's interests and obligations with respect to the VIE's assets and liabilities are limited to those accorded to Portola in its agreement with the VIE. See Note 8, "Asset Acquisition and License Agreements," to these condensed consolidated financial statements. See accompanying notes to the unaudited condensed consolidated financial statements.

## PORTOLA PHARMACEUTICALS, INC.

## Condensed Consolidated Statements of Operations

(Unaudited)

(In thousands, except share and per share data)

	Three Months Ended March 31,	
	2018	2017
<b>Revenues:</b>		
Product revenue, net	\$ 606	\$—
Collaboration and license revenue	6,038	5,128
Total revenues	6,644	5,128
<b>Operating expenses:</b>		
Cost of sales	336	—
Research and development	60,067	30,645
Selling, general and administrative	31,541	15,021
Total operating expenses	91,944	45,666
Loss from operations	(85,300 )	(40,538 )
Interest and other income, net	3,371	413
Interest expense	(2,581 )	(1,639 )
Net loss	(84,510 )	(41,764 )
Net loss attributable to noncontrolling interest (SRX Cardio)	332	45
Net loss attributable to Portola	\$(84,178 )	\$(41,719 )
Net loss per share attributable to Portola common stockholders:		
Basic and diluted	\$(1.28 )	\$(0.74 )
Shares used to compute net loss per share attributable to Portola common stockholders:		
Basic and diluted	65,509,945	56,692,788

See accompanying notes to the unaudited condensed consolidated financial statements.

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PORTOLA PHARMACEUTICALS, INC.

Condensed Consolidated Statements of Comprehensive Loss

(Unaudited)

(In thousands)

	Three Months Ended March 31,	
	2018	2017
Net loss	\$(84,510)	\$(41,764)
Other comprehensive loss:		
Unrealized loss on available-for-sale securities, net of tax	(392 )	(79 )
Comprehensive loss	(84,902)	(41,843)
Comprehensive loss attributable to noncontrolling interest (SRX Cardio)	332	45
Total comprehensive loss attributable to Portola	\$(84,570)	\$(41,798)

See accompanying notes to the unaudited condensed consolidated financial statements.

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## PORTOLA PHARMACEUTICALS, INC.

## Condensed Consolidated Statements of Cash Flows

(Unaudited)

(In thousands)

	Three Months Ended March 31,	
	2018	2017
Operating activities		
Net loss	\$(84,510 )	\$(41,764 )
Adjustments to reconcile net loss to cash used in operating activities:		
Depreciation and amortization	756	504
Net amortization/accretion on investment securities	(491 )	161
Non-cash interest expense	2,581	1,639
Stock-based compensation expense	10,980	9,034
Remeasurement gain on embedded derivatives liabilities	(1,634 )	—
Changes in operating assets and liabilities:		
Inventories	(2,149 )	—
Trade and other receivables, net	4,763	—
Unbilled - collaboration and license revenue	2,034	—
Prepaid research and development	(2,715 )	1,401
Prepaid expenses and other current assets	(8,529 )	(632 )
Prepaid and other long-term assets	4,797	1,046
Accounts payable	11,469	(5,123 )
Accrued compensation and employee benefits	(6,413 )	(1,407 )
Accrued research and development	(22,868 )	(12,649 )
Accrued and other liabilities	2,089	1,587
Deferred revenue	1,650	(5,128 )
Other long-term liabilities	(230 )	(205 )
Net cash used in operating activities	(88,420 )	(51,536 )
Investing activities		
Purchases of property and equipment	(512 )	(113 )
Purchases of investments	(78,048 )	(147,781 )
Proceeds from maturities of investments	148,141	95,304
Net cash provided by (used in) investing activities	69,581	(52,590 )
Financing activities		
Proceeds from debt issuance, net	—	48,000
Debt issuance costs paid	—	(536 )
Proceeds from issuance of common stock pursuant to equity award plans	5,679	3,847
Dividends to Noncontrolling interest (SRX Cardio)'s shareholders	(120 )	—
Net cash provided by financing activities	5,559	51,311
Net decrease in cash, cash equivalents and restricted cash	(13,280 )	(52,815 )

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Cash, cash equivalents and restricted cash at beginning of period	181,741	188,658
Cash, cash equivalents and restricted cash at end of period	\$168,461	\$135,843

See accompanying notes to the unaudited condensed consolidated financial statements.

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PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Consolidated Financial Statements

(Unaudited)

1. Organization

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

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. Our two U.S. Food and Drug Administration (“FDA”)-approved medicines are Bevyxxa<sup>®</sup> (betrixaban), the first and only oral, once-daily Factor Xa inhibitor, and Andexxa<sup>®</sup> [coagulation factor Xa (recombinant), inactivated-zhzo], the first and only antidote for the Factor Xa inhibitors rivaroxaban and apixaban. We received approval for Bevyxxa and Andexxa in June 2017 and May 2018, respectively. We are also advancing cerdulatinib, a spleen tyrosine kinase, or Syk, and Janus kinases, or JAK, inhibitor in development to treat hematologic cancers and have a partnered program, which is focused on developing selective Syk inhibitors for inflammatory conditions.

We refer to our two approved drugs in this report as Andexxa and Bevyxxa. If approved in the EU or other parts of the world we expect each drug may be marketed under different brand names. In addition, an international nonproprietary name (“INN”) has been designated for each drug. Our previous INN for Andexxa was andexanet alfa; however, in the United States this INN has been replaced with “coagulation factor Xa (recombinant), inactivated-zhzo.” For the EU and other parts of the world, andexanet alfa could remain the INN for Andexxa. Our use of Andexxa or Bevyxxa in this document in the context of continued development activities for which we have not yet received regulatory approval should not be read to imply that we have received regulatory approval for any indication or in any jurisdiction not reflected in our product labels.

2. Summary of Significant Accounting Policies

Consolidation and Basis of Presentation

The accompanying unaudited condensed consolidated financial statements include the amounts of Portola and its wholly-owned subsidiaries and a development partner that is a variable interest entity (a “VIE”) for which Portola is deemed, under applicable accounting guidance, to be the primary beneficiary as of March 31, 2018. The unaudited condensed consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles (“U.S. GAAP”), and follow the requirements of the Securities and Exchange Commission (“SEC”) for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by U.S. GAAP can be condensed or omitted. These condensed consolidated financial statements have been prepared on the same basis as our annual consolidated financial statements and, in the opinion of management, reflect

all adjustments, consisting only of normal recurring adjustments that are necessary for a fair statement of our financial information.

The results of operations for the three months ended March 31, 2018 are not necessarily indicative of the results to be expected for the year ending December 31, 2018 or for any other interim period or for any other future year. The condensed consolidated balance sheet as of December 31, 2017 has been derived from the audited consolidated financial statements at that date but does not include all of the information required by U.S. GAAP for complete financial statements.

The accompanying unaudited condensed consolidated financial statements and related financial information should be read in conjunction with the audited consolidated financial statements and the related notes thereto for the year ended December 31, 2017 included in our Annual Report on Form 10-K filed on March 1, 2018 with the SEC.

#### Reclassification

Certain prior period amounts on the accompanying condensed consolidated financial statements have been reclassified to conform to current period presentation. This reclassification did not have an impact on our results of operations or financial condition as of December 31, 2017.

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PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Consolidated Financial Statements

(Unaudited)

Use of Estimates

The preparation of condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities, disclosure of contingent liabilities and the reported amounts of revenues and expenses in the condensed consolidated financial statements and the accompanying notes. On an ongoing basis, management evaluates its estimates, including those related to revenue recognition, inventory, clinical trial accruals, fair value of assets and liabilities, income taxes, in-process research and development, carrying value of notes payable and long term debt, the consolidation of VIEs and deconsolidation of VIEs, 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.

Variable Interest Entities

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

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

Intangible Assets

Intangible assets include an in-process research and development asset related to our consolidated VIE and a milestone payment made to Millennium Pharmaceuticals, Inc. (“Millennium”) upon FDA approval of Bevyxxa.

The in-process research and development asset is considered to be indefinite-lived until the completion or abandonment of the associated research and development efforts. If the project is completed, which generally occurs if and when regulatory approval to market a product is obtained, the carrying value of the related intangible asset is amortized as a part of cost of sales over the remaining estimated life of the asset beginning in the period in which the project is completed. If the asset becomes impaired or is abandoned, the carrying value of the related intangible asset is written down to its fair value and an impairment charge is taken in the period in which the impairment occurs. The in-process research and development asset is tested for impairment on an annual basis, and more frequently if indicators are present or changes in circumstances suggest that impairment may exist. Please refer to Note 8, “Asset Acquisition and License Agreement” for further information.

A milestone payment made pursuant to the regulatory approval of Bevyxxa in the United States is considered to be finite-lived and will be amortized on a straight-line basis over the remaining estimated patent life. The intangible asset with a finite useful life is reviewed for impairment when facts or circumstances suggest that the carrying value of the

asset may not be recoverable.

#### 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.

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## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Consolidated Financial Statements

(Unaudited)

## Cash as Reported in Condensed Consolidated Statements of Cash Flows

Cash as reported in the condensed consolidated statements of cash flows includes the aggregate amounts of cash and cash equivalents and the restricted cash, and consists of following (in thousands):

	March 31, 2018	December 31, 2017	March 31, 2017	December 31, 2016
Cash and cash equivalents	\$ 168,414	\$ 181,568	\$ 135,669	\$ 188,480
Restricted cash (SRX Cardio)	47	173	174	178
Total cash balance in condensed consolidated statements of cash flows	\$ 168,461	\$ 181,741	\$ 135,843	\$ 188,658

## Inventories

Inventories are stated at the lower of cost or estimated net realizable value, on a first-in, first-out, or FIFO, basis. We primarily use actual costs to determine our cost basis for inventories.

Prior to the regulatory approval of our product candidates, we incur expenses for the manufacture of drug product that could potentially be available to support the commercial launch of our products. Until the first reporting period when regulatory approval has been received, we record all such costs as research and development expense. Beginning in the fourth quarter of 2017, we began to capitalize inventory costs associated with Bevyxxa when it was determined that the inventory had a probable future economic benefit. We periodically analyze our inventory levels, and write down inventory that has become obsolete, inventory that has a cost basis in excess of its estimated realizable value and inventory in excess of expected sales requirements as cost of sales. No such write downs have been recorded to date.

The active pharmaceutical ingredient (“API”) in Bevyxxa is currently produced by a single supplier. As the API has undergone significant manufacturing specific to its intended purpose at the point it is purchased by us, we classify the API as work-in-process inventory.

## Investments in Marketable Securities

All investments in marketable securities 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 our 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 (loss). Realized gains and losses and declines in fair value judged to be other than temporary, if any, on available-for-sale securities are included in interest and other income, net. The cost of securities sold is based on the specific-identification method. Interest on marketable securities is included in interest and other income, net.



#### Fair Value Measurements

Fair value accounting is applied for all financial assets and liabilities and non-financial assets and liabilities that are recognized or disclosed at fair value in the financial statements on a recurring basis.

#### Property and Equipment

Property and equipment are stated at cost and depreciated using the straight-line method over the estimated useful lives of the assets, ranging from two to five years. Leasehold improvements are amortized over the shorter of their estimated useful lives or the related lease term.

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PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Consolidated Financial Statements

(Unaudited)

Impairment of Long-Lived Assets

We review long-lived assets for impairment whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Specific potential indicators of impairment include a significant decrease in the fair value of an asset, a significant change in the extent or manner in which an asset is used or a significant physical change in an asset, a significant adverse change in legal factors or in the business climate that affects the value of an asset, an adverse action or assessment by the FDA or another regulator or a projection or forecast that demonstrates continuing losses associated with an income producing asset. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of the asset and its eventual disposition are less than its carrying amount. Impairment, if any, is assessed using discounted cash flows or other appropriate measures of fair value. Through March 31, 2018, there have been no such losses.

Deferred Rent

We recognize rent expense on a straight-line basis over the noncancelable term of our operating lease and, accordingly, record the difference between cash rent payments and the recognition of rent expense as a deferred rent liability. We also record lessor-funded lease incentives, such as reimbursable leasehold improvements, as a deferred rent liability, which is amortized as a reduction of rent expense over the noncancelable term of our operating lease.

Concentration of Risk

Financial instruments that potentially subject us to concentrations of credit risk consist of cash, cash equivalents, receivables related to our product revenue and collaborations, and investments. Our investment policy limits investments to certain types of debt securities issued by the U.S. government, its agencies and institutions with investment-grade credit ratings and places restrictions on maturities and concentration by type and issuer. We are exposed to credit risk in the event of a default by the financial institutions holding our cash, cash equivalents and investments and issuers of investments to the extent recorded on the condensed consolidated balance sheets.

Receivables related to our product revenue and collaborations are typically unsecured and are concentrated in the pharmaceutical industry. Accordingly, we may be exposed to credit risk generally associated with pharmaceutical companies or specific to our collaboration agreements. To date, we have not experienced any losses related to these receivables.

Collaboration Customer Concentration

Collaboration customers who accounted for 10% or more of total revenues were as follows:

Three  
Months  
Ended  
March 31,

	2018	2017
Bayer Pharma, AG and Janssen Pharmaceuticals, Inc.	47%	16%
Daiichi Sankyo, Inc.	27%	43%
Bristol-Myers Squibb Company and Pfizer Inc.	17%	34%

#### Revenue Recognition

On January 1, 2018, we adopted Accounting Standards Codification, or ASC, Topic 606 (ASC 606), Revenue from Contracts with Customers, using the modified retrospective method to all contracts that were not completed as of January 1, 2018. We recognized the cumulative effect of applying the new revenue standard as an adjustment to the opening balance of accumulated deficit at the beginning of 2018. The results for our reporting period beginning after January 1, 2018 are presented under ASC 606, while prior period amounts are not adjusted and continue to be reported under the accounting standards in effect for the prior period.

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PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Consolidated Financial Statements

(Unaudited)

Pursuant to ASC 606, we recognize revenue when our customer obtains control of promised goods or services, in an amount that reflects the consideration that we expect to receive in exchange for those goods or services. To determine revenue recognition for arrangements that we determine are within the scope of ASC 606, we perform the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) we satisfy a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract and determine those that are performance obligations, and assess whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Product Revenue, Net

To date, our only source of product revenue has been from the U.S. sales of Bevyxxa, which we began shipping to customers in January 2018. We sell Bevyxxa to a limited number of wholesalers in the United States ("Customers"). These Customers subsequently resell our products to hospitals, pharmacies and long-term care centers. In addition to distribution agreements with Customers, we enter into arrangements with group purchasing organizations and payors that provide for privately negotiated rebates, chargebacks, and discounts with respect to the purchase of our products.

We recognize revenue on product sales when the Customer obtains control of our product, which occurs at a point in time (upon delivery). Product revenues are recorded net of applicable reserves for variable consideration, including discounts and allowances. We expense incremental costs of obtaining a contract when incurred, if the expected amortization period of the asset that we would have recognized is one year or less. To date, we have not incurred such costs.

Reserves for Variable Consideration

Revenues from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established and which result from discounts, returns, chargebacks, rebates, copay assistance and other allowances that are offered within contracts between us and our Customers, group purchasing organizations, payors and other indirect customers relating to our product sales. These reserves as detailed below are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if the amount is payable to the Customer) or a current liability (if the amount is payable to a party other than a Customer). Where appropriate, these estimates take into consideration a range of possible outcomes that are probability-weighted in accordance with the expected value method in ASC 606 for relevant factors such as current contractual and statutory requirements, specific known market events and trends, industry data, and/or forecasted customer buying and payment patterns. Overall, these reserves reflect our best estimates of the amount of

consideration to which we are entitled based on the terms of the respective underlying contracts.

The amount of variable consideration that is included in the transaction price may be constrained, and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

**Trade Discounts and Allowances:** We generally provide Customers with discounts which include incentive fees that are explicitly stated in our contracts and are recorded as a reduction of revenue in the period the related product revenue is recognized. In addition, we compensate our Customers and indirect customers for sales order management, data, administrative and distribution services. However, we have determined such services received to date are not distinct from our sale of products to the Customer and may not reasonably represent fair value for these services, therefore, these payments have been recorded as a reduction of revenue within the condensed consolidated statement of operations for the three months ended March 31, 2018.

**Product Returns:** We generally offer Customers a right of return based on the product's expiration date or other market-based factors for product that has been purchased from us. We estimate the amount of our product sales that may be returned by our Customers and record this estimate as a reduction of revenue in the period the related product revenue is recognized. We currently estimate product return liabilities using available industry data, our own sales information and our visibility into the inventory remaining in the distribution channel. We have not received any returns to date and estimate that product returns in future periods will be minimal.

PORTOLA PHARMACEUTICALS, INC.

Notes to Condensed Consolidated Financial Statements

(Unaudited)

**Chargebacks:** Chargebacks are discounts that occur when contracted customers, which currently consist primarily of group purchasing organizations purchase directly from our wholesalers at a discounted price. The specialty wholesalers, in turn, charge us back the difference between the price initially paid by the wholesaler and the discounted price paid to the wholesaler by the healthcare providers. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and receivables. Chargeback amounts are generally determined at the time of resale to the qualified healthcare provider by Customers, and we generally issue credits for such amounts within a few weeks of the Customer's notification to us of the resale. Reserves for chargebacks consist of credits that we expect to issue for units that remain in the distribution channel inventories at each reporting period end that we expect will be sold to qualified healthcare providers, and chargebacks that Customers have claimed but for which we have not yet issued a credit.

**Payor Rebates:** We contract with various private payor organizations, primarily insurance companies and pharmacy benefit managers, for the payment of rebates with respect to utilization of our products. We estimate these rebates and record such estimates in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability.

#### Collaboration and License Revenue

We enter into collaboration and license agreements for the development and commercialization of our products that are within the scope of ASC 606. The terms of collaboration and license agreements typically include payments to us of one or more of the following: non-refundable or partially refundable upfront or license fees; development, regulatory and commercial milestone payments; manufacturing supply services; partial or complete reimbursement of research and development costs; and royalties on net sales of licensed products. Each of these payments results in collaboration and license revenue, except for royalties on net sales of licensed products, which are classified as royalty revenues.

In determining the appropriate amount of revenue to be recognized as we fulfill our obligations under each of its agreements, we perform the following steps: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) we satisfy each performance obligation. As part of the accounting for these arrangements, we must apply judgment to determine whether the performance obligations are distinct, and develop assumptions in determining the stand-alone selling price for each distinct performance obligation identified in the contract. To determine the stand-alone selling price, we rely on assumptions which may include forecasted revenues, development timelines, reimbursement rates for personnel costs, discount rates and probabilities of technical and regulatory success.

**Licenses of Intellectual Property:** If the license to our intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, we recognize revenues from nonrefundable, up-front fees

allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, we utilize judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue from non-refundable, up-front fees. We evaluate the measure of progress each reporting period and, if necessary, adjust the measure of performance and related revenue recognition.

**Milestone Payments:** At the inception of each arrangement that includes development milestone payments, we evaluate whether the milestones are considered probable of being reached and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within our control or that of the licensee, such as regulatory approvals, are constrained until those approvals are received. The transaction price is then allocated to each performance obligation on a relative stand-alone selling price basis, for which we recognize revenue as or when the performance obligations under the contract are satisfied. At the end of each subsequent reporting period, we re-evaluate the probability of achievement of such development milestones and any related constraint, and if necessary, adjust our estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration and license revenue in the period of adjustment.

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**Manufacturing Supply Services:** Arrangements that include a promise for future supply of drug substance or drug product for either clinical development or commercial supply at the licensee's discretion are generally considered as options. We assess whether these options provide a material right to the licensee and if so, they are accounted for as separate performance obligations. If we are entitled to additional payments when the licensee exercises these options, any additional payments are recorded in collaboration and license revenue when the licensee obtains control of the goods, which is upon delivery.

**Royalties:** For arrangements that include sales-based royalties, including milestone payments based on the level of sales, and the license is deemed to be the predominant item to which the royalties relate, we recognize revenue at the later of (i) when the related sales occur, or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any of our out-licensing arrangements.

**Research and Development Activities:** Amounts related to research and development and regulatory activities 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.

We receive payments from our collaborators based on billing schedules established in each contract. Upfront payments and fees are recorded as deferred revenue upon receipt or when due, and may require deferral of revenue recognition to a future period until we perform our obligations under these arrangements. Amounts are recorded as accounts receivable when our right to consideration is unconditional. We do not assess whether a contract has a significant financing component if the expectation at contract inception is such that the period between payment by the collaborators and the transfer of the promised goods or services to the collaborators will be one year or less.

#### Cost of Sales – Intangible Asset Amortization

Cost of sales for intangible asset amortization consists of the amortization of a capitalized milestone payment made to Millennium upon FDA approval of Bevyxxa. The milestone payment is amortized on a straight-line basis over the estimated remaining patent life of Bevyxxa.

#### Research and Development

Research and development costs are expensed as incurred and consist of salaries and benefits, lab supplies, materials and facility costs, as well as fees paid to nonemployees and entities that conduct certain research and development and manufacturing activities on our behalf. Amounts incurred in connection with collaboration and license agreements are also included in research and development expense. Payments made prior to the receipt of goods or services to be used in research and development are capitalized until the goods are received or services are rendered.

#### Clinical Trial Accruals

Clinical trial costs are a component of research and development expenses. We accrue and expense clinical trial activities performed by third parties based upon actual work completed in accordance with agreements established



with clinical research organizations and clinical sites. We determine the actual costs through monitoring patient enrollment and discussions with internal personnel and external service providers as to the progress or stage of completion of trials or services and the agreed-upon fee to be paid for such services. We have not experienced any material deviations between the accrued clinical trial expenses and actual clinical trial expenses. However, actual services performed, number of patients enrolled and the rate of patient enrollment may vary from our estimates, resulting in adjustments to clinical trial expense in futures periods.

We also accrue costs for manufacturing activities performed by third parties based upon actual work completed in accordance with our manufacturing agreements.

#### Stock-Based Compensation

Employee stock-based compensation cost is measured at the grant date, based on the fair value of the award. The compensation cost is recognized as expense on a straight-line basis over the vesting period for options and restricted stock units (“RSUs”) and on an accelerated basis for performance stock options (“PSOs”), market-based performance stock units (“M-PSUs”) and performance-based

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stock units (“PSUs”). For stock option grants including PSOs, we use the Black-Scholes option pricing model to determine the fair value of stock options. This model requires us to make assumptions such as expected term and volatility that determine the stock options fair value. We are also required to make estimates as to the probability of achieving the specific performance criteria underlying the PSOs and PSUs. For M-PSUs, we use the Monte-Carlo option pricing model to determine the fair value of awards at the date of issue. The Monte-Carlo option-pricing model uses similar input assumptions as the Black-Scholes model; however, it further incorporates into the fair-value determination the possibility that the performance-based market condition may not be satisfied. Compensation costs related to awards with a market-based condition are recognized regardless of whether the market condition is ultimately satisfied. Compensation cost is not reversed if the achievement of the market condition does not occur. For RSUs and PSUs, we base the fair value of awards on the closing market value of our common stock at the date of grant.

Equity instruments issued to nonemployees, consisting of stock options granted to consultants and restricted stock units and performance stock units granted to employees that have converted to nonemployees, are valued using the Black-Scholes option-pricing model for stock options and period-end market price for restricted stock units and performance stock units. Stock-based compensation expense for nonemployee services is subject to remeasurement as the underlying equity instruments vest and is recognized as an expense over the period during which services are received.

#### Interest Expense

Notes payable and long-term debt are eligible to be repaid based on royalties from our soon to be marketed product, Andexxa. The recognition of interest expense requires us to estimate the total amount of future royalty payments to be generated from product sales by jurisdiction over the life of the agreement. Consequently, we impute interest on the carrying value of the notes payable and long-term debt and record interest expense using an imputed effective interest rate. We reassess the expected royalty payments each reporting period and account for any changes through an adjustment to the effective interest rate on a prospective basis, with a corresponding impact to the classification of our debt and note payable liabilities. The assumptions used in determining the expected repayment term of the debt and amortization period of the issuance costs requires that we make estimates that could impact the short and long term classification of these costs, as well as the period over which these costs will be amortized.

#### Income Taxes

We provide for income taxes under the asset and liability method. Current income tax expense or benefit represents the amount of income taxes expected to be payable or refundable for the current year. Deferred income tax assets and liabilities are determined based on differences between the condensed consolidated financial statement reporting and tax basis of assets and liabilities and net operating loss and credit carryforwards, and are measured using the enacted tax rates and laws that will be in effect when such items are expected to reverse. Deferred income tax assets are reduced, as necessary, by a valuation allowance when management determines it is more likely than not that some or all of the tax benefits will not be realized. The recognition, derecognition and measurement of a tax position is based on management’s best judgment given the facts, circumstances and information available at the reporting date. Our

policy is to recognize interest and penalties related to the underpayment of income taxes as a component of income tax expense or benefit. To date, there have been no interest or penalties charged in relation to the underpayment of income taxes.

#### Foreign Currency Transactions

We have financial transactions denominated in foreign currencies, primarily the Euro and British Pound, and, as a result, are exposed to changes in foreign currency exchange rates.

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

Basic net loss per share attributable to Portola Common Stockholders is calculated by dividing the net loss attributable to Portola Common Stockholders by the weighted-average number of shares of Common Stock outstanding for the period. Diluted net loss per share attributable to Portola Common Stockholders is the same as basic net loss per share attributable to Portola Common Stockholders, since the effects of potentially dilutive securities are antidilutive.

#### Recent Accounting Pronouncements Not Adopted

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In February 2016, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update (“ASU”) No. 2016-02, Leases (Topic 842), which amends the existing accounting standards for leases. The new standard requires lessees to record a right-of-use asset and a corresponding lease liability on the balance sheet (with the exception of short-term leases). For lessees, leases will continue to be classified as either operating or financing in the income statement. This ASU becomes effective in the first quarter of fiscal year 2019 and early adoption is permitted. This ASU is required to be applied with a modified retrospective approach and requires application of the new standard at the beginning of the earliest comparative period presented. We are currently evaluating the impact that ASU 2016-02 will have on our condensed consolidated financial statements.

Recent Accounting Pronouncements Adopted

In May 2017, the FASB issued ASU No. 2017-09, Compensation-Stock Compensation (Topic 718): Scope of Modification Accounting. This ASU includes guidance on determining which changes to the terms and conditions of share-based payment awards require an entity to apply modification accounting under Topic 718. This guidance is effective for annual and interim periods of public entities beginning after December 15, 2017, with early adoption permitted. We adopted this guidance on January 1, 2018, and the adoption had no impact on our condensed consolidated financial statements.

In November 2016, the FASB issued ASU No. 2016-18, Statement of Cash Flows: Restricted Cash. This ASU requires changes in restricted cash during the period to be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. If cash, cash equivalents and restricted cash are presented in more than one line item on the balance sheet, the new guidance requires a reconciliation of the total in the statement of cash flows to the related captions in the balance sheet. This guidance is effective for annual and interim periods of public entities beginning after December 15, 2017, with early adoption permitted. The amendments in this ASU should be applied retrospectively to all periods presented. We adopted this guidance on January 1, 2018. The adoption of this ASU increased our beginning and ending cash balances within our condensed consolidated statements of cash flows. The adoption had no other material impacts to our condensed consolidated statements of cash flows and had no impact on our results of operations or financial position.

In August 2016, the FASB issued ASU No. 2016-15, Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments. The pronouncement addresses the presentation of certain items on the statement of cash flows including among other things settlement of zero coupon debt instruments or other debt instruments with coupon interest rates that are insignificant to the effective interest rate of the borrowing. Pursuant to the new guidance, at the settlement of our promissory notes to Bristol-Myers Squibb Company (“BMS”) and Pfizer Inc. (“Pfizer”) and funding received from HealthCare Royalty Partners and its Affiliates, we should classify the portion of the cash payment attributable to the accreted interest related to the debt discount as cash outflows for operating activities, and the

portion of the cash payment attributable to the principal as cash outflows for financing activities. This guidance is effective for annual and interim periods of public entities beginning after December 15, 2017, with early adoption permitted. We adopted this guidance on January 1, 2018, and the adoption had no impact on our condensed consolidated financial statements for the period ended March 31, 2018.

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In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which amends the existing accounting standards for revenue recognition. We adopted the new revenue standard effective January 1, 2018 using the modified retrospective method to all contracts that were not completed as of January 1, 2018. The cumulative effect of applying the new guidance was recorded as an adjustment to accumulated deficit as of the adoption date. As a result, the following adjustments were made to the condensed consolidated balance sheet as of January 1, 2018:

	As of January 1, 2018		
	As revised under	As originally reported	Effect of change
	ASC 606		
<b>Assets:</b>			
Unbilled - collaboration and license revenue	\$6,694	\$—	\$6,694
Trade and other receivables, net	2,706	—	2,706
Prepaid expenses and other current assets	—	2,706	(2,706 )
<b>Liabilities:</b>			
Deferred revenue, current portion	6,354	11,169	(4,815 )
Deferred revenue, long-term	1,269	18,798	(17,529)
<b>Stockholders' equity:</b>			
Accumulated deficit	\$(1,175,481)	\$(1,204,519)	\$29,038

The following table compares the reported condensed consolidated balance sheet and statement of operations information to the balances that do not reflect the adoption of ASC 606 as of and for the three months ended March 31, 2018:

	As of March 31, 2018		
	As reported	Balances without the adoption of ASC 606	Effect of change
<b>Assets:</b>			
Unbilled - collaboration and license revenue	\$4,660	\$—	\$4,660
Trade and other receivables, net	968	—	968
Prepaid expenses and other current assets	—	968	(968 )
<b>Liabilities:</b>			
Deferred revenue, current portion	3,861	9,547	(5,686 )
Deferred revenue, long-term	5,412	23,064	(17,652)

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Stockholders' equity:

Accumulated deficit	(1,259,660)	(1,287,659)	27,999
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	Three Months Ended March 31, 2018		
	As reported	Balances without the adoption of ASC 606	Effect of change
<b>Revenue:</b>			
Collaboration and license revenue	\$6,038	\$6,354	\$(316 )
<b>Operating expenses:</b>			
Research and development	60,067	59,345	722
Loss from operations	(85,300)	(84,262)	(1,038)
Net loss	(84,510)	(83,472)	(1,038)
Net loss attributable to Portola	(84,178)	(83,140)	(1,038)
Net loss per share attributable to Portola common stockholders: Basic and diluted	\$(1.28 )	\$(1.27 )	\$(0.01 )

Our financial position with respect to product revenues would not have been materially different without the adoption of ASC 606, however, we would have deferred revenue recognition under ASC Topic 605 until product sold through to the end customer.

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## 3. Revenue Recognition

Revenues are recognized when control of the promised goods or services is transferred to our customers, in an amount that reflects the consideration we expect to be entitled to in exchange for those goods or services.

The following table presents our revenues, disaggregated by timing of transfer of goods or services (in thousands):

	Three Months Ended March 31, 2018		
	Product Revenue, net	Collaboration and License Revenue	Total
Timing of revenue recognition:			
Transferred at a point in time	\$ 606	\$ —	\$ 606
Transferred over time	—	6,038	6,038
Total	\$ 606	\$ 6,038	\$ 6,644

The following table presents changes in our contract assets and liabilities for the three months ended March 31, 2018 (in thousands):

	Balance at Beginning of Period	Addition	Deduction	Balance at End of Period
Contract assets:				
Unbilled - collaboration and license revenue	\$ 6,694	\$ 2,761	\$ (4,795 )	\$ 4,660
Total contract assets	\$ 6,694	\$ 2,761	\$ (4,795 )	\$ 4,660
Contract liabilities:				
Deferred revenue	\$ 7,623	\$ 5,173	\$ (3,523 )	\$ 9,273
Total contract liabilities	\$ 7,623	\$ 5,173	\$ (3,523 )	\$ 9,273



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During the three months ended March 31, 2018, we recognized \$1.8 million in collaboration and license revenue as a cumulative catch-up adjustment related to a change in our estimated transaction price. Additionally, \$3.5 million of collaboration and license revenue was recognized during the three months ended March 31, 2018 related to amounts included in the beginning contract liability balance.

The following table includes estimated revenue expected to be recognized in the future related to performance obligations that are unsatisfied or partially unsatisfied as of March 31, 2018 (in thousands):

Collaborator	Transaction Price Allocated to the Remaining Performance Obligation as of March 31, 2018	Expected Year	By Which Revenue Recognition Will Be Completed	Percentage of Revenue Recognized	
BMS and Pfizer - 2014 agreement	\$ 909	2019		94	%
BMS and Pfizer - 2016 agreement	4,131	2021		73	%
Daiichi Sankyo - 2014 agreement	3,173	2019		91	%
Daiichi Sankyo - 2016 agreement	3,818	2021		69	%
Bayer and Janssen - 2014 agreement	1,420	2019		94	%
Bayer - 2016 agreement	3,389	2021		73	%
Total	\$ 16,840				

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Milestone payments that are not considered probable of being achieved are excluded from the transaction price until they are probable.

Sales-based royalties, including milestone payments based on the level of sales, related to license arrangements are excluded from variable consideration and will be recognized at the later of (a) when the related sales occur, or (b) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, we have not recognized any royalty revenue resulting from any of our licensing arrangements.

Product Revenue, Net

To date, our only source of product revenue has been from the U.S. sales of Bevyxxa, which we began shipping to customers in January 2018. No costs to obtain or fulfill the contracts have been capitalized. For the three months ended March 31, 2018 we recorded a total of \$0.7 million as a reduction to trade and other receivables consisting primarily of chargebacks.

Collaboration and License Revenue

BMS and Pfizer

Agreement Terms

In January 2014, we entered into an agreement with BMS and Pfizer to further study Andexxa as a reversal agent for their jointly-owned, FDA-approved oral Factor Xa inhibitor, apixaban, through Phase 3 studies (the “2014 BMS and Pfizer Agreement”). We are responsible for the cost of conducting this clinical study. Pursuant to our agreement with BMS and Pfizer we are obligated to provide research, development and regulatory approval services and participate in the Joint Collaboration Committee (“JCC”) in exchange for a partially refundable upfront fee of \$13.0 million and up to \$12.0 million of contingent milestone payments due upon achievement of certain development and regulatory events. All consideration received and to be earned under this agreement is subject to a 50% refund contingent upon certain regulatory and/or clinical events.

In February 2016, we entered into a collaboration and license agreement with BMS and Pfizer whereby BMS and Pfizer obtained exclusive rights to develop and commercialize Andexxa in Japan (the “2016 BMS and Pfizer Agreement”). BMS and Pfizer are responsible for all development, regulatory and commercial activities in Japan and we will reimburse BMS and Pfizer for expenses they incur for research and development activities specific to Factor Xa inhibitors other than apixaban. Pursuant to this agreement, we are obligated to provide certain research and development activities outside of Japan, provide clinical drug supply and related manufacturing services and to participate on various committees in exchange for a non-refundable upfront fee of \$15.0 million. We are also eligible to receive, contingent payments totaling up to \$20.0 million which may be earned upon achievement of certain regulatory events and up to \$70.0 million which may be earned upon achievement of specified annual net sales volumes in Japan. We are also entitled to receive royalties ranging from 5% to 15% on net sales of Andexxa in Japan.

## Revenue Recognition

We assessed the 2014 BMS and Pfizer Agreement and the 2016 BMS and Pfizer Agreement in accordance with ASC 606 and concluded that BMS and Pfizer are customers.

We identified the following performance obligations under the 2014 BMS and Pfizer Agreement: (1) to provide research, development and regulatory services, and (2) to provide manufacturing and supply services. We determined that the research, development and regulatory services can only provide benefit to BMS and Pfizer in combination with the manufacture and supply of Andexxa and because the manufacturing know-how is proprietary to us and cannot be provided by other vendors, the services do not qualify as distinct performance obligations. As the manufacturing and supply services are a required input to the research, development and regulatory services, we have combined all activities into a single performance obligation. The nature of the combined performance obligation is to provide research, development and regulatory services necessary to obtain approval of Andexxa as a reversal agent to apixaban in both the United States and Europe.

For revenue recognition purposes, we determined that the duration of the contract began on the effective date in January 2014 and ends upon Andexxa approval in United States and Europe, expected to be achieved by the first quarter of 2019. The contract duration is defined as the period in which parties to the contract have present enforceable rights and obligations. We analyzed the impact of BMS and Pfizer terminating the agreement prior to Andexxa approval and determined that there were substantive non-monetary penalties to BMS and Pfizer for doing so. We considered quantitative and qualitative factors to reach this conclusion.

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We determined that the transaction price of the 2014 BMS and Pfizer Agreement was \$16.5 million as of March 31, 2018. In order to determine the transaction price, we evaluated all the payments to be received during the duration of the contract and whether the 50% refundable feature associated with such payments was probable of occurrence. We have concluded that no portion of the cash receipts should be constrained related to the refund provision because the activities that would trigger a refund are under our control and considered to be remote. As of March 31, 2018, there are no additional payments eligible to be earned.

We are utilizing a cost-based input method to measure proportional performance and to calculate the corresponding amount of revenue to recognize. We believe this is the best measure of progress because other measures do not reflect how we transfer the performance obligation to our counter party. In applying the cost based input methods of revenue recognition, we use actual costs incurred relative to budgeted costs to fulfill the combined performance obligation. These costs consist primarily of third-party contract costs and internal full-time equivalent effort. A cost-based input method of revenue recognition requires us to make estimates of costs to complete the performance obligations. The cumulative effect of revisions to estimated costs to complete the performance obligations will be recorded in the period in which changes are identified and amounts can be reasonably estimated. A significant change in these assumptions and estimates could have a material impact on the timing and amount of revenue recognized in future periods.

For the three months ended March 31, 2018, we have recognized \$0.6 million as license and collaboration revenue under the 2014 BMS and Pfizer Agreement and \$0.9 million is recorded in deferred revenue under contract liabilities as of March 31, 2018 on the condensed consolidated balance sheets.

There were no costs incurred to obtain or fulfill the contract.

We identified the following performance obligations under the 2016 BMS and Pfizer Agreement: 1) grant of intellectual property license in Japan, 2) provide research and development services, and 3) provide manufacturing services and supply Andexxa for development and commercial purposes. Because the Andexxa program had already progressed into a late-phase of development at inception of the 2016 BMS and Pfizer Agreement, we concluded that the Japan license has standalone functionality and is capable of being distinct. However, we determined that the license is not distinct from the other obligations within the context of the agreement because the research and development services and manufacture and supply services are necessary to increase the utility of the intellectual property and the performance of such services requires our unique expertise and experience. Accordingly, we have concluded that research and development services and manufacturing and supply services are not distinct from the license within the context of the contract and therefore the license, research and development services, manufacture and supply services are combined into a single performance obligation.

In addition, we have identified the following customer options that will create a manufacturing obligation for us upon exercise by BMS and Pfizer: 1) commercial supply of Andexxa for sale in Japan and 2) BMS and Pfizer's participation in manufacturing capacity expansion. We considered the status of Andexxa approval in the United States and Europe and its impact on Japan, Andexxa's manufacturing complexities, Andexxa's expansion plan with our existing vendors and BMS and Pfizer's manufacturing capabilities to determine if these options constituted options with material rights. These options are not options with material rights because the \$15.0 million upfront payment received by us was not negotiated to provide incremental discount for the commercial supplies payments and BMS and Pfizer's payment for capacity expansion to be received in the future.

For revenue recognition purposes, we have determined that the duration of the contract begins on the effective date in February 2016 and ends upon completion of the Andexxa phase 4 expansion clinical trial in Japan. The contract duration is defined as the period in which parties to the contract have present enforceable rights and obligations. We analyzed the impact of BMS and Pfizer terminating the agreement prior to the completion of Andexxa phase 4 expansion clinical trial in Japan and determined that there were substantive non-monetary penalties to BMS and Pfizer for doing so. We considered quantitative and qualitative factors to reach this conclusion.

We determined that the transaction price of the 2016 BMS and Pfizer Agreement was \$15.1million as of March 31, 2018. In order to determine the transaction price, we evaluated all the payments to be received during the duration of the contract. As of March 31, 2018, transaction price includes, \$15.0 million of upfront payment, \$5.0 million for acceptance of the Japan New Drug Application ("JNDA") in Japan, as management expects it to be probable of achievement, \$3.3 million of estimated variable consideration for cost-sharing payments from BMS and Pfizer for agreed upon research and development services for clinical trials outside of Japan, \$0.2 million for the estimated costs of Andexxa clinical supplies to BMS and Pfizer for Andexxa phase 4 expansion clinical trial in Japan, and the transaction price is reduced by \$8.4 million payable to BMS and Pfizer for estimated costs they will incur in developing Andexxa in Japan. Regulatory approval milestones were fully constrained and therefore are not included in the transaction price, as the receipts of such milestones are outside of our control. In determining whether to constrain other milestones, we considered numerous factors, including whether receipt of the milestones is within our control, contingent upon success in future clinical trials

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and/or the licensee's efforts. Any consideration related to sales-based milestones (including royalties) will be recognized when the related sales occur as they were determined to relate predominantly to the license granted to BMS and Pfizer and therefore have also been excluded from the transaction price. We will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

We are responsible to perform certain clinical trials outside of Japan and BMS and Pfizer are responsible to perform research and development services in Japan. Outside of Japan, we are primarily responsible to perform an ethnic sensitivity study ("ESS-Study") of Japanese ethnicity. BMS and Pfizer are responsible to expand our current phase 3/4 clinical trial of Andexxa into Japan and to perform any further studies requested by the Japanese regulatory authorities. BMS and Pfizer will reimburse us for 33% of our costs and expenses incurred to conduct the ESS-Study and we will reimburse 66% of costs and expenses incurred by BMS and Pfizer related to research and development services in Japan specific to Factor Xa inhibitors other than apixaban under the terms of the arrangement.

All parties to this agreement will make quarterly cost-sharing payments to one another in amounts necessary to ensure that each party bears its contractual share of the overall shared costs incurred. We account for cost-sharing payments received from BMS and Pfizer as increases to our transaction price while cost-sharing payments we make to BMS and Pfizer are accounted for as reductions to our transaction price. Costs incurred by us related to agreed upon services under the agreement are recorded as research and development expenses in our consolidated condensed statements of operations.

We are utilizing a cost-based input method to measure proportional performance and to calculate the corresponding amount of revenue to recognize. We believe this is the best measure of progress because other measures do not reflect how we transfer the performance obligation to our counter party. In applying the cost based input methods of revenue recognition, we use actual costs incurred relative to budgeted costs to fulfill the combined performance obligation. These costs consist primarily of third-party contract costs and internal full-time equivalent effort. A cost-based input method of revenue recognition requires management to make estimates of costs to complete the performance obligations. The cumulative effect of revisions to estimated costs to complete the performance obligations will be recorded in the period in which changes are identified and amounts can be reasonably estimated. A significant change in these assumptions and estimates could have a material impact on the timing and amount of revenue recognized in future periods.

For the three months ended March 31, 2018, we have recognized \$0.6 million as license and collaboration revenue under the 2016 BMS and Pfizer Agreement and have \$8.2 million recorded as deferred revenue under contract liabilities as of March 31, 2018 on the condensed consolidated balance sheets.

There were no costs incurred to obtain or fulfill the contract.

Daiichi Sankyo, Inc. (“Daiichi Sankyo”)

#### Agreement Terms

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

In October 2016, we amended this agreement to expedite the expansion of our Phase 4 trial in exchange for an upfront fee of \$15.0 million, \$8.0 million of which is payable back to Daiichi Sankyo based solely on quarterly royalty payments of 1% of world-wide net sales of Andexxa. We are also eligible to receive up to three contingent payments totaling \$10.0 million payable upon achieving specified clinical site activation and patient enrollment targets. Additionally, the \$2.5 million contingent payment associated with scaling up our manufacturing process from the original agreement has been removed by this amendment.

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In March 2016, we entered into an agreement with Daiichi Sankyo to perform an ethnic sensitivity study (“ESS-Study”) of Japanese ethnicity, perform any further studies requested by the Japanese regulatory authorities and to deliver services in connection with our collaboration agreement to commercialize Andexxa in Japan with BMS and Pfizer (the “2014 Daiichi Sankyo Agreement”). Daiichi Sankyo will reimburse us for 33% of our costs and expenses incurred to conduct the ESS-Study and between 33% and 100% of costs and expenses we incur for other studies that involve edoxaban under the terms of the arrangement.

Revenue Recognition

We assessed the 2014 Daiichi Sankyo Agreement as amended in October 2016 and the 2016 Daiichi Sankyo Agreement in accordance with ASC 606 and concluded that the Daiichi Sankyo is a customer.

We concluded that the 2014 Daiichi Sankyo Agreement and the October 2016 amendment are linked and should be accounted for as a combined agreement. We identified the following performance obligations under the combined agreement: (1) to provide research, development and regulatory services, and (2) to provide manufacturing and supply services. We determined that the research, development and regulatory services can only provide benefit to Daiichi Sankyo in combination with the manufacture and supply of Andexxa and because the manufacturing know-how is proprietary to us and cannot be provided by other vendors, the services do not qualify as distinct performance obligations. As the manufacturing and supply services are a required input to the research, development and regulatory services, we have combined all activities into a single performance obligation. The nature of the combined performance obligation is to provide research, development and regulatory services necessary to obtain approval of Andexxa as a reversal agent to edoxaban in both the United States and Europe.

For revenue recognition purposes, we determined that the duration of the contract begins on the effective date in July 2014 and ends upon Andexxa approval in United States and Europe for edoxaban, expected to be achieved in 2019. The contract duration is defined as the period in which parties to the contract have present enforceable rights and obligations. We analyzed the impact of Daiichi Sankyo’s terminating the agreement prior to Andexxa approval and determined that there were substantive non-monetary penalties to Daiichi Sankyo for doing so. We considered quantitative and qualitative factors to reach this conclusion.

We determined that the transaction price of the Daiichi Sankyo combined 2014 Daiichi Sankyo Agreement and October 2016 amendment was \$34.0 million as of March 31, 2018. In order to determine the transaction price, we evaluated all the payments to be received during the duration of the contract. As of March 31, 2018, transaction price includes \$22.0 million of upfront payments, \$9.0 million in milestones already received upon achievement of specified events and a \$3.0 million milestone related to clinical metrics we have determined is probable of achievement. As of March 31, 2018, we have \$5.5 million of further milestone payments eligible to be included in transaction price but have determined they are not probable of achievement and therefore constrained. As part of our



evaluation of the constraint, we considered numerous factors, including whether receipt of the milestones is outside of our control and/or contingent upon success in future clinical trial. We will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

We are utilizing a cost-based input method to measure proportional performance and to calculate the corresponding amount of revenue to recognize. We believe this is the best measure of progress because other measures do not reflect how we transfer the performance obligation to our counter party. In applying the cost based input method of revenue recognition, we use actual costs incurred relative to budgeted costs to fulfill the combined performance obligation. These costs consist primarily of third-party contract costs and internal full-time equivalent effort. A cost-based input method of revenue recognition requires management to make estimates of costs to complete the performance obligations. The cumulative effect of revisions to estimated costs to complete the performance obligations will be recorded in the period in which changes are identified and amounts can be reasonably estimated. A significant change in these assumptions and estimates could have a material impact on the timing and amount of revenue recognized in future periods.

For the three months ended March 31, 2018, we have recognized \$1.3 million as license and collaboration revenue under the combined 2014 Daiichi Sankyo Agreement and October 2016 amendment and have \$0.2 million recorded as deferred revenue under contract liabilities as of March 31, 2018 on the condensed consolidated balance sheets.

There were no costs incurred to obtain or fulfill the contract.

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We identified the following performance obligations under the 2016 Daiichi Sankyo Agreement: 1) the obligation to provide research and development services 2) the obligation to provide regulatory approval services, and 3) the obligation to manufacture and provide clinical supply of Andexxa. We determined that our obligation to provide research and development and regulatory services can only provide benefit to Daiichi Sankyo in combination with our supply of clinical Andexxa for the phase 4 expansion clinical study. The Andexxa manufacturing know-how is specialized and proprietary to us and cannot be provided by other vendors. Therefore, we have concluded that the research, development, regulatory and Andexxa supply services are not distinct within the context of the contract, and thus these obligations are combined into a single performance obligation.

We have exclusive rights to develop Andexxa outside of Japan and are solely responsible for performing such activities, including the ESS-Study, in support of the JNDA. Development activities occurring in Japan, including the expansion of our Phase 4 clinical trial, are the responsibility of BMS and Pfizer, however, the costs of such activities related to Factor Xa inhibitors other than apixaban are borne by us. Pursuant to this agreement, we are responsible to ensure edoxaban is included in all development activities related to Andexxa and Daiichi Sankyo will compensate us accordingly. We account for the expected cost-sharing payments from Daiichi Sankyo as an increase to our transaction price.

We determined that the transaction price of the 2016 Daiichi Sankyo Agreement was \$12.5 million as of March 31, 2018. In order to determine the transaction price, we evaluated all the payments to be received during the duration of the contract. As of March 31, 2018, transaction price includes \$5.0 million of upfront payment and \$3.3 million of estimated variable consideration for cost-sharing payments from Daiichi Sankyo for ESS-study, and \$4.2 million of estimated variable consideration for cost-sharing payments from Daiichi Sankyo associated with the development of Andexxa in Japan. As of March 31, 2018, we have \$10.0 million of further regulatory milestone payments eligible for achievement, however, regulatory milestones have been fully constrained and thus are not included in the transaction price. In determining whether to constrain these milestones, we considered numerous factors, including whether receipt of the milestones is within our control and/or contingent upon success in future clinical trials. We will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

We are utilizing a cost-based input method to measure proportional performance and to calculate the corresponding amount of revenue to recognize. We believe this is the best measure of progress because other measures do not reflect how we transfer the performance obligation to our counter party. In applying the cost based input methods of revenue recognition, we use actual costs incurred relative to budgeted costs to fulfill the combined performance obligation. These costs consist primarily of third-party contract costs and internal full-time equivalent effort. A cost-based input method of revenue recognition requires management to make estimates of costs to complete the performance obligations. The cumulative effect of revisions to estimated costs to complete the performance obligations will be recorded in the period in which changes are identified and amounts can be reasonably estimated. A significant change

in these assumptions and estimates could have a material impact on the timing and amount of revenue recognized in future periods.

For the three months ended March 31, 2018, we have recognized \$0.5 million as license and collaboration revenue under the 2016 Daiichi Sankyo Agreement and have \$1.8 million recorded as Unbilled - collaboration and license revenue as of March 31, 2018 on the condensed consolidated balance sheets.

None of the costs to obtain or fulfill the contract were capitalized.

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

#### Agreement Terms

In January 2014, we entered into an agreement with Bayer and Janssen to study Andexxa as a reversal agent to rivaroxaban in our Phase 3 studies and to seek regulatory approval in the United States and Europe (the “2014 Bayer and Janssen Agreement”). We are responsible for the costs associated with this agreement. We are obligated to provide research, development, manufacturing and regulatory services in exchange for an upfront nonrefundable fee of \$10.0 million, up to three payments totaling \$7.0 million which are payable upon achievement of certain events associated with scaling up our manufacturing process to support a commercial launch, and up to three payments totaling \$8.0 million which are payable upon initiation of our Phase 3 study and regulatory approval of Andexxa as a reversal agent to rivaroxaban in United States and Europe.

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Revenue Recognition

We assessed the 2014 Bayer and Janssen Agreement in accordance with ASC 606 and concluded that Bayer and Janssen are customers.

We identified the following performance obligation under the 2014 Bayer and Janssen Agreement: (1) the obligation to provide research and development services, (2) to provide manufacturing services and to supply Andexxa, and (3) the obligation to provide regulatory approval services. We determined that the research, development and regulatory services can only provide benefit to Bayer and Janssen in combination with the manufacture and supply of Andexxa and because the manufacturing know-how is specialized and proprietary to us and cannot be provided by other vendors, the services do not qualify as distinct performance obligations. As the manufacturing and supply services are a required input to the research, development and regulatory services, we have combined all activities into a single performance obligation. The nature of the combined performance obligation is to provide research, development and regulatory services necessary to obtain approval of Andexxa as a reversal agent to rivaroxaban in both the United States and Europe.

For revenue recognition purposes, we determined that the duration of the contract begins on the effective date in 2014 Bayer and Janssen Agreement ends upon Andexxa approval in the United States and Europe for rivaroxaban, expected to be achieved in 2019. The contract duration is defined as the period in which parties to the contract have present enforceable rights and obligations. We analyzed the impact of Bayer and Janssen terminating the agreement prior to Andexxa approval and determined that there were substantive non-monetary penalties to Bayer and Janssen Pfizer for doing so. We considered quantitative and qualitative factors to reach this conclusion.

We determined that the transaction price of the 2014 Bayer and Janssen Agreement was \$25.0 million as of March 31, 2018. In order to determine the transaction price, we evaluated all the payments to be received during the duration of the contract. As of March 31, 2018, transaction price includes, \$10 million of upfront payment, \$13 million in milestones that have already been achieved and a \$2 million milestone that we deem probable of achievement following the CHMP's positive trend vote and subsequent discussions with the EMA during the quarter ended March 31, 2018. There is no further consideration eligible to be included in transaction price.

We are utilizing a cost-based input method to measure proportional performance and to calculate the corresponding amount of revenue to recognize. We believe this is the best measure of progress because other measures do not reflect how we transfer the performance obligation to our counter party. In applying the cost based input method of revenue recognition, we use actual costs incurred relative to budgeted costs to fulfill the combined performance obligation. These costs consist primarily of third-party contract costs and internal full-time equivalent effort. A cost-based input

method of revenue recognition requires management to make estimates of costs to complete the performance obligations. The cumulative effect of revisions to estimated costs to complete the performance obligations will be recorded in the period in which changes are identified and amounts can be reasonably estimated. A significant change in these assumptions and estimates could have a material impact on the timing and amount of revenue recognized in future periods.

For the three months ended March 31, 2018, we have recognized \$2.7 million as license and collaboration revenue under the 2014 Bayer and Janssen Agreement and have \$0.6 million recorded as Unbilled - collaboration and license revenue as of March 31, 2018 on the condensed consolidated balance sheets.

None of the costs to obtain or fulfill the contract were capitalized.

Bayer Pharma, AG (“Bayer”)

Agreement Terms

In February 2016, we entered into an agreement with Bayer to perform an ESS-Study of Japanese ethnicity, perform any further studies requested by the Japanese regulatory authorities and to deliver services, in connection with our collaboration agreement to commercialize Andexxa in Japan with BMS and Pfizer (the “2016 Bayer Agreement”). Bayer will reimburse us 33% of our costs and expenses incurred to conduct the ESS-Study and between 33% and 100% of costs and expenses we incur for other studies that involve rivaroxaban under the terms of the arrangement.

We are obligated to provide research and development services, provide clinical drug supply and related manufacturing services and to provide regulatory approval services in exchange for an upfront nonrefundable fee of \$5.0 million. We are also eligible to receive,

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one payment of \$10.0 million which is payable upon the initial regulatory approval for Andexxa for rivaroxaban in Japan. The \$10.0 million payment will be reduced to \$7.0 million if Japanese regulatory approval is attained based only upon the ESS study results.

Revenue Recognition

We assessed the 2016 Bayer Agreement in accordance with ASC 606 and concluded that Bayer is a customer.

We identified the following performance obligations under the 2016 Bayer Agreement: 1) the obligation to provide research and development services 2) the obligation to provide regulatory approval services, and 3) the obligation to manufacture and provide clinical supply of Andexxa. We determined that our obligation to provide research and development and regulatory services can only provide benefit to Bayer in combination with our supply of clinical Andexxa for the phase 4 expansion clinical study. The Andexxa manufacturing know-how is specialized and proprietary to us and cannot be provided by other vendors. Therefore, we have concluded that the research, development, regulatory and Andexxa supply services are not distinct within the context of the contract, and thus these obligations are combined into a single performance obligation.

We have exclusive rights to develop Andexxa outside of Japan and are solely responsible for performing such activities, including the ESS-Study, in support of the JNDA. Development activities occurring in Japan, including the expansion of our Phase 4 clinical trial, are the responsibility of BMS and Pfizer, however, the costs of such activities related to Factor Xa inhibitors other than apixaban are borne by us. Pursuant to this agreement, we are responsible to ensure rivaroxaban is included in all development activities related to Andexxa and Bayer will compensate us accordingly. We account for the expected cost-sharing payments from Bayer as an increase to our transaction price.

We determined that the transaction price of the 2016 Bayer Agreement was \$12.5 million as of March 31, 2018. In order to determine the transaction price, we evaluated all the payments to be received during the duration of the contract. As of March 31, 2018, the transaction price includes a \$5.0 million upfront payment, \$3.3 million of estimated variable consideration for cost-sharing payments from Bayer for the ESS-study and \$4.2 million of estimated variable consideration for cost-sharing payments from Bayer associated with the development of Andexxa in Japan. As of March 31, 2018, we have \$10.0 million of further regulatory milestone payments eligible for achievement, however, regulatory milestones have been fully constrained and thus are not included in the transaction price. In determining whether to constrain these milestones, we considered numerous factors, including whether receipt of the milestones is within our control and/or contingent upon success in future clinical trials. We will re-evaluate the transaction price in each reporting period and as uncertain events are resolved or other changes in circumstances occur.

We are utilizing a cost-based input method to measure proportional performance and to calculate the corresponding amount of revenue to recognize. We believe this is the best measure of progress because other measures do not reflect how we transfer the performance obligation to our counter party. In applying the cost based input methods of revenue recognition, we use actual costs incurred relative to budgeted costs to fulfill the combined performance obligation. These costs consist primarily of third-party contract costs and internal full-time equivalent effort. A cost-based input method of revenue recognition requires management to make estimates of costs to complete the performance obligations. The cumulative effect of revisions to estimated costs to complete the performance obligations will be recorded in the period in which changes are identified and amounts can be reasonably estimated. A significant change in these assumptions and estimates could have a material impact on the timing and amount of revenue recognized in future periods.

For the three months ended March 31, 2018, we have recognized \$0.4 million as license and collaboration revenue under the 2016 Bayer Agreement and have \$2.3 million recorded as Unbilled - collaboration and license revenue as of March 31, 2018 on the condensed consolidated balance sheets.

There were no costs incurred to obtain or fulfill the contract.

#### 4. 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, restricted cash, short-term investments, receivables from collaborations, prepaid research and development, prepaid expenses and other current assets and accounts payable, accrued research and development, accrued compensation and employee benefits, accrued and other liabilities and deferred revenue and approximate their fair value due to their

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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 in the sale of an asset or paid to transfer a liability (an exit price) in an orderly transaction between market participants at the reporting date. The accounting guidance establishes a three-tiered hierarchy, which prioritizes the inputs used in the valuation methodologies in measuring fair value as follows:

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

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

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

A financial instrument's categorization within the valuation hierarchy is based upon the lowest level of input that is significant to the fair value measurement. Where quoted prices are available in an active market, securities are classified as Level 1. We classify money market funds as Level 1. When quoted market prices are not available for the specific security, we estimate fair value by using quoted prices for identical or similar instruments in markets that are not active and model-based valuation techniques for which all significant inputs are observable in the market or can be corroborated by observable market data for substantially the full term of the assets. Where applicable, these models project future cash flows and discount the future amounts to a present value using market-based observable inputs obtained from various third-party data providers, including but not limited to, benchmark yields, interest rate curves, reported trades, broker/dealer quotes and market reference data. We classify our corporate notes, commercial paper, U.S. Treasury bills and government agency securities and foreign currency forward contracts as Level 2. 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. 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.

In certain cases where there is limited activity or less transparency around inputs to valuation, the related assets or liabilities are classified as Level 3. Our embedded derivative liabilities are measured at fair value using a Monte Carlo simulation model and are included as a component of other long-term liabilities on the consolidated balance sheets. The embedded derivative liabilities are subject to remeasurement at the end of each reporting period, with changes in fair value recognized as a component of interest and other income (expense), net, in our condensed consolidated statements of operations. The assumptions used in the Monte Carlo simulation model include: 1) our estimates of both the probability and timing of regulatory approval of Andexxa by geographical region and other related events; 2) the probability-weighted net sales of Andexxa; 3) our risk adjusted discount rate that includes a company specific risk premium; 4) our cost of debt; 5) volatility; 6) the probability of a change in control occurring during the term of the note; and 7) the probability of an event of default. The valuation of our embedded derivative liabilities is most



sensitive to the probability of Andexxa achieving regulatory approval given the binary nature of such an approval event and the correlation to other assumptions included in the model.

There were no transfers between Level 1, Level 2 and Level 3 during the periods presented.

In certain cases where there is limited activity or less transparency around inputs to valuation, securities are classified as Level 3. Our noncontrolling interest in SRX Cardio includes the fair value of the contingent milestone and royalty payments, which is valued based on Level 3 inputs. See Note 7, "Asset Acquisition and License Agreements," to these condensed consolidated financial statements for further information.

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

	March 31, 2018			
	Level 1	Level 2	Level 3	Total
Financial Assets:				

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Money market funds	\$19,507	\$—	\$—	\$19,507
Corporate notes and commercial paper	—	260,950	—	260,950
U.S. Treasury bills and government agency securities	—	154,412	—	154,412
Total financial assets	\$19,507	\$415,362	\$—	\$434,869
Financial Liabilities:				
Embedded derivatives liabilities	\$—	\$—	\$7,220	\$7,220

	December 31, 2017			Total
	Level 1	Level 2	Level 3	
Financial Assets:				
Money market funds	\$31,836	\$—	\$—	\$31,836
Corporate notes and commercial paper	—	313,164	—	313,164
U.S. Treasury bills and government agency securities	—	170,458	—	170,458
Total financial assets	\$31,836	\$483,622	\$—	\$515,458
Financial Liabilities:				
Embedded derivatives liabilities	\$—	\$—	\$8,854	\$8,854

Level 3 liabilities are comprised of embedded derivatives liabilities as described in Note 8 “Asset Acquisition and License Agreements.” The following table sets forth a summary of the changes in the estimated fair value of our embedded derivatives liabilities, which were measured at fair value on a recurring basis (in thousands):

Balance as of December 31, 2017	\$8,854
Net decrease in fair value included in interest and other income, net	(1,634)
Balance as of March 31, 2018	\$7,220

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

There were no transfers between any of the levels of the fair value hierarchy during the periods presented.



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## 5. Financial Instruments

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

	March 31, 2018				December 31, 2017			
	Cost	Unrealized Gain	Unrealized (Loss)	Estimated Fair Value	Cost	Unrealized Gain	Unrealized (Loss)	Estimated Fair Value
Money market funds	\$ 19,507	\$ —	\$ —	\$ 19,507	\$ 31,836	\$ —	\$ —	\$ 31,836
Corporate notes and								
commercial paper	261,375	—	(425 )	260,950	313,307	2	(145 )	313,164
U.S. Treasury bills and								
government agency								
securities	154,787	—	(375 )	154,412	170,724	—	(266 )	170,458
	\$ 435,669	\$ —	\$ (800 )	\$ 434,869	\$ 515,867	\$ 2	\$ (411 )	\$ 515,458
Classified as:								
Cash equivalents				\$ 152,198				\$ 162,793
Short-term investments				240,157				281,589
Long-term investments				42,514				71,076
Total cash equivalents and								
investments				\$ 434,869				\$ 515,458

At March 31, 2018, the remaining contractual maturities of available-for-sale securities were less than two years. There have been no significant realized losses on available-for-sale securities for the periods presented. Available-for-sale debt securities that were in a continuous loss position but were not deemed to be other than temporarily impaired were immaterial at both March 31, 2018 and December 31, 2017.

## 6. Balance Sheet Components

## Inventories

Inventories consisted of the following (in thousands):

	March 31, 2018	December 31, 2017
Current:		
Work in process	\$ 3,185	\$ 1,032
Finished goods	63	67
Total inventories	\$ 3,248	\$ 1,099

We began capitalizing inventory during the quarter ended December 31, 2017 as a result of the FDA's approval of Bevyxxa, as the related costs are expected to be recoverable through the commercialization of the product. As of March 31, 2018 and December 31, 2017, prepaid expenses and other current assets on the Condensed Consolidated Balance Sheets include prepayments of \$8.2 million and \$2.3 million, respectively, made to a manufacturer for the purchase of inventories which we expect to be converted to finished goods within the next twelve months. Prepayments of \$4.8 million and \$9.6 million as of March 31, 2018 and December 31, 2017, respectively, is classified as prepaid and other long-term assets as the production is expected after the next twelve months and the amount is deemed recoverable.

No amounts have been capitalized as inventory for Andexxa as of March 31, 2018.

No losses were incurred on valuation of inventories at lower of cost or market value or write-off of obsolete inventories during the three months ended March 31, 2018.

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## Accrued and Other Liabilities

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

	March 31, 2018	December 31, 2017
Commercial related	\$ 2,663	\$ 1,694
Legal and accounting fees	329	256
Deferred rent	899	879
Product revenue provision	53	–
Other	1,697	723
Total accrued liabilities	\$ 5,641	\$ 3,552

## 7. Purchase Commitments

## Bevyxxa Manufacturing Agreement

In 2016 we entered into a Manufacturing Agreement, as amended, with Hovione, Limited, (“Hovione”), pursuant to which Hovione will manufacture active pharmaceutical ingredient (“API”) for Bevyxxa at commercial scale and perform process validation during the term of the agreement. As of March 31, 2018, we have recorded \$8.2 million in prepaid expenses and other current assets and \$4.8 million in prepaid and other long-term under the agreement, and will make up to \$8.6 million of additional payments over the remaining term of the Hovione Agreement, ending June 2019.

## Lonza Manufacturing Services Agreement

In August 2017, we executed a Manufacturing Services Agreement with Lonza AG (“Lonza”) to develop a higher-capacity and lower cost process (“generation 2 process”) for Andexxa bulk drug substance. The manufacturing commitments included therein are contingent upon marketing approval by either the FDA or the European Medicines Agency (“EMA”) of Andexxa manufactured at the current Porrino facility under the generation 2 process and will remain in effect for a period of ten years. Additionally, the agreement provides Lonza with two separate rights to purchase shares of our common stock at a purchase price of one dollar per share, contingent upon certain events. The

first purchase right will be earned by Lonza upon the approval of the generation 2 process and the commencement of process transfer activities to an additional, new facility in Switzerland. The second purchase right will be earned by Lonza upon the approval of the drug substance manufactured at the new facility and the number of shares will be determined based on the achievement of specified performance metrics at the new facility. The number of shares subject to the first and the second purchase right, each, will be the lesser of either the number of shares with an aggregate market value of \$15.0 million based on a 20 day trailing market value average from the date the first or the second purchase right is earned by Lonza, or 500,000 shares.

We measure the fair value of the equity instrument contingently issuable to Lonza by using the stock price and other measurement assumptions as of the earlier of the date at which either: (1) a commitment for performance by the counterparty has been reached; or (2) the counterparty's performance is complete. We determined that Lonza does not have a performance commitment in this arrangement because there is no substantive disincentive for nonperformance. As such, our measurement date for the contingently issuable equity awards will be when the specified performance criteria have been achieved. Until such achievement, the contingently issuable equity awards will be measured at their then-current lowest aggregate fair value at each financial reporting date. As of

March 31, 2018, the lowest aggregate fair value of the awards was zero.

## 8. Asset Acquisition and License Agreements

### SRX Cardio, LLC

In December 2015, we entered into an option agreement with SRX Cardio to explore a novel approach to develop a drug in the field of hypercholesterolemia. This agreement provided us an option to enter into an exclusive license agreement as well as responsibility to lead and fund the development effort during the option period. We made an upfront payment of \$0.5 million.

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In September 2016, we exercised our right to enter into an exclusive license agreement. Pursuant to the terms of the agreement, we made an upfront payment of \$2.2 million to acquire the license and are obligated to pay up to \$152.5 million in research and development milestones related to the advancement of the program and royalties in the range of 2% to 6% of worldwide net sales. We may terminate the license agreement upon 90 days' notice for convenience and the agreement may also be terminated by either party for a material breach by the other party.

We determined that SRX Cardio is and continues to be a variable interest entity and that we hold a variable interest in SRX Cardio's intellectual property assets and the related potential future product candidates these assets may produce. Due to the absence of other significant development programs at SRX Cardio, we concluded that the variable interest was in the entity as a whole. Given the stage of development, we concluded that SRX Cardio is not considered a business as they lack the processes required to generate outputs. Further, because we control those activities most significant to SRX Cardio, we are considered to be the primary beneficiary of SRX Cardio. Accordingly, SRX Cardio is subject to consolidation and we have consolidated the financial statements of SRX Cardio by (a) eliminating all intercompany balances and transactions; and (b) allocating income or loss attributable to the noncontrolling interest in SRX Cardio to net income or loss attributable to noncontrolling interest in our consolidated statement of operations and reflecting noncontrolling interest on our consolidated balance sheet. Our interest in SRX Cardio is limited to the development of the intellectual property asset. The upfront payments of \$0.5 million and \$2.2 million and the obligation to fund the development plan represent our maximum exposure to loss under the agreement. We did not acquire any equity interest in SRX Cardio, any interest in SRX Cardio's cash and cash equivalents or any control over their activities that do not relate to the exclusive license agreement. SRX Cardio does not have any right to our assets except as provided in the exclusive license agreement.

At the inception of the agreement, the identifiable assets, assumed liabilities and non-controlling interest of SRX Cardio were recorded at their estimated fair value upon the initial consolidation of SRX Cardio, including the in-process research and development intangible asset. We estimated the fair value of these indefinite lived intangible assets to be \$3.2 million and the noncontrolling interest to be \$2.9 million. The fair value was estimated using present-value models on potential contingent milestones and royalty payments ("contingent future payments"), based on assumptions regarding the probability of achieving the development milestones, estimate of time to develop the drug candidate, estimates of future cash flows from potential product sales and assumptions regarding the appropriate discount rate.

As of March 31, 2018, we have not provided financial or other support to SRX Cardio that was not previously contracted or required. We recorded SRX Cardio's \$47,000 and \$173,000 of cash as restricted cash as of March 31, 2018 and December 31, 2017, because (a) we do not have any interest in or control over SRX Cardio's cash and (b) the agreement does not provide for these assets to be used for the development of the intellectual property assets developed pursuant to this agreement. We recorded \$332,000 as net loss attributable to noncontrolling interest (SRX Cardio) for the three months ended March 31, 2018 on our condensed consolidated statements of operations, reflecting a change in fair value of our contingent future payments liability to SRX Cardio as of March 31, 2018.

Millennium Pharmaceuticals, Inc.

In August 2004, we entered into an agreement with Millennium to license certain exclusive rights to research, develop and commercialize certain compounds that inhibit Factor Xa, including Bevyxxa. The license agreement requires us to



make license fee, milestone, royalty and sublicense sharing payments to Millennium as we develop, commercialize or sublicense Bevyxxa. The license agreement will continue in force, on a country-by-country basis, until the expiration of the relevant patents or ten years after the launch, whichever is later, or termination by either party pursuant to the agreement. This license agreement may be terminated by either party for the other party's uncured material breach. In addition, we may terminate this agreement for convenience with 30 days' advance written notice.

Under the agreement, milestone payments are determined based on the indication included in our filing and become payable upon acceptance of our new drug application, or NDA, and regulatory approval in the United States and Europe. In December 2016, the FDA accepted our NDA for Bevyxxa for extended-duration prophylaxis of venous thromboembolism, triggering a \$2.0 million milestone payment to Millennium which is recorded as a research and development expense in our condensed consolidated statement of operations. In June 2017, Bevyxxa received regulatory approval in the United States, triggering a \$5.0 million milestone payment to Millennium which is recorded as finite-lived intangible assets in our condensed consolidated balance sheet and will be amortized on a straight-line basis over the remaining estimated patent life. Amortization expenses were \$141,000 for the three months ended March 31, 2018. These amortization expenses were recorded as cost of sales. Should Bevyxxa receive regulatory approval in Europe, another \$5.0 million will become payable to Millennium by us. Future net product sales generated by us will be subject to a tier royalty ranging between 2% and 8%.

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Notes to Condensed Consolidated Financial Statements

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An additional \$23.0 million in milestone payments would become due if Bevyxxa is approved for other indications specified in the agreement in the United States and Europe.

Astellas Pharma, Inc. (“Astellas”)

In 2010, we amended and restated the license agreement we executed with Astellas in August 2005. The amended and restated license agreement provides us certain exclusive rights to research, develop and commercialize Syk inhibitors. Pursuant to the agreement, we may be required to pay Astellas up to \$71.5 million in milestone payments upon the achievement of certain regulatory, approval and sales events for each Syk inhibitor we develop. Additionally, in the event that we enter into an agreement with a third party to develop and commercialize Syk inhibitors, we would be required to pay Astellas 20% of any payments (excluding royalties) received under the collaboration. These payments would be creditable against the aforementioned milestone payments. In addition, we are required to pay Astellas royalties for worldwide sales for any commercial Syk inhibitor product.

In December 2016, we out-licensed exclusive rights to cerdulatinib in topical formulation, excluding oncology, to Dermavant Sciences GmbH (“Dermavant”). Twenty percent of the milestone payments received from Dermavant are payable to Astellas. We recognized zero research and development expense in our condensed consolidated statement of operations for the three months ended March 31, 2018 and 2017, respectively, associated with our payment obligations to Astellas.

## 9. Notes Payable

### BMS and Pfizer Promissory Notes

In December 2016, we entered into a supplemental funding support agreement with BMS and Pfizer whereby we received \$50.0 million in exchange for two promissory notes totaling \$65.0 million that become due in December 2024 (“Notes”). The use of funds is restricted to development activities needed for regulatory approval of Andexxa by the FDA and European Medicines Agency (“EMA”) as provided for in the agreement.

Pursuant to the terms of the agreement, we are required to pay down the Notes each quarter in an amount equal to 5% of net sales of Andexxa in the United States and European Union (“EU”). Should the initial regulatory approval of Andexxa in the United States and EU not be achieved by January 1, 2019, one hundred percent of payments due to us under the Japan License agreement and fifty percent of all other Andexxa license fees and milestone payments received from third-party collaborators will be applied to the Notes. In addition, if the approval of Andexxa in the United States and EU is not achieved by January 1, 2019, we are able to reduce the repayment amount to \$60.0 million if such amount is paid by December 31, 2021 and regardless of the timing of regulatory approval, we may reduce the repayment amount to \$62.5 million if such amount is paid by December 31, 2023. Any unpaid amounts shall become immediately due upon: 1) a change of control of our company; 2) an event of default; and 3) termination for breach. We have the right to prepay the repayment amount at any time without any penalty.

The accounting for such funding agreement requires us to make certain estimates and assumptions, including timing of Andexxa approval, timing of royalty payments due to BMS and Pfizer, the expected rate of return to BMS and Pfizer, the split between current and long-term portions of the obligation and accretion of related interest expense.

The upfront cash receipt of \$50.0 million is recorded as Notes payable, long term at issuance. We are accruing for interest over the term of the related note. The carrying values of the Notes payable at March 31, 2018 and December 31, 2017, including accrued interest of \$5.1 million and \$4.2 million, are \$51.4 million and \$50.6 million, respectively. The total carrying value of the Notes, including accrued interest, will be classified as long-term on the condensed consolidated balance sheet until we receive regulatory approval of Andexxa or until amounts are contractually payable to BMS and Pfizer.

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Notes to Condensed Consolidated Financial Statements

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We evaluated the features of the Notes and determined that certain features require acceleration of payments such as pursuant to a change of control or an event of default, as well as the terms that adjust the total amount of interest required to be paid based upon the timing of initial regulatory approval in the United States and EU. We determined that these features (embedded derivatives) require bifurcation and fair value recognition. We determined the fair value of each derivative using a Monte Carlo simulation model taking into account the probability of these events occurring and potential repayment amounts and timing of such payments that would result under various scenarios (see Note 3 “Revenue Recognition” to these condensed consolidated financial statements). We will remeasure the embedded derivatives to fair value each reporting period until the repayment, termination or maturity of the long-term note payable. For the three months ended March 31, 2018, we recognized \$0.6 million loss upon remeasurement of the embedded derivatives.

The estimated fair value of the Notes payable at March 31, 2018 and December 31, 2017 was \$54.0 million and \$55.5 million, respectively, and the fair value was measured using Level 3 inputs. The estimated fair market value was calculated using a Monte Carlo simulation model with inputs consistent with those used in determining the embedded derivative values as described in Note 3 “Revenue Recognition” to these condensed consolidated financial statements.

#### Royalty-based Financing

In February 2017, we entered into a purchase and sale agreement (the “Royalty Sales Agreement”) with HealthCare Royalty Partners and its affiliates. (“HCR”) whereby HCR acquired a royalty interest in future worldwide net sales of Andexxa. We received \$50.0 million upon closing and are due to receive an additional \$100.0 million following the U.S. regulatory approval of Andexxa in May 2018.

We are required to pay HCR a royalty of 2.0% based on tiered net worldwide sales of Andexxa. Upon the second closing and receipt of \$100.0 million from HCR, the tiered royalty rate will increase to a range of 7.85% to 3.58%, as the applicable rate decreases starting at worldwide net sales levels above \$150.0 million. Total royalty payments are capped at 195% of the funded amount, however, the royalty rates are subject to increase based on the timing of manufacturing approvals from the FDA and/or EMA. We have evaluated the terms of the Royalty Sales Agreement and concluded that the features of the funded amount are similar to those of a debt instrument. Accordingly, we have accounted for the transaction as long-term debt.

As the repayment of the funded amount is contingent upon the sales volumes of Andexxa, the repayment term may be shortened or extended depending on the actual sales of Andexxa. The repayment period is commencing from the first commercial sale of Andexxa in any country and expiring on the date when HCR has received cash payments totaling an aggregate of 195% of the funded amounts.

We evaluated the terms of the debt and determined that certain features, such as the increase in the repayment amount up to \$125.0 million upon a change of control and the variability in the royalty payments based upon the timing of initial regulatory approval in the United States and EU, are embedded derivatives that require bifurcation from the debt instrument and fair value recognition. We determined the fair value of each derivative using a Monte Carlo simulation model taking into account the probability of these events occurring and potential repayment amounts and

timing of such payments that would result under various scenarios, as further described in Note 3 “Revenue Recognition” to these condensed consolidated financial statements. We will remeasure the embedded derivatives to fair value each reporting period until the time the features lapse and/or termination of the Royalty Sales Agreement. For the three months ended March 31, 2018, we recognized a gain of \$2.2 million upon remeasurement of the embedded derivatives.

The effective interest rate as of March 31, 2018 was 14.0%. For the three months ended March 31, 2018 and 2017, accrued interest in the amount of \$1.8 million and \$0.9 million, respectively, were added to the principal balance of the debt.

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## Notes to Condensed Consolidated Financial Statements

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In connection with the Royalty Sales Agreement, we paid HCR a fee of \$2.0 million and incurred additional debt issuance costs totaling \$0.6 million, which includes expenses that we paid on behalf of HCR and expenses incurred directly by us. Debt issuance costs have been netted against the debt as of March 31, 2018 and are being amortized over the estimated term of the debt using the effective interest method. For the three months ended March 31, 2018 and 2017, we recognized interest expense, including amortization of the debt discount, related to the debt of \$1.8 million and \$0.9 million, respectively. The assumptions used in determining the expected repayment term of the debt and amortization period of the issuance costs requires that we make estimates that could impact the short and long-term classification of these costs, as well as the period over which these costs will be amortized. The carrying value of the long-term debt as of March 31, 2018 and December 31, 2017 was \$56.0 million and \$54.3 million, respectively, inclusive of accrued interest expense of \$9.2 million and \$7.4 million, respectively, and net of unamortized debt discount of \$2.3 million and \$2.3 million, respectively. The total carrying value of the debt, including accrued interest, will be classified as long-term on the consolidated balance sheet until we achieve regulatory approval of Andexxa.

The estimated fair value of long-term debt at March 31, 2018 and December 31, 2017 was \$57.8 million and \$58.8 million, respectively, and the fair value was measured using Level 3 inputs. The estimated fair market value was calculated using a Monte Carlo simulation model with inputs consistent with those used in determining the embedded derivative values as described in Note 3 “Revenue Recognition” to these condensed consolidated financial statements.

## 10. Stock Based Compensation

## Stock Options

The following table summarizes stock option activity under our 2013 Equity Incentive Plan (the “2013 Plan”) and an Inducement Plan, and related information during the three months ended March 31, 2018:

	Shares Subject to Outstanding Options	Weighted- Average Exercise Price Per Share
Balance at December 31, 2017	6,514,538	\$ 31.36
Options granted	1,144,976	47.83
Options exercised	(185,664 )	22.30
Options canceled	(180,117 )	37.37
Balance at March 31, 2018	7,293,733	\$ 34.03

## Performance Stock Options (“PSOs”)

The following table summarizes PSO activities under our 2013 Plan and related information during the three months ended March 31, 2018:

	Shares Subject to Outstanding PSOs	Weighted- Average Exercise Price Per Share
Balance at December 31, 2017	164,783	\$ 23.76
Options granted	—	—
Options exercised	(1,302 )	23.76
Options canceled	—	—
Balance at March 31, 2018	163,481	\$ 23.76

## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Consolidated Financial Statements

(Unaudited)

## Restricted Stock Units (“RSUs”)

The following table summarizes RSU activity under our 2013 Plan and Inducement Plan, and related information during the three months ended March 31, 2018:

	Shares Subject to Outstanding RSUs	Weighted- Average Grant Date Fair Value Per Share
Balance at December 31, 2017	600,334	\$ 27.87
RSUs granted	359,057	48.78
RSUs released	(279,555 )	28.49
RSUs canceled	(33,711 )	34.11
Balance at March 31, 2018	646,125	\$ 38.90

## Performance Stock Units (“PSUs”)

In March 2018, the Compensation Committee of our Board of Directors approved a program to award up to 102,600 PSUs to the management team based on the achievement of certain regulatory and revenue goals related to Andexxa. The following table summarizes PSU activity under our 2013 Plan and related information during the three months ended March 31, 2018:

	Shares Subject to Outstanding PSUs	Weighted- Average Grant Date Fair Value Per Share
Balance at December 31, 2017	304,754	\$ 25.34
PSUs granted	102,600	32.66
PSUs released	(6,004 )	49.83
PSUs canceled	(8,125 )	25.54
Balance at March 31, 2018	393,225	\$ 26.87

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



	Three Months Ended March 31,	
	2018	2017
Research and development	\$4,452	\$4,150
Selling, general and administrative	6,528	4,884
Total stock-based compensation	\$10,980	\$9,034

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## PORTOLA PHARMACEUTICALS, INC.

## Notes to Condensed Consolidated Financial Statements

(Unaudited)

## 11. Net Loss per Share Attributable to Portola Common Stockholders

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

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

	Three Months Ended March 31,	
	2018	2017
Stock options to purchase common stock	7,293,733	6,585,143
Performance stock options	163,481	180,752
Common stock warrants	1,500	1,500
Restricted stock units	646,125	631,743
Performance stock units	393,225	369,118
Employee stock purchase plan	12,391	5,816

Up to one million shares may be contingently issued, if certain performance conditions are met under an agreement with one of our contract manufacturers.

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

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the consolidated financial statements and notes thereto included elsewhere in this report and with the audited consolidated financial statements and related notes thereto included as part of our Annual Report on Form 10-K for the year ended December 31, 2017.

### Special note regarding forward-looking statements

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

### OVERVIEW

We are a biopharmaceutical company focused on the development and commercialization of novel therapeutics in the areas of thrombosis, other hematologic disorders and inflammation for patients who currently have limited or no approved treatment options. Our two FDA-approved medicines are Bevyxxa® (betrixaban), the first and only oral, once-daily Factor Xa inhibitor, and Andexxa® [coagulation factor Xa (recombinant), inactivated-zhzo], the first and only antidote for the Factor Xa inhibitors rivaroxaban and apixaban. We are also advancing cerdulatinib, a spleen tyrosine kinase, or Syk, and Janus kinases, or JAK, inhibitor in development to treat hematologic cancers and have a partnered program, which is focused on developing selective Syk inhibitors for inflammatory conditions. These compounds come from our own internal research efforts and represent important advances that address significant unmet needs.

We refer to our two approved drugs in this report as Andexxa and Bevyxxa. If approved in the EU or other parts of the world we expect each drug may be marketed under different brand names. In addition, an international nonproprietary name ("INN") has been designated for each drug. Our previous INN for Andexxa was andexanet alfa; however, in the United States this INN has been replaced with "coagulation factor Xa (recombinant), inactivated-zhzo." For the EU and other parts of the world, andexanet alfa could remain the INN for Andexxa. Our use of Andexxa or Bevyxxa in this document in the context of continued development activities for which we have not yet received regulatory approval should not be read to imply that we have received regulatory approval for any indication or in any jurisdiction not reflected in our product labels.

We are focusing our efforts on the commercialization of Bevyxxa and Andexxa in the United States. Additionally, we are evaluating our strategy to commercialize in Europe following a positive trend vote for Andexxa in February 2018 and a negative opinion for Bevyxxa received in March 2018 from the Committee for Medicinal Products for Human Use, or CHMP, regarding each product candidate's respective Marketing Authorisation Applications, or MAA. We are

also continuing to advance cerdulatinib through a phase 2a clinical trial.

Our strategy

Key elements of our strategy are as follows:

- commercialize Bevyxxa and Andexxa in the United States using our hospital-focused sales force;
- advance our Generation 2 Andexxa manufacturing process through FDA approval;
- strategically scale up our field force and increase engagement with medical, scientific and academic professionals and associations to ensure awareness of unmet need;
- advance Bevyxxa and Andexxa through the European approval process;

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- advance development of cerdulatinib for the treatment of hematologic cancers;
- continue to advance our current development pipeline and expand it with multiple preclinical or clinical stage product candidates that align with our scientific expertise and experience; and
- continue to seek and evaluate partnerships that provide support for the further development or launch of our product candidates while retaining significant economic and commercial rights.

#### Recent developments

We launched Bevyxxa in the United States in January 2018 when drug supply became commercially available and we are continuing physician education activities, as well as hospital and payor negotiations regarding formulary placement. We also began the appeal process related to the negative opinion rendered by the CHMP and have continued to progress supplementary manufacturing approvals of our commercial product.

Andexxa was approved by the FDA on May 3, 2018. We are working to provide European regulators with the information they requested as part of the positive trend vote, including additional data from ANNEXA-4 and more information on our Generation 2 product. Beginning in June, we will be offering Andexxa to a very limited number of hospitals through an Early Supply Program. This will be a dedicated program for 30-40 hospitals that are predominantly ANNEXA-4 clinical trial sites, and also a limited number of level 1 trauma centers and comprehensive stroke centers. We are almost through our second successful Generation 2 commercial manufacturing campaign and have built up significant supply, which will be subject to FDA approval, and enable a broader commercial launch. These costs have not been capitalized as inventory on our condensed consolidated balance sheet as of March 31, 2018 and will not be capitalized until the FDA has approved the Generation 2 commercial manufacturing process.

We are continuing to enroll patients in a Phase 2a proof-of-concept study for cerdulatinib in patients with B- and T-cell NHL, including patients with FL, PTCL, and CTCL, who have failed or relapsed on existing marketed therapies or products in development. We have begun discussions with the FDA about our future plans for this program. Cerdulatinib has the potential for broad activity in hematologic cancers because it blocks the B-cell receptor pathway via Syk and key cytokine receptors via JAK. In addition, pre-clinical evidence suggests that Syk is also expressed in PTCL and activating mutations in JAK pathway are frequently observed in PTCL.

#### Financial operations overview

##### Revenue

We generate revenues from both product sales and collaboration and license agreements. The recognition of product revenues occur at a point in time (upon delivery to our customers) while our collaboration and license revenues are recognized over a period of time as the related services are rendered. In the future, we expect our product revenues to increase according to the timing of hospital formulary approvals and to further increase if we receive approval for our other product candidates in the U.S. and/or E.U. Collaboration and license revenues will continue to fluctuate according to changes in our assumptions underlying the transaction price for such collaboration agreements, including the probability of achieving milestones, and the timing of costs incurred relative to the total budgeted effort to satisfy performance obligations identified within the contract.

The following table summarizes the sources of our product revenue and collaboration and license revenue for the three months ended March 31, 2018 and 2017:

Three Months Ended March 31,	
2018	2017

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	(in thousands)	
Total product revenue, net	\$606	\$—
Bristol-Myers Squibb Company and Pfizer Inc.	\$1,136	\$1,758
Daiichi Sankyo, Inc.	1,776	2,181
Bayer Pharma, AG and Janssen Pharmaceuticals, Inc.	2720	799
Bayer	406	390
Total collaboration and license revenue	\$6,038	\$5,128
Total revenues	\$6,644	\$5,128

## Cost of sales

Cost of sales of \$0.3 million for the three months ended March 31, 2018 consists primarily of costs associated with manufacturing of Bevyxxa, net sales based royalties payable to Millennium and amortization of an intangible asset associated with the program.

Inventories manufactured prior to the FDA's approval of Bevyxxa, totaling approximately \$21.2 million, were expensed as research and development expense as incurred. We expect costs of sales to be impacted by our zero-cost, pre-launch inventories for at least the next twelve months and for cost of sales relative to net product sales to increase once such inventory reserves are depleted.

## Research and development expenses

Research and development expenses represent costs incurred to conduct research, such as the discovery and development of our un-partnered 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. Payments made prior to the receipt of goods or services to be used in research and development are capitalized until the goods are received or services are rendered.

We expect our research and development expenses to be consistent in the near term and to begin to decrease if and when we receive regulatory approval of our product candidates as the costs associated with our manufacturing processes may qualify for capitalization as inventory and will be subsequently expensed as costs of goods sold when the inventory is sold. The timing and amount of expenses incurred will depend upon regulatory approval of our product candidates and associated manufacturing processes and the outcomes of current or future clinical studies for our product candidates as well as the related regulatory requirements, start-up manufacturing and supply chain costs and any costs associated with the advancement of our preclinical programs.

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

Product candidate	Phase of Development	Three Months Ended March 31,	
		2018	2017
		(in thousands)	
		(unaudited)	
Betrixaban	Phase 3	\$8,289	\$14,477
Andexanet alfa	Phase 2/3/4	45,886	13,233
Cerdulatinib	Phase 1/2a	4,447	1,872
Syk selective inhibitor	Pre-clinical	29	40
Other research and development expenses <sup>(1)</sup>		1,416	1,023
Total research and development expenses		\$60,067	\$30,645

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

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 and manufacturing of our product candidates.

#### Selling, general and administrative expenses

Selling, general and administrative expenses consist primarily of personnel costs, allocated facilities costs and other expenses for outside professional services, including legal, human resources, audit and accounting services and sales and marketing expenses related to commercial launch preparation. Personnel costs consist of salaries, benefits and stock-based compensation.

We expect selling, general and administrative expenses to increase significantly in the future as we incur significant additional expenses associated with the establishment of a hospital-based sales force in the United States and possibly other major markets, as well as commercial infrastructure initiatives including information technology systems quality and compliance systems, and personnel support for the commercial organization.



#### Interest and other income, net

Interest and other income, net consists primarily of interest received on our cash, cash equivalents and investments, unrealized gains and losses from the remeasurement of our embedded derivatives associated with BMS and Pfizer promissory notes and a royalty-based financing arrangement with HealthCare Royalty Partners and its affiliates, and foreign currency deposits.

#### Interest expense

Interest expense consists of amounts accrued pursuant to our long-term debt and notes payable balances outstanding during the period.

#### Critical accounting policies and significant judgments and estimates

Our management's discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with United States generally accepted accounting principles, or U.S. GAAP. The preparation of these condensed consolidated financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent liabilities at the date of the condensed consolidated financial statements, as well as the reported revenue generated and expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Except as noted below, there have been no significant and material changes in our critical accounting policies during the three months ended March 31, 2018, as compared to those disclosed in "Management's Discussion and Analysis of Financial Condition and Results of Operations-Critical Accounting Policies and Significant Judgments and Estimates" in our Annual Report on Form 10-K for the year ended December 31, 2017 filed with the SEC on March 1, 2018.

#### Revenue Recognition

On January 1, 2018, we adopted ASC 606, Revenue from Contracts with Customers, using the modified retrospective method to all contracts that were not completed as of January 1, 2018. Pursuant to ASC 606, we recognize revenue when our customer obtains control of promised goods or services, in an amount that reflects the consideration that we expect to receive in exchange for those goods or services. To determine revenue recognition for arrangements that we determine are within the scope of ASC 606, we perform the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) we satisfy a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract and determine those that are performance obligations, and assess whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

#### Collaboration and license revenues

Upon adoption of ASC 606, we recognized a \$29.0 million cumulative effect adjustment to decrease the accumulated deficit balance as of January 1, 2018. Results for a reporting period beginning after January 1, 2018 are presented under ASC 606, while prior period amounts are not adjusted and continue to be reported under the accounting standards in effect for the prior period. For further discussion of the change in our significant accounting policies, please refer to Note 2, “Summary of Significant Accounting Policies—Revenue Recognition,” of the condensed consolidated financial statements.

#### Product Revenue, Net

In January 2018 we began shipping Bevyxxa to a limited number of wholesalers, or Customers, in the United States. These Customers subsequently resell our products to hospitals, pharmacies and long-term care centers. In addition to distribution agreements with Customers, we enter into arrangements with group purchasing organizations and payors that provide for privately negotiated rebates, chargebacks, and discounts with respect to the purchase of our products.

We recognize revenue on product sales when the Customer obtains control of our product, which occurs at a point in time (upon delivery). Product revenues are recorded net of applicable reserves for variable consideration, including discounts and allowances. We expense incremental costs of obtaining a contract when incurred, if the expected amortization period of the asset that we would have recognized is one year or less.

#### Reserves for Variable Consideration

Revenues from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established and which result from discounts, returns, chargebacks, rebates, copay assistance and other allowances that are offered within contracts between us and our Customers, group purchasing organizations, payors and other indirect customers relating to our product sales. These reserves as detailed below are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if the amount is payable to the Customer) or a current liability (if the amount is payable to a party other than a Customer). Where appropriate, these estimates take into consideration a range of possible outcomes that are probability-weighted in accordance with the expected value method in ASC 606 for relevant factors such as current contractual and statutory requirements, specific known market events and trends, industry data, and forecasted customer buying and payment patterns. Overall, these reserves reflect our best estimates of the amount of consideration to which we are entitled based on the terms of the respective underlying contracts.

The amount of variable consideration that is included in the transaction price may be constrained, and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and earnings in the period such variances become known.

**Trade Discounts and Allowances:** We generally provide Customers with discounts which include incentive fees that are explicitly stated in our contracts and are recorded as a reduction of revenue in the period the related product revenue is recognized. In addition, we compensate (through trade discounts and allowances) our Customers for sales order management, data, and distribution services. However, we have determined such services received to date are not distinct from our sale of products to the Customer and, therefore, these payments have been recorded as a reduction of revenue.

**Product Returns:** Consistent with industry practice, we generally offer Customers a limited right of return based on the product's expiration date for product that has been purchased from us. We estimate the amount of our product sales that may be returned by our Customers and record this estimate as a reduction of revenue in the period the related product revenue is recognized. We currently estimate product return liabilities using available industry data, our own sales information and our visibility into the inventory remaining in the distribution channel. We have not received any returns to date and believe that product returns in future periods will be minimal.

**Chargebacks:** Chargebacks are discounts that occur when contracted customers, which currently consist primarily of group purchasing organizations purchase directly from our wholesalers at a discounted price. The specialty wholesalers, in turn, charge us back the difference between the price initially paid by the wholesaler and the discounted price paid to the wholesaler by the healthcare providers. These reserves are established in the same period that the related revenue is recognized, resulting in a reduction of product revenue and contract assets. Chargeback amounts are generally determined at the time of resale to the qualified healthcare provider by Customers, and we generally issue credits for such amounts within a few weeks of the Customer's notification to us of the resale. Reserves for chargebacks consist of credits that we expect to issue for units that remain in the distribution channel inventories at each reporting period end that we expect will be sold to qualified healthcare providers, and chargebacks that Customers have claimed but for which we have not yet issued a credit.

**Payor Rebates:** We contract with various private payor organizations, primarily insurance companies and pharmacy benefit managers, for the payment of rebates with respect to utilization of our products. We estimate these rebates and record such estimates in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a current liability.

Other Incentives: Other incentives that we offer include voluntary patient assistance programs such as co-pay assistance. Co-pay assistance programs are intended to provide financial assistance to qualified commercially insured patients with prescription drug co-payments required by payors. The calculation of the accrual for co-pay assistance is based on an estimate of claims and the cost per claim that we expect to receive associated with product that has been recognized as revenue, but remains in in the distribution channel inventories at period end.

#### Inventories

We present as current assets inventories for which production is expected after twelve months from the balance sheet date as the amount is deemed recoverable.

## Results of operations

Comparison of the three months ended March 31, 2018 and 2017

## Revenue

	Three Months Ended March 31,			
	2018	2017	Increase	% Increase
	(in thousands, except percentages)			
Product revenue, net	\$606	\$—	\$ 606	—
Collaboration and license revenue	6,038	5,128	910	18 %
Total revenues	\$6,644	\$5,128	\$ 1,516	30 %

The increase in total revenues during the three months ended March 31, 2018 compared to the three months ended March 31, 2017 was primarily attributable to:

- commercial product revenue earned from the U.S. sales of Bevyxxa which we began shipping to customers in January 2018; and
  - an increase in collaboration and license revenue primarily due to partial recognition of a \$2.0 million milestone within the Bayer phase 3 agreement pursuant to the new revenue recognition accounting standard.
- Research and development expenses

	Three Months Ended March 31,			
	2018	2017	Increase	% Increase
	(in thousands, except percentages)			
Research and development expenses	\$60,067	\$30,645	\$29,422	96 %

The increase in research and development expenses during the three months ended March 31, 2018 compared to the three months ended March 31, 2017 was primarily attributable to:

- increased program costs of \$32.7 million related to Andexxa, which was primarily driven by manufacturing activities associated with our Generation 2 product with our contract manufacturer, Lonza, during the current quarter;
  - increased program costs of \$2.6 million related to cerdulatinib primarily due to increased manufacturing expenses; and
  - decreased program costs of \$6.2 million related to Bevyxxa, which was largely the result of capitalizing manufacturing expenses that were recorded as research in development expenses in 2017.
- Selling, general and administrative expenses

	Three Months Ended March 31,			
	2018	2017	Increase	% Increase
	(in thousands, except percentages)			
Selling, general and administrative expenses	\$31,541	\$15,021	\$16,520	110 %

The increase in selling, general and administrative expenses during the three months ended March 31, 2018 compared to the three months ended March 31, 2017 was primarily attributable to:

- increased headcount-related costs of \$10.0 million resulting from the hiring of our sales force and supporting commercial functions; and
- increased external costs of \$6.0 million associated with commercial and marketing initiatives to support the launch of Bevyxxa.

Interest and other income, net

	Three Months Ended March 31, 2018 2017 Increase % Increase (in thousands, except percentages)			
Interest and other income, net	\$3,371	\$413	\$ 2,958	716 %

The increase in interest and other income, net during the three months ended March 31, 2018 compared to the three months ended March 31, 2017 was primarily due to:

\$1.6 million of gain recognized upon remeasurement of embedded derivative liabilities; and  
 \$1.3 million of interest income earned from higher investment balances in the current period.  
 Interest expense

	Three Months Ended March 31, 2018 2017 Increase % Increase (in thousands, except percentages)			
Interest expense	\$2,581	\$1,639	\$ 942	57 %

The increase in interest expense during the three months ended March 31, 2018 compared to the three months ended March 31, 2017 was primarily due to:

long-term debt from HealthCare Royalty Partners and its affiliates that was outstanding for three months during 2018 as compared to two months in 2017.

#### Liquidity and capital resources

Due to our significant research and development expenditures, we have generated significant operating losses since our inception. We have financed our operations primarily through sales of our equity securities, collaborations, including loans from our collaboration partners, a royalty-based financing arrangement, and sales of commercial and development rights to some of our product candidates. Our expenditures are primarily related to research and development activities, including clinical trial and manufacturing related costs, and commercial preparation costs. At March 31, 2018, we had available cash, cash equivalents and investments of \$451.1 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.

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 from the date of this filing. 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 the \$100.0 million payment from HCR to be paid to us in connection with the FDA's approval of Andexxa, and potential milestones receivable under our current collaboration and license agreements. Our future funding requirements will depend on many factors, including the following:

- the cost, timing and outcomes of regulatory approvals;
- the cost of manufacturing our product candidates, including process improvements in order to manufacture product candidates at commercial scale, and establishing commercial supplies of our product candidates;
- the cost and timing of establishing sales, marketing and distribution capabilities;
- the timing, receipt and amount of sales, profit sharing or royalties, if any, from our potential products;

- the terms and timing of any other collaborative, licensing and other arrangements that we may establish;
- the receipt of any collaboration payments;
- the number and characteristics of product candidates that we pursue;
- the scope, rate of progress, results and cost of our clinical studies, preclinical testing and other related activities;
- 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.

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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.

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

	March 31,		Increase /		% Increase
	2018	2017	(Decrease)	/	(Decrease)
	(in thousands, except percentages)				
Cash used in operating activities	\$ (88,420)	\$ (51,536)	\$ 36,884	72	%
Cash provided by (used in) investing activities	\$ 69,581	\$ (52,590)	\$ 122,171	232	%
Cash provided by financing activities	\$ 5,559	\$ 51,311	\$ (45,752 )	(89	%)
Net decrease in cash, cash equivalents and restricted cash	\$ (13,280)	\$ (52,815)	\$ (39,535 )	(75	%)

#### Cash used in operating activities

Cash used in operating activities was \$88.4 million for the three months ended March 31, 2018, compared to cash used of \$51.5 million for the same period in 2017. Operating cash flows can differ from our condensed consolidated net loss as a result of differences in the timing of cash receipts and non-cash charges.

Cash used in operating activities for the three months ended March 31, 2018 includes payments made to our contract manufacturing organizations for the manufacture of Andexxa and Bevyxxa totaling \$40.3 million and \$3.1 million, respectively, \$35.2 million of disbursements to third party vendors to support routine research and development and selling and general and administrative operations, and \$22.5 million in payroll and related employee costs. These cash outflows were partially offset by cash receipts of \$15.7 million, which was primarily from \$11.7 million in receipts from our Andexxa collaboration agreements and the receipt of \$3.8 million associated with a milestone earned pursuant to our out-license of cerdulatinib in topical formulation.

Cash used in operating activities for the three months ended March 31, 2017 includes payments made to our contract manufacturing organizations for the manufacture of Andexxa and Bevyxxa totaling \$14.4 million and \$1.0 million, respectively, \$21.5 million of disbursements to third party vendors to support routine research and development and selling and general and administrative operations, and \$14.6 million in payroll and related employee costs.

Cash provided by (used in) investing activities

Cash provided by investing activities was \$69.6 million for the three months ended March 31, 2018, compared to \$52.6 million of cash used in investing activities for the same period in 2017.

Cash provided by investing activities for the three months ended March 31, 2018 was primarily related to proceeds from maturities of investments of \$148.1 million, partially offset by investments of \$78.0 million.

Cash used in investing activities for the three months ended March 31, 2017 was primarily related to purchase of investment of \$147.8 million, partially offset by proceeds from maturities of investments of \$95.3 million.

Cash provided by financing activities

Cash provided by financing activities was \$5.6 million for the three months ended March 31, 2018, compared to \$51.3 million of cash provided for the same period in 2017.

Cash provided by financing activities of \$5.6 million for the three months ended March 31, 2018 was primarily related to proceeds from purchases under our Employee Stock Purchase Plan of \$1.5 million and exercises of stock options of \$4.2 million. These cash flows were partially offset by a \$0.1 million dividend payment to SRX.

Cash provided by financing activities of \$51.3 million for the three months ended March 31, 2017 was related to net proceeds from debt issuance of \$48.0 million, proceeds from purchases under our Employee Stock Purchase Plan of \$0.9 million and exercises of stock options of \$2.9 million. These cashflows were partially offset by payments of debt issuance costs of \$0.5 million.

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

There were no material changes during the three months ended March 31, 2018 outside the ordinary course of business in our specified contractual obligations as disclosed in our Annual Report on Form 10-K for the year ended December 31, 2017, filed with the SEC on March 1, 2018.

#### 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 March 31, 2018, we had cash, cash equivalents and investments of \$451.1 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, manufacturing, regulatory and commercialization activities with vendors in Europe. We made payments in the aggregate amount of €26.6 million and €6.5 million to our European vendors during the three months ended March 31, 2018 and 2017, respectively. We are subject to exposure due to fluctuations in foreign exchange rates in connection with these agreements and with our cash balance denominated in Euros and British Pounds, to a lesser extent. For the three months ended March 31, 2018, the effect of the exposure to these fluctuations in foreign exchange rates was not material.

#### ITEM 4: CONTROLS AND PROCEDURES

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

#### Evaluation of Disclosure Controls and Procedures

As required by Rule 13a-15(b) under the Exchange Act, our management, under the supervision and with the participation of our principal executive officer and principal financial officer, has evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as such term is defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) as of March 31, 2018. Based on such evaluation, our principal executive officer and principal financial officer have concluded that, as of March 31, 2018, our disclosure controls and procedures were effective at the reasonable assurance level.

#### Changes in Internal Control Over Financial Reporting

There were no changes in our internal controls over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the three months ended March 31, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

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

#### Item 1A. RISK FACTORS.

Investing in our common stock involves a high degree of risk. You should consider carefully the following risks, together with all the other information in this report, including our financial statements and notes thereto, before you invest in our common stock. If any of the following risks actually materializes, our operating results, future prospects, 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.

In assessing these risks, you should also refer to other information contained in this quarterly report on Form 10-Q, including our Condensed Consolidated Financial Statements and related Notes. We have marked with an asterisk (\*) those risks described below that reflect substantive changes from, or additions to, the risks described in our annual report on Form 10-K for the year ended December 31, 2017.

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

\*We have incurred significant losses, and expect to incur substantial and increasing losses as we continue to develop and commercialize our product candidates.

We are an early stage commercial biopharmaceutical company. We launched our first commercial product, Bevyxxa, in January 2018 and received FDA approval of our second commercial product, Andexxa in May 2018. Andexxa and Bevyxxa are our only approved products, and we continue to incur significant expenses related to the commercialization of these products, our ongoing and planned future clinical studies, research and development activities for cerdulatinib and our other product candidates, and selling, general and administrative activities. Our operating expenses increased during the first quarter of 2018, and we do not anticipate a decrease in the near term. As of March 31, 2018, we had an accumulated deficit of approximately \$1.3 billion.

To date, we have financed our operations primarily through sales of our equity securities, collaborations, including a loan from one of our collaboration partners, a sale of a royalty stream from future product sales, sales of commercial and development rights to some of our product candidates, 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. We anticipate that we will continue to incur substantial expenses as we:

- establish and scale-up manufacturing capabilities and a sales, marketing and distribution infrastructure to commercialize Andexxa, Bevyxxa and other products for which we may obtain regulatory approval, including process improvements in order to manufacture Andexxa at commercial scale;
- initiate or continue clinical studies of our three most advanced product candidates, including initiating a post-marketing randomized controlled trial of Andexxa, as required by the FDA;
- 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; and
- enhance operational, compliance, financial, quality and information management systems and hire more personnel, including personnel to support development of our product candidates and support our commercialization efforts.

To be profitable in the future, we must succeed in commercializing Andexxa and Bevyxxa and developing and commercializing other products with significant market potential. This will require us to be successful in a range of

activities, including advancing our product candidates, successfully completing clinical studies of our product candidates, obtaining regulatory approval for these product candidates, and manufacturing, marketing and selling Andexxa, Bevyxxa and any other 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 operating results are difficult to predict and will likely fluctuate from quarter to quarter and year to year. Due to the recent approval by the FDA of Andexxa and Bevyxxa and the absence of historical sales data, our product sales will be difficult to predict from period to period and as a result, you should not rely on sales results in any period as being indicative of future performance and sales may be below the expectation of securities analysts or investors in the future. We believe that our quarterly and annual results of operations may be affected by a variety of factors, including:

- the level of demand;
- the extent to which coverage and reimbursement is available from government and health administration authorities, private health insurers, managed care programs and other third-party payors;
- rebates, discount, other pricing concessions and fees that we may provide to integrated delivery networks, group purchasing organizations, other purchasers and pharmacy benefits managers and other third-party payors;
- the timing, cost and level of investment in our marketing efforts to support sales;
- the timing, cost and level of investment in our research and development activities involving approved products and product candidates;
- the cost of manufacturing, distribution and the amount of legally mandated discounts to government entities, other discounts and rebates, product returns and other gross-to-net deductions;
- the risk/benefit profile, cost and reimbursement of existing and potential future drugs which compete with approved products; and
- expenditures that we will or may incur to acquire or develop additional product candidates and technologies;

In addition, from time to time, we enter into collaboration agreements with other companies that include development funding and upfront and milestone payments, and we expect that amounts earned from our collaboration agreements will continue to be an important source of revenue. These upfront and milestone payments may vary significantly from quarter to quarter and any such variance could cause a significant fluctuation in our operating results from one quarter to the next.

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

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

We have recently launched commercial operations and are advancing multiple product candidates through the research and clinical development process. The development and commercialization of Andexxa, Bevyxxa and our product candidates will continue to require substantial funds. Our future financing requirements will depend on many factors, some of which are beyond our control, including the following:

product sales of Andexxa, Bevyxxa, and if approved for commercial marketing, our product candidates;  
the costs of commercialization activities, including product sales, marketing, manufacturing and distribution and general corporate and commercial infrastructure;  
the timing of, and costs involved in, seeking and obtaining approvals from the FDA and other regulatory authorities;  
the possible development of additional product candidates, including through in-licensing and acquisitions;  
the degree and rate of market acceptance of any products launched by us or partners;  
our ability to enter into additional collaboration, licensing, commercialization or other financing arrangements and the terms and timing of such arrangements;

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- the rate of progress and cost of our clinical studies; and
- the emergence of competing technologies or other adverse market developments.

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

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

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

Our success depends heavily on the launch and commercialization of Andexxa and Bevyxxa. Our commercialization and development of our 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 and commercialization of Andexxa, Bevyxxa, and, to a lesser extent, cerdulatinib and our selective Syk inhibitor program. Our ability to generate product revenue from our product candidates, which will not occur until after regulatory approval, if ever, will depend on the successful development, regulatory approval and eventual commercialization of our product candidates. The success of our products and product candidates will depend on several factors, including the following:

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

Andexxa, Bevyxxa and potential future product candidates may fail to achieve the degree of market acceptance by physicians, patients, healthcare payors and others in the medical community necessary for commercial success.

The commercial success of Andexxa, Bevyxxa and any potential future product candidates for which we may obtain marketing approval from the FDA or other regulatory authorities will depend upon their acceptance by the medical community and third-party payors as clinically useful, cost-effective and safe. The degree of market acceptance of any drug depends on a number of factors, such as:

- the prevalence and severity of any side effects;
- efficacy and potential advantages compared to alternative treatments;
- the price we charge for our product candidates;
- interpretations of the results of our clinical trials;
- the willingness of physicians and healthcare organizations 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 willingness of the target patient population to pay for our products, including co-pays under their health coverage plans;
- the strength of marketing and distribution support; and
- the availability of third-party coverage and adequate reimbursement.

Failure to attain market acceptance among the medical community and third-party payors may have an adverse impact on our operations and profitability. Although certain of our employees have commercialization experience, as a company we currently have only limited commercial capabilities. We may not be able to attract and retain qualified personnel to serve in our sales and marketing organization to effectively support our commercialization activities. If we are not successful in commercializing Andexxa, Bevyxxa or current or potential future product candidates in the event they receive regulatory approval, our future product revenue will suffer and we may incur significant additional losses.

We currently have limited sales and distribution personnel and are in the initial stages of developing marketing capabilities for Andexxa and Bevyxxa. If we are unable to develop effective sales, marketing and distribution capabilities on our own or through collaborations or other marketing partners, we will not be successful in commercializing these products or our other future products.

We are in the early stages of developing our sales and marketing infrastructure and have limited history of selling, marketing or distributing therapeutic products. To achieve commercial success for Andexxa, Bevyxxa or any current or potential product candidate, we must continue to 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 our products globally. 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 Andexxa or a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

We also may not be successful entering into arrangements with third parties to sell and market our product candidates or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively, which could damage our reputation. If we do not establish sales and marketing capabilities successfully, either on our

own or in collaboration with third parties, we will not be successful in commercializing our product candidates.

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

The development and commercialization of new therapeutic products is highly competitive. We face competition with respect to commercializing Bevyxxa and developing our current product candidates, and we 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 anticoagulants for use in various disease states, including injectable anticoagulants for the prevention of VTE in acutely ill medical 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. Some of these competitors are or may be attempting to develop therapeutics for our target indications.

In addition, most 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 or maintain market share and undermine the value proposition that we might otherwise be able to offer to payors. Bevyxxa is indicated for the prophylaxis of VTE in adult patients hospitalized for an acute medical illness who are at risk for thromboembolic complications due to moderate or severe restricted mobility and other risk factors. The current standard of care for VTE prophylaxis in acute medically ill patients in the United States is a 6- to 14-day administration of enoxaparin, marketed as Lovenox<sup>®</sup> and also available in generic form. Enoxaparin is a low cost therapy that is widely accepted by physicians, patients and third-party payors. As a result, we may face difficulties in marketing Bevyxxa in this patient population. Additionally 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 acutely ill medical patients. For example, we believe that Bayer and Janssen are near completion of their Phase 3 MARINER clinical trial to evaluate the safety and efficacy of rivaroxaban for up to 45 days post hospital discharge (after enoxaparin in hospital) to reduce the risk of symptomatic VTE in medical ill patients. If the MARINER trial is successful, Bevyxxa is expected to face increased competition in the marketplace from a drug which would be used as a different treatment strategy (post discharge only) in an overlapping patient population. Such treatment strategy would not require physicians, patients and third-party payors to replace enoxaparin with a higher priced therapy in the hospital.

While there are no therapies other than Andexxa approved specifically as antidotes for fXa inhibitors, we are aware of at least one drug candidate that has been studied in early stage clinical trials as a potential antidote to fXa inhibitors. In addition, in December 2014, Bristol-Myers Squibb Company and Pfizer Inc. announced that a clinical trial of 15 healthy human subjects demonstrated that two 4-factor PCCs reversed the steady-state pharmacodynamic effects of Eliquis (apixaban) in several coagulation assessments. Andexxa may compete with other currently approved treatments designed to enhance coagulation, such as FFP, PCCs, rFVIIa or whole blood. Although there is no clinical evidence supporting the use of such treatments in patients taking fXa 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 fXa inhibitors.

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

\*We obtained regulatory approval of Andexxa in the United States through an Accelerated Approval process. Continued approval may be contingent upon post-marketing study results to demonstrate an improvement in hemostasis in patients.

The Accelerated Approval regulations allow drugs that are being developed to treat an unmet medical need to be approved substantially based on evidence of an effect on a surrogate biomarker endpoint that is considered reasonably likely to predict clinical benefit rather than a clinical endpoint such as survival or irreversible morbidity. Our approval of Andexxa was supported by data from two Phase 3 ANNEXA studies (ANNEXA-R and ANNEXA-A), which evaluated the safety and efficacy of Andexxa in reversing the anticoagulant activity of the Factor Xa inhibitors rivaroxaban and apixaban in healthy volunteers, and patient data from our ongoing ANNEXA-4 single-arm, open-label study in patients with major bleeding. However, these studies have inherent limitations as compared with a randomized controlled trial. As a condition to approval, the FDA has required us to conduct a post-marketing randomized controlled trial of Andexxa. This trial will randomize patients to receive either Andexxa or the type of care the enrolling institution would provide in the absence of Andexxa. This study is scheduled to be initiated in 2019 and be reported in 2023. We expect the practical implementation and ethical considerations of a randomized controlled trial for Andexxa to present challenges, and we cannot be sure that we will be able to successfully conduct and enroll such a trial in a manner satisfactory to the FDA or within the time period required by the FDA. Further, if the randomized controlled trial is not successful, the FDA could terminate our marketing approval for Andexxa.

\*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. 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:

- 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;
- 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 cost of clinical studies or the manufacturing of our product candidates may be greater than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or meet their contractual obligations to us in a timely manner, or at all;
- we might have to suspend or terminate clinical studies of our product candidates for various reasons, including unanticipated serious side effects, other unexpected characteristics or unacceptable health risks;
- 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 may also increase if we experience delays in testing or approvals. We do not know whether any anticipated clinical studies will begin as planned, or whether anticipated or ongoing clinical studies will need to be restructured or will be completed on schedule, or at all. Significant clinical study delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which would impair our ability to commercialize our product candidates and harm our business and results of operations.

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 proof-of concept studies of Andexxa, our Phase 3 ANNEXA-A (apixaban) and ANNEXA-R (rivaroxaban) studies, and the interim results from our ANNEXA-4 patient study may not be predictive of success in our planned randomized controlled trial of Andexxa. Reversal of fXa inhibitor anticoagulation by Andexxa may not ensure hemostasis, for example, if damage to the blood vessel integrity is severe, bleeding may not stop following the administration of Andexxa. We also do not know how the results from our ANNEXA trials will translate into clinical use in patients or the effect of repeat doses. Finally, the favorable interim results from our Phase 2a proof-of-concept study for cerdulatinib in patients with NHL, or CLL, who have failed or relapsed on existing marketed therapies or products in development, including patients with identified mutations, may not be confirmed in future clinical studies or predictive of final results.



\*Failure to obtain regulatory approvals in foreign jurisdictions will prevent us from marketing our products internationally. Following the negative opinion by the CHMP for Bevyxxa, it is unlikely that we will obtain marketing approval to commercialize Bevyxxa in the EU at this time. While the CHMP has communicated a positive trend vote for Andexxa, the CHMP has requested additional data with respect to Andexxa and extended the timetable for the review. This will delay the CHMP opinion and could even reduce the likelihood of a favorable opinion.

In order to market Bevyxxa or 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 EMA or the competent authorities of the member states of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

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

In February 2018, the CHMP communicated a negative trend vote for the MAA for Bevyxxa for the prevention of VTE in adult patients hospitalized for an acute medical illness with risk factors for VTE. In March 2018, the CHMP issued a negative opinion for Bevyxxa. Although we have appealed this opinion and the CHMP has agreed to re-examine our MAA, we have not included any additional clinical data in our appeal and can provide no assurance that the CHMP will reverse its negative opinion.

The CHMP noted that uncertainties remained regarding a positive benefit risk analysis for Bevyxxa, which was not supported by a second confirmatory study, biological plausibility for Bevyxxa in another approved indication or external support within the class from other Factor Xa inhibitors with respect to the acute medically ill population. Failure to obtain marketing approval of Bevyxxa in the EU will reduce the commercial potential of Bevyxxa and could also have a negative impact on our efforts to commercialize and obtain market acceptance for Bevyxxa in the US market.

In February 2018, the CHMP also communicated a positive trend vote on the MAA for Andexxa. While a positive trend vote is considered a positive (although not determinative) indicator for the potential outcome for the formal CHMP opinion, the CHMP also requested additional data with respect to Andexxa which will delay the date of the formal opinion until the fourth quarter of 2018. In addition, at this time we cannot be certain that we will be able to provide the additional data to the CHMP in a form satisfactory to the CHMP, or that the additional data will support a positive opinion. Failure to obtain marketing approval of Andexxa in the EU would reduce the commercial potential of Andexxa.

If serious adverse side effects are identified with respect to any of our product candidates or either of our approved products, we may need to abandon our development of that product candidate or discontinue sale of that product.

It is impossible to guarantee when or if any of our product candidates will prove safe enough to receive regulatory approval. In addition, there can be no assurance that our clinical studies will identify all relevant safety issues. Known

or previously unidentified adverse side effects can adversely affect regulatory approvals or marketing of approved products. In such an event, we might need to abandon marketing efforts or development of that product or product candidate or enter into a partnership to continue development.

For example, Bevyxxa, like all currently marketed inhibitors of fXa, carries some risk of life-threatening bleeding. In addition, patients taking Bevyxxa in our Phase 2 studies had an increased rate of gastrointestinal issues, such as diarrhea, nausea and vomiting, and other side effects such as back pain, dizziness, headaches, rashes and insomnia as compared to subjects taking a placebo or an active comparator.

While no serious adverse side effects have been observed in our completed healthy patient studies with Andexxa, there is a risk that adverse side effects could be observed through our ANNEXA-4 patient study results, our post-marketing randomized controlled trial of Andexxa, additional clinical experience or repeat doses that are determined to have been caused by Andexxa. Some protein-based biologics have encountered problems with immunogenicity, that is, their tendency to trigger an unwanted immune response against themselves. To date, no neutralizing antibodies against Andexxa or antibodies to fXa have been detected; however there is still a risk that such antibodies could be identified through our ANNEXA-4 patient study results, additional clinical experience or from repeat doses. In addition, in our ANNEXA-4 patient trial, reversing the anticoagulant activity of fXa inhibitors in patients with underlying medical conditions requiring anticoagulation has been associated with thromboembolic events, ischemic events, cardiac arrest and sudden deaths, and the FDA has included a boxed warning in the Andexxa label to this effect.

If a regulatory agency discovers adverse events of unanticipated severity or frequency it may impose restrictions on that product or us, including requiring withdrawal of the product from the market. Among other legal and administrative actions, a regulatory agency may:

- mandate modifications to product labelling or promotional materials or require us to provide corrective information to healthcare practitioners;
- suspend any regulatory approvals;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements to approved applications filed by us, our partners or our potential future partners;
- impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products or require a product recall.

In addition, the occurrence of any of the foregoing, even if promptly remedied, could negatively impact the perception of us or the relevant product among the medical community, patients or third party payors.

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

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

\*The FDA's approval of Andexxa was limited to patients treated with rivaroxaban and apixaban, when reversal of anticoagulation is needed due to life-threatening or uncontrolled bleeding, and additional clinical studies and regulatory applications will be required to expand Andexxa indications and we can provide no assurances that such clinical studies or regulatory applications will be successful.

We are developing Andexxa as a universal antidote for patients receiving a fXa inhibitor anticoagulant when reversal of anticoagulation is needed, such as in life-threatening or uncontrolled bleeding or for emergency surgery/urgent procedures. Our approval of Andexxa was limited to patients treated with rivaroxaban and apixaban, when reversal of anticoagulation is needed due to life-threatening or uncontrolled bleeding. Our studies have also not yet included patients requiring emergency surgery or urgent procedures and we do not anticipate obtaining this indication without clinical data. We expect that we will also be required to provide additional clinical data to support addition to our label of other fXa inhibitors, including Bevyxxa, edoxaban and enoxaparin. Additional clinical studies will require additional time and expense and may not prove successful. Limitations in our label for Andexxa will reduce the number of patients for whom Andexxa is indicated and could reduce the size of the anticipated market and our financial prospects. In addition, our label for Andexxa includes a boxed warning that treatment with Andexxa has been associated with serious and life threatening adverse events, thromboembolic events, ischemic events, cardiac arrest and sudden deaths. This boxed warning may adversely impact market acceptance and the commercial potential of Andexxa. There can be no assurance that further clinical experience will provide a basis to remove this boxed warning.

\*There are risks associated with scaling up manufacturing to commercial scale. Our commercial manufacturing strategy for Andexxa is particularly complex and challenging and we have also experienced manufacturing challenges in the past in connection with validating the commercial manufacturing process for Bevyxxa. If our manufacturers are unable to manufacture our products on a commercial scale or scale to increased production, this will likely delay regulatory approval and slow commercialization and could materially adversely affect our results of operations and

growth prospects.

There are risks associated with scaling up manufacturing to commercial volumes including, among others, cost overruns, technical problems with process scale-up, process reproducibility, stability issues, lot consistency and timely availability of raw materials. There is no assurance that our manufacturer will be able to manufacture our products 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 we and our manufacturers are unable to produce sufficient quantities of our products for commercialization, either on a timely basis or at all, our commercialization efforts would be impaired, which would have a material adverse effect on our business, financial condition, results of operations and growth prospects.

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We have encountered uncertainties and risks associated with scaling up the manufacturing for Andexxa. The manufacture of biologics involves complex processes, typically including developing cell lines or cell systems to produce the biologic, growing large quantities of such cells and harvesting and purifying the biologic produced by them. The cost to manufacture biologics is generally far higher than traditional small molecule chemical compounds, and the manufacturing process is more complex and can be difficult to reproduce. Even though we have completed our process validation campaign for Generation 2 commercial scale manufacturing, there is no guarantee we will be successful in obtaining regulatory approval for this process. Due to the high cost to manufacture Andexxa and the inherent uncertainty related to manufacturing costs, there is a relatively greater risk that Andexxa may not be commercially viable.

We have received commercial approval from the FDA for Andexxa based on Generation 1 supply from CMC Biologics. However, our Generation 1 manufacturing process was designed to produce Andexxa for our clinical studies on a small scale and is capable of manufacturing only limited supply to support a commercial launch in relation to projected demand. We are currently discussing options with the FDA and our commercial manufacturing organizations for expanding commercial supply post-approval. Commercial supply of Andexxa at launch will be limited to our Generation 1 supply until such time as we can obtain approval for Generation 2 material.

In order to obtain FDA approval of Generation 2 material produced by Lonza, the vendor's manufacturing facility will need to pass a pre-approval regulatory inspection and we will need to demonstrate that such material is comparable to the clinical material we previously used and material produced in our Generation 1 process. Demonstrating comparability can require significant pre-clinical and clinical studies. The material may also be considered a new biological entity and a new clinical program, possibly commencing with Phase 1, and a full BLA submission may be required for approval, resulting in additional time and expense. If we are not able to establish a commercial-scale manufacturing process for Andexxa in a timely manner, or at all, our business, financial condition, results of operations and growth prospects would be materially adversely affected.

#### 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. Our reliance on these third parties for clinical development activities reduces our control over these activities but does not relieve us of our responsibilities. 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 Andexxa, Bevyxxa or 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 Andexxa, Bevyxxa and our product candidates for us. If one of our suppliers or manufacturers fails to perform adequately or fulfill our needs, we may be required to incur significant costs and devote significant efforts to find new suppliers or manufacturers. We may also face significant delays in the development and commercialization of our product candidates.

We do not own facilities for clinical-scale or commercial manufacturing of our product candidates and we rely on third-party suppliers to manufacture Andexxa, Bevyxxa and our product candidates. For example, we have entered into a manufacturing agreement with Hovione for the manufacture of Bevyxxa and expect to rely on this manufacturing organization to supply Bevyxxa for U.S. commercial launch and, if approved by the EMA, the EU launch. If Hovione fails for any reason to deliver adequate quantities of Bevyxxa, the commercial launch of Bevyxxa will be delayed or disrupted. We have contracted with CMC Biologics to manufacture Andexxa bulk drug substance to support our U.S. commercial launch, and we have engaged Lonza to develop a new, higher-capacity and lower cost process for Andexxa bulk drug substance in order to support our broader, worldwide commercialization strategy. We

also rely or expect to rely on other third party providers for raw materials, drug substance and drug product manufacturing, packaging, labeling and supply chain distribution. If we and our suppliers cannot agree to the terms and conditions for them to provide the drug supply necessary for our clinical and commercial needs, or if any single source supplier breaches an agreement with us, or terminates the agreement in response to an alleged breach by us or otherwise becomes unable to fulfill its supply obligations, we would not be able to manufacture and distribute the product candidate until a qualified alternative supplier is identified, which could also significantly disrupt, delay the development of, and impair our ability to commercialize, our product candidates.

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

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

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

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

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



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

- collaborators have significant discretion in determining the efforts and resources that they will apply to any such collaborations;
  - collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs based on clinical study results, changes in their strategic focus due to the acquisition of competitive products, availability of funding or other external factors, such as a business combination that diverts resources or creates competing priorities;
  - collaborators may delay clinical studies, provide insufficient funding for a clinical study program, stop a clinical study, abandon a product candidate, repeat or conduct new clinical studies or require a new formulation of a product candidate for clinical testing;
  - collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates;
  - a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to their marketing and distribution;
  - collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;
  - disputes may arise between us and a collaborator that causes the delay or termination of the research, development or commercialization of our product candidates or that results in costly litigation or arbitration that diverts management attention and resources;
  - collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates; and
  - collaborators may own or co-own intellectual property covering our products that results from our collaborating with them, and in such cases, we would not have the exclusive right to commercialize such intellectual property.
- Any termination or disruption of our collaboration with potential collaborators could result in delays in the development and commercialization of our product candidates, increases in our costs to develop and commercialize the product candidate, or the termination of development of a product candidate.

## RISKS RELATED TO THE OPERATION OF OUR BUSINESS

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

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

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

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

Over the next several years, we expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug development, regulatory affairs, quality, commercial compliance, medical affairs, and sales and marketing. To manage our anticipated future growth, we must continue to implement and improve our managerial,

operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The physical expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

We incur significant costs as a result of operating as a public company, and our management is required to devote substantial time to existing and new public company compliance and reporting regulations.

As a public company, we incur significant legal, accounting and other expenses. For example, the Sarbanes-Oxley Act, and rules of the SEC and those of The Nasdaq Stock Market, or the Nasdaq, have imposed various requirements on public companies including requiring establishment and maintenance of effective disclosure and financial controls. Our management and other personnel have and will need to continue to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations are continuously being revised, have increased and will continue to increase our legal and financial compliance costs and will make some activities more time-consuming and costly.

The Sarbanes-Oxley Act requires, among other things, that we maintain effective internal control over financial reporting and disclosure controls and procedures. In particular, we must perform system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting, as required by Section 404 of the Sarbanes-Oxley Act. In addition, we are required to have our independent registered public accounting firm attest to the effectiveness of our internal control over financial reporting. Our compliance with Section 404 of the Sarbanes-Oxley Act, as applicable, requires us to incur substantial accounting expense and expend significant management efforts. We currently do not have an internal audit group, and we will need to continue to hire additional accounting and financial staff with appropriate public company experience and technical accounting knowledge. If we 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 of the Sarbanes-Oxley Act, as applicable, requires us to be able to prepare timely and accurate financial statements. We expect that we will need to continue to improve existing, and implement new operational and financial systems, procedures and controls to manage our business effectively. Any delay in the implementation of, or disruption in the transition to, new or enhanced systems, procedures or controls, may cause our operations to suffer and we may be unable to conclude that our internal control over financial reporting is effective and to obtain an unqualified report on internal controls from our auditors as required under Section 404 of the Sarbanes-Oxley Act. If we fail to maintain an effective system of internal control over financial reporting, we may not be able to accurately report our financial results, and current and potential stockholders may lose confidence in our financial reporting. This, in turn, could have an adverse impact on trading prices for our common stock, and could adversely affect our ability to access the capital markets.

\*Product liability lawsuits against us could cause us to incur substantial liabilities and to limit sales of Bevyxxa and 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 the commercial manufacturing, distribution and sale of Andexxa and Bevyxxa. For example, the manufacturers of currently marketed fXa inhibitors and other manufacturers of anticoagulants have faced substantial litigation due to certain alleged bleeding risks. In addition, in our ANNEXA-4 patient trial, reversing the anticoagulant

activity of fXa inhibitors in patients with underlying medical conditions requiring anticoagulation has been associated with thromboembolic events, ischemic events, cardiac arrest and sudden deaths, and the FDA has included a boxed warning in the Andexxa label to this effect. If we cannot successfully defend ourselves against claims that Andexxa, Bevyxxa or 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 Andexxa, Bevyxxa or 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;

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• loss of revenue; and  
• the inability to commercialize any products that we may develop.

We currently carry \$15.0 million in product liability insurance coverage, which may not be adequate to cover all liabilities that we may incur. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

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

Because we have limited financial and managerial resources, we focus on research programs and product candidates for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products.

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

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

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

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

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

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

natural or manmade disaster.

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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;
  - differing payor reimbursement regimes, governmental payors or patient self-pay systems and price control;
- 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 Andexxa and Bevyxxa development, we are currently utilizing certain suppliers outside of the United States, which subjects us to certain of the above risks.

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 known system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our drug development programs. For example, the loss of clinical study data from completed or ongoing clinical studies for any of our product candidates could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed.

## RISKS RELATED TO INTELLECTUAL PROPERTY

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

We are a party to intellectual property license agreements with third parties, including with respect to Bevyxxa, cerdulatinib, one of our selective Syk inhibitors, and our PCSK9 program, and we 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.

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

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

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

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

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

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

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

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

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

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the proprietary rights or intellectual property of third parties. We may become party to, or be threatened with, future adversarial proceedings or litigation regarding intellectual property rights with respect to our products and technology, including interference proceedings before the USPTO. An interference proceeding is a proceeding before the USPTO to determine the priority among multiple patents or patent applications. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future. If we are found to infringe a third-party's intellectual property rights, we could be required to obtain a license from such third-party to continue developing and marketing our products and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all.

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

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

In addition to our patented technology and products, we rely upon trade secrets, including unpatented know-how, technology and other proprietary information to develop and maintain our competitive position, which we seek to protect, in part, by confidentiality agreements with our employees and our collaborators and consultants. We also have agreements with our employees and 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 proceedings could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

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

## RISKS RELATED TO GOVERNMENT REGULATION

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

The research, testing, manufacturing, labeling, approval, selling, import, export, marketing and distribution of drug products are subject to extensive regulation by the FDA and other regulatory authorities in the United States and other countries, which regulations differ from country to country. We will not be permitted to market our product candidates in the United States until we receive approval of an NDA or a BLA, from the FDA. Obtaining approval of an NDA or BLA can be a lengthy, expensive and uncertain process that may not be successful. In addition, failure to comply with FDA and other applicable U.S. and foreign regulatory requirements may subject us to administrative or judicially imposed sanctions, including the following:

- warning letters;
- civil or criminal penalties and fines;
- injunctions;
- suspension or withdrawal of regulatory approval;
- suspension of any ongoing clinical studies;
- voluntary or mandatory product recalls and publicity requirements;
- refusal to accept or approve applications for marketing approval of new drugs or biologics or supplements to approved applications submitted by us;
- restrictions on operations, including costly new manufacturing requirements; or
- seizure or detention of our products or import bans.

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

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

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

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



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

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

The regulations that govern marketing approvals, pricing and reimbursement for new therapeutic products vary widely from country to country. Some countries, including EU member countries, require approval of the sale price of a product before it can be marketed. In many countries, including EU member 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. In some foreign markets, including the EU member countries, current standard of care and/or competitive products may be used as a benchmark or reference to determine pricing and reimbursement level for novel products such as Andexxa and Bevyxxa. To the extent that comparators are available at lower prices than our anticipated pricing for Andexxa or Bevyxxa, the pricing and reimbursement level of our products in the EU could be negatively impacted. 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, or even reduce the commercial viability of the product to an extent that prevents the launch altogether.

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

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

There may be significant delays in obtaining reimbursement for approved products, and coverage may be more limited than the purposes for which the product is approved by the FDA or regulatory authorities in other countries. Moreover, eligibility for reimbursement does not imply that any product will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Interim payments for new products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Payment rates

may vary according to the use of the product and the clinical setting in which it is used, may be based on payments allowed for lower cost products that are already reimbursed and may be incorporated into existing payments for other services. Net prices for products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of products from countries where they may be sold at lower prices than in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Our inability to promptly obtain coverage and profitable payment rates from both government funded and private payors for our existing or 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.



Healthcare reform measures could hinder or prevent the commercial success of Andexxa, Bevyxxa or our product candidates.

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 Reconciliation Act, collectively, the Affordable Care Act, was enacted in 2010. The Affordable Care Act contains a number of provisions, including those governing enrollment in federal healthcare programs, reimbursement changes and fraud and abuse measures, all of which will impact existing government healthcare programs and will result in the development of new programs. The Affordable Care Act, among other things:

- imposes a non-deductible annual fee on pharmaceutical manufacturers or importers who sell “branded prescription drugs,” effective 2011;
- increases the minimum level of Medicaid rebates payable by manufacturers of brand-name drugs from 15.1% to 23.1%, effective 2011;
- expanded Medicaid drug rebates to cover drugs paid by Medicaid managed care organizations;
- changes the Medicaid rebate rates for line extensions or new formulations of oral solid dosage form;
- expands the types of entities eligible for the “Section 340B discounts” for outpatient drugs;
- requires manufacturers to participate in a coverage gap discount program, under which they must agree to offer 50% (and 70% commencing January 1, 2019) 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.

Legislative changes to or regulatory changes under the Affordable Care Act remain possible and appear likely in the 115th U.S. Congress and under the current administration. In addition, since January 2017, the President has signed two Executive Orders and other directives designed to delay the implementation of certain provisions of the Affordable Care Act or otherwise circumvent some of the requirements for health insurance mandated by the Affordable Care Act. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the Affordable Care Act. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the Affordable Care Act have been signed into law. The Tax Cuts and Jobs Act of 2017 includes a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the Affordable Care Act on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the “individual mandate”. Additionally, a January 22, 2018 continuing resolution on appropriations for fiscal year 2018 delayed the implementation of certain Affordable Care Act-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amends the Affordable Care Act, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole”. We cannot assure that the Affordable Care Act, as currently enacted or as amended in the future, will not adversely affect our business and financial results and we cannot predict how future federal or state legislative or administrative changes relating to healthcare reform will affect our business.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. For example, the Budget Control Act of 2011, or Budget Control Act, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee did not achieve a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, which

triggered the legislation's automatic reduction to several government programs, including aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, starting in April 2013, and due to subsequent legislative amendments to the statute, including the BBA, will remain in effect through 2027 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, or the ATRA, among other things, 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.

Further, there has been heightened governmental scrutiny in the United States of pharmaceutical pricing practices in light of the rising cost of prescription drugs and biologics. Such scrutiny has resulted in several recent congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for products. At

the federal level, the administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

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

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

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

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

- the federal Anti-Kickback Statute, which prohibits, among other things, any person or entity from knowingly and willfully offering, soliciting, receiving or providing remuneration, directly or indirectly, in exchange for or to induce either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs, such as the Medicare and Medicaid programs;
- the federal Physician Payments Sunshine Act or Open Payments Program provisions and the implementing regulations which will require, among other things, extensive tracking of physician and teaching hospital payments, maintenance of a payments database, and public reporting of the payment data;
- the federal False Claims Act, which prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, false claims, or knowingly using false statements, to obtain payment from the federal government;
- federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the Foreign Corrupt Practices Act and similar statutes and regulations in foreign jurisdictions, which makes it unlawful for certain classes of persons and entities to make payments to foreign government officials to assist in obtaining or retaining business;
- the federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information;

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the Drug Quality and Security Act which requires manufacturers and other distribution parties to create systems to trace certain prescription drugs as they are distributed in the United States; 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.

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The Affordable Care Act, among other things, amends the intent requirement of the federal Anti-Kickback Statute and criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the Affordable Care Act provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal 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 substantial penalties, including civil and criminal penalties, damages, fines, possible exclusion from participation in Medicare, Medicaid, and other federal healthcare programs, integrity oversight and reporting obligations to resolve allegations of non-compliance with these laws, 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:

- our ability to meet the expectations of investors related to the commercialization of Andexxa and Bevyxxa;
- regulatory actions or decisions affecting Andexxa or Bevyxxa, including the timing and outcome of any potential future FDA or EMA decision, or other products or product candidates, including those of our competitors;
- inaccurate sales or cash forecasting of Andexxa or Bevyxxa;
- the timing and amount of revenues generated from sale of Andexxa or Bevyxxa;
- changes in laws or regulations applicable to Andexxa or Bevyxxa;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures or capital commitments;
- results of clinical trials or regulatory actions with respect to our products or product candidates;
- 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. In addition, following our update call on September 5, 2017, at least three plaintiffs' securities litigation firms publicly announced that they are investigating potential securities fraud claims that they may wish to make against us. 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.

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 forecasts of analysts, our stock price will likely decline.

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

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

- our board of directors is divided into three classes with staggered three-year terms which may delay or prevent a change of our management or a change in control;
- our board of directors has the right to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death or removal of a director, which prevents stockholders from being able to fill vacancies on our board of directors;
- our stockholders may not act by written consent or call special stockholders' meetings; as a result, a holder, or holders, controlling a majority of our capital stock would not be able to take certain actions other than at annual stockholders' meetings or special stockholders' meetings called by the board of directors, the chairman of the board, the chief executive officer or the president;
- our certificate of incorporation prohibits cumulative voting in the election of directors, which limits the ability of minority stockholders to elect director candidates;
- stockholders must provide advance notice and additional disclosures in order to nominate individuals for election to the board of directors or to propose matters that can be acted upon at a stockholders' meeting, which may discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of our company; and
- our board of directors may issue, without stockholder approval, shares of undesignated preferred stock; the ability to issue undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to acquire us.

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





Our 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 or discourage third parties from seeking business combinations.

Our executive officers are parties to agreements that contain change in control and severance provisions providing for aggregate cash payments of up to approximately \$4.8 million for severance and other benefits and acceleration of vesting of equity awards with a value of approximately \$30.3 million as of December 31, 2017, based on the closing price of our common stock of \$48.68 on such date in the event of a termination of employment in connection with a change in control of us. The accelerated vesting of equity awards could result in dilution to our existing stockholders and harm the market price of our common stock. The payment of these severance benefits could harm our financial condition and results. In addition, these potential severance payments may discourage or prevent third parties from seeking a business combination with us.

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

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

ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS

None.

ITEM 3. DEFAULTS UPON SENIOR SECURITIES

None.

ITEM 4. MINE SAFETY DISCLOSURES

Not Applicable.

ITEM 5. OTHER INFORMATION

None.

## ITEM 6. EXHIBITS

Exhibit Number	Exhibit Description	Incorporation By Reference			
		Form	SEC File No.	Exhibit	Filing Date
3.1	<u>Amended and Restated Certificate of Incorporation of Portola Pharmaceuticals, Inc.</u>	8-K	001-35935	3.1	5/28/2013
3.2	<u>Amended and Restated Bylaws of Portola Pharmaceuticals, Inc.</u>	8-K	001-35935	3.2	5/28/2013
4.1	Reference is made to Exhibits <u>3.1</u> through <u>3.2</u> .				
4.2	<u>Form of Common Stock Certificate of Portola Pharmaceuticals, Inc.</u>	S-1	333-187901	4.1	5/17/2013
4.5	<u>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.</u>	10-Q	001-35935	4.7	11/06/2013
4.6	<u>Warrant to Purchase Shares of Common Stock by and between Portola Pharmaceuticals, Inc., and HCP Life Science Assets TRS, LLC, dated December 15, 2006.</u>	10-Q	001-35935	4.8	11/06/2013
4.7	<u>Warrant to Purchase Shares of Common Stock by and between Portola Pharmaceuticals, Inc., and Bristow Investments, L.P., dated December 15, 2006.</u>	10-Q	001-35935	4.9	11/06/2013
10.5+*	<u>Amended and Restated Non-Employee Director Compensation Policy</u>				
31.1*	<u>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.</u>				
31.2*	<u>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.</u>				
32.1*	<u>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></u>				

101.INS\* XBRL Instance Document

101.SCH\* XBRL Taxonomy Extension Schema Document

101.CAL\* XBRL Taxonomy Extension Calculation Linkbase Document

101.DEF\* XBRL Taxonomy Extension Definition Linkbase Document

101.LAB\* XBRL Taxonomy Extension Label Linkbase Document

101.PRE\* XBRL Taxonomy Extension Presentation Linkbase Document

\*Filed herewith

+Management contract or compensatory plan

(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.

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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.

Date: May 10, 2018 By: /s/ William Lis  
William Lis  
Chief Executive Officer

Date: May 10, 2018 By: /s/ Mardi C. Dier  
Mardi C. Dier  
Chief Financial Officer