

VERTEX PHARMACEUTICALS INC / MA

Form 10-Q

April 27, 2018

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UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

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FORM 10-Q

☒ 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 000-19319

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Vertex Pharmaceuticals Incorporated

(Exact name of registrant as specified in its charter)

Massachusetts

04-3039129

(State or other jurisdiction of

(I.R.S. Employer

incorporation or organization)

Identification No.)

50 Northern Avenue, Boston, Massachusetts 02210

(Address of principal executive offices) (Zip Code)

Registrant's telephone number, including area code (617) 341-6100

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

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

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

Large accelerated filer ☒ Accelerated filer ☐ Non-accelerated filer ☐ Smaller reporting company ☐

Emerging growth company ☐

(Do not check if a smaller reporting 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 ☒

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date.

Common Stock, par value \$0.01 per share 254,830,232

Class

Outstanding at April 20, 2018

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

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| <p>"We," "us," "Vertex" and the "Company" as used in this Quarterly Report on Form 10-Q refer to Vertex Pharmaceuticals Incorporated, a Massachusetts corporation, and its subsidiaries.</p> <p>"Vertex," "KALYDECO," "ORKAMBI" and "SYMDEKO" are registered trademarks of Vertex. Other brands, names and trademarks contained in this Quarterly Report on Form 10-Q are the property of their respective owners.</p> |           |

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## Part I. Financial Information

## Item 1. Financial Statements

## VERTEX PHARMACEUTICALS INCORPORATED

## Condensed Consolidated Statements of Operations

(unaudited)

(in thousands, except per share amounts)

|                                                         | Three Months Ended<br>March 31, |            |
|---------------------------------------------------------|---------------------------------|------------|
|                                                         | 2018                            | 2017       |
| Revenues:                                               |                                 |            |
| Product revenues, net                                   | \$637,729                       | \$480,622  |
| Royalty revenues                                        | 1,356                           | 1,551      |
| Collaborative revenues                                  | 1,714                           | 232,545    |
| Total revenues                                          | 640,799                         | 714,718    |
| Costs and expenses:                                     |                                 |            |
| Cost of sales                                           | 71,613                          | 46,988     |
| Research and development expenses                       | 310,553                         | 273,563    |
| Sales, general and administrative expenses              | 129,808                         | 113,326    |
| Restructuring (income) expenses                         | (76)                            | ) 9,999    |
| Total costs and expenses                                | 511,898                         | 443,876    |
| Income from operations                                  | 128,901                         | 270,842    |
| Interest expense, net                                   | (11,097)                        | ) (16,765) |
| Other income (expense), net                             | 96,838                          | (544)      |
| Income before (benefit from) provision for income taxes | 214,642                         | 253,533    |
| (Benefit from) provision for income taxes               | (12,659)                        | ) 3,985    |
| Net income                                              | 227,301                         | 249,548    |
| Income attributable to noncontrolling interest          | (17,038)                        | ) (1,792)  |
| Net income attributable to Vertex                       | \$210,263                       | \$247,756  |

## Amounts per share attributable to Vertex common shareholders:

## Net income:

|         |        |        |
|---------|--------|--------|
| Basic   | \$0.83 | \$1.01 |
| Diluted | \$0.81 | \$0.99 |

## Shares used in per share calculations:

|         |         |         |
|---------|---------|---------|
| Basic   | 253,231 | 246,024 |
| Diluted | 258,526 | 248,700 |

The accompanying notes are an integral part of these condensed consolidated financial statements.

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VERTEX PHARMACEUTICALS INCORPORATED  
Condensed Consolidated Statements of Comprehensive Income  
(unaudited)  
(in thousands)

|                                                                                                                      | Three Months Ended<br>March 31, |            |
|----------------------------------------------------------------------------------------------------------------------|---------------------------------|------------|
|                                                                                                                      | 2018                            | 2017       |
| Net income                                                                                                           | \$227,301                       | \$249,548  |
| Changes in other comprehensive loss:                                                                                 |                                 |            |
| Unrealized holding (losses) gains on marketable securities, net of tax of zero and \$(1.0) million, respectively     | (460                            | ) 3,534    |
| Unrealized losses on foreign currency forward contracts, net of tax of \$0.3 million and \$0.9 million, respectively | (862                            | ) (6,681 ) |
| Foreign currency translation adjustment                                                                              | (2,729                          | ) (2,001 ) |
| Total changes in other comprehensive loss                                                                            | (4,051                          | ) (5,148 ) |
| Comprehensive income                                                                                                 | 223,250                         | 244,400    |
| Comprehensive income attributable to noncontrolling interest                                                         | (17,038                         | ) (1,792 ) |
| Comprehensive income attributable to Vertex                                                                          | \$206,212                       | \$242,608  |
| The accompanying notes are an integral part of these condensed consolidated financial statements.                    |                                 |            |

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## VERTEX PHARMACEUTICALS INCORPORATED

## Condensed Consolidated Balance Sheets

(unaudited)

(in thousands, except share and per share amounts)

|                                                                                                                                                                            | March 31,<br>2018 | December<br>31,<br>2017 |
|----------------------------------------------------------------------------------------------------------------------------------------------------------------------------|-------------------|-------------------------|
| Assets                                                                                                                                                                     |                   |                         |
| Current assets:                                                                                                                                                            |                   |                         |
| Cash and cash equivalents                                                                                                                                                  | \$1,995,893       | \$1,665,412             |
| Marketable securities                                                                                                                                                      | 481,124           | 423,254                 |
| Restricted cash and cash equivalents (VIE)                                                                                                                                 | 9,573             | 1,489                   |
| Accounts receivable, net                                                                                                                                                   | 327,294           | 281,343                 |
| Inventories                                                                                                                                                                | 117,346           | 111,830                 |
| Prepaid expenses and other current assets                                                                                                                                  | 109,886           | 165,635                 |
| Total current assets                                                                                                                                                       | 3,041,116         | 2,648,963               |
| Property and equipment, net                                                                                                                                                | 800,670           | 789,437                 |
| Intangible assets                                                                                                                                                          | 29,000            | 29,000                  |
| Goodwill                                                                                                                                                                   | 50,384            | 50,384                  |
| Other assets                                                                                                                                                               | 31,804            | 28,230                  |
| Total assets                                                                                                                                                               | \$3,952,974       | \$3,546,014             |
| Liabilities and Shareholders' Equity                                                                                                                                       |                   |                         |
| Current liabilities:                                                                                                                                                       |                   |                         |
| Accounts payable                                                                                                                                                           | \$74,062          | \$73,994                |
| Accrued expenses                                                                                                                                                           | 411,231           | 443,961                 |
| Capital lease obligations, current portion                                                                                                                                 | 16,919            | 22,531                  |
| Early access sales accrual                                                                                                                                                 | 268,446           | 232,401                 |
| Other liabilities, current portion                                                                                                                                         | 56,654            | 34,373                  |
| Total current liabilities                                                                                                                                                  | 827,312           | 807,260                 |
| Capital lease obligations, excluding current portion                                                                                                                       | 16,911            | 20,496                  |
| Deferred tax liability                                                                                                                                                     | 9,636             | 6,341                   |
| Construction financing lease obligation, excluding current portion                                                                                                         | 561,295           | 563,406                 |
| Advance from collaborator, excluding current portion                                                                                                                       | 79,537            | 78,431                  |
| Other liabilities, excluding current portion                                                                                                                               | 27,462            | 27,774                  |
| Total liabilities                                                                                                                                                          | 1,522,153         | 1,503,708               |
| Commitments and contingencies                                                                                                                                              |                   |                         |
| Shareholders' equity:                                                                                                                                                      |                   |                         |
| Preferred stock, \$0.01 par value; 1,000,000 shares authorized; none issued and outstanding                                                                                | —                 | —                       |
| Common stock, \$0.01 par value; 500,000,000 shares authorized; 254,934,949 and 253,253,362 shares issued; and 254,867,865 and 253,253,362 shares outstanding, respectively | 2,541             | 2,512                   |
| Additional paid-in capital                                                                                                                                                 | 7,314,369         | 7,157,362               |
| Accumulated other comprehensive loss                                                                                                                                       | (39,743 )         | (11,572 )               |
| Accumulated deficit                                                                                                                                                        | (4,876,111 )      | (5,119,723 )            |
| Total Vertex shareholders' equity                                                                                                                                          | 2,401,056         | 2,028,579               |
| Noncontrolling interest                                                                                                                                                    | 29,765            | 13,727                  |
| Total shareholders' equity                                                                                                                                                 | 2,430,821         | 2,042,306               |
| Total liabilities and shareholders' equity                                                                                                                                 | \$3,952,974       | \$3,546,014             |
| The accompanying notes are an integral part of these condensed consolidated financial statements.                                                                          |                   |                         |



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## VERTEX PHARMACEUTICALS INCORPORATED

## Condensed Consolidated Statements of Shareholders' Equity and Noncontrolling Interest

(unaudited)

(in thousands)

|                                                                                  | Common Stock |         | Additional<br>Paid-in<br>Capital | Accumulated<br>Other<br>Comprehensive<br>Income<br>(Loss) |                        | Total Vertex<br>Shareholders'<br>Equity | Noncontrolling<br>Interest | Total<br>Shareholders'<br>Equity |
|----------------------------------------------------------------------------------|--------------|---------|----------------------------------|-----------------------------------------------------------|------------------------|-----------------------------------------|----------------------------|----------------------------------|
|                                                                                  | Shares       | Amount  |                                  |                                                           | Accumulated<br>Deficit |                                         |                            |                                  |
| Balance at<br>December 31,<br>2016                                               | 248,301      | \$2,450 | \$6,506,795                      | \$ 21,173                                                 | \$(5,373,836)          | \$1,156,582                             | \$ 181,609                 | \$1,338,191                      |
| Cumulative<br>effect adjustment<br>for adoption of<br>new accounting<br>guidance | —            | —       | 9,371                            | —                                                         | (9,371 )               | —                                       | —                          | —                                |
| Other<br>comprehensive<br>loss, net of tax                                       | —            | —       | —                                | (5,148 )                                                  | —                      | (5,148 )                                | —                          | (5,148 )                         |
| Net income                                                                       | —            | —       | —                                | —                                                         | 247,756                | 247,756                                 | 1,792                      | 249,548                          |
| Issuance of<br>common stock<br>under benefit<br>plans                            | 590          | 9       | 31,019                           | —                                                         | —                      | 31,028                                  | —                          | 31,028                           |
| Stock-based<br>compensation<br>expense                                           | —            | —       | 69,790                           | —                                                         | —                      | 69,790                                  | —                          | 69,790                           |
| Other VIE<br>activity                                                            | —            | —       | —                                | —                                                         | —                      | —                                       | (616 )                     | (616 )                           |
| Balance at March<br>31, 2017                                                     | 248,891      | \$2,459 | \$6,616,975                      | \$ 16,025                                                 | \$(5,135,451)          | \$1,500,008                             | \$ 182,785                 | \$1,682,793                      |
| Balance at<br>December 31,<br>2017                                               | 253,253      | \$2,512 | \$7,157,362                      | \$(11,572 )                                               | \$(5,119,723)          | \$2,028,579                             | \$ 13,727                  | \$2,042,306                      |
| Cumulative<br>effect adjustment<br>for adoption of<br>new accounting<br>guidance | —            | —       | —                                | (24,120 )                                                 | 33,349                 | 9,229                                   | —                          | 9,229                            |
| Other<br>comprehensive<br>loss, net of tax                                       | —            | —       | —                                | (4,051 )                                                  | —                      | (4,051 )                                | —                          | (4,051 )                         |
| Net income                                                                       | —            | —       | —                                | —                                                         | 210,263                | 210,263                                 | 17,038                     | 227,301                          |
| Repurchases of<br>common stock                                                   | (67 )        | (1 )    | (11,250 )                        | —                                                         | —                      | (11,251 )                               | —                          | (11,251 )                        |
| Issuance of<br>common stock                                                      | 1,682        | 30      | 89,656                           | —                                                         | —                      | 89,686                                  | —                          | 89,686                           |

under benefit  
plans

|                                  |         |         |             |             |               |             |          |             |
|----------------------------------|---------|---------|-------------|-------------|---------------|-------------|----------|-------------|
| Stock-based compensation expense | —       | —       | 78,601      | —           | —             | 78,601      | —        | 78,601      |
| Other VIE activity               | —       | —       | —           | —           | —             | —           | (1,000 ) | (1,000 )    |
| Balance at March 31, 2018        | 254,868 | \$2,541 | \$7,314,369 | \$(39,743 ) | \$(4,876,111) | \$2,401,056 | \$29,765 | \$2,430,821 |

The accompanying notes are an integral part of these condensed consolidated financial statements.

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## VERTEX PHARMACEUTICALS INCORPORATED

## Condensed Consolidated Statements of Cash Flows

(unaudited)

(in thousands)

|                                                                                   | Three Months Ended<br>March 31, |              |
|-----------------------------------------------------------------------------------|---------------------------------|--------------|
|                                                                                   | 2018                            | 2017         |
| Cash flows from operating activities:                                             |                                 |              |
| Net income                                                                        | \$ 227,301                      | \$ 249,548   |
| Adjustments to reconcile net income to net cash provided by operating activities: |                                 |              |
| Stock-based compensation expense                                                  | 78,136                          | 69,439       |
| Depreciation expense                                                              | 16,343                          | 14,850       |
| Write-downs of inventories to net realizable value                                | 3,619                           | 3,787        |
| Deferred income taxes                                                             | 3,587                           | 1,212        |
| Impairment of property and equipment                                              | —                               | 1,946        |
| Unrealized gain on equity securities                                              | (95,458)                        | ) —          |
| Other non-cash items, net                                                         | 5,827                           | (5,152)      |
| Changes in operating assets and liabilities:                                      |                                 |              |
| Accounts receivable, net                                                          | (13,473)                        | ) (7,798)    |
| Inventories                                                                       | (8,208)                         | ) (7,894)    |
| Prepaid expenses and other assets                                                 | 25,482                          | (44,498)     |
| Accounts payable                                                                  | 2,154                           | 717          |
| Accrued expenses and other liabilities                                            | 7,347                           | (10,465)     |
| Net cash provided by operating activities                                         | 252,657                         | 265,692      |
| Cash flows from investing activities:                                             |                                 |              |
| Purchases of marketable securities                                                | (38,653)                        | ) (248,273)  |
| Maturities of marketable securities                                               | 94,365                          | 98,393       |
| Expenditures for property and equipment                                           | (29,279)                        | ) (11,099)   |
| Investment in equity securities                                                   | (21,500)                        | ) —          |
| Net cash provided by (used in) investing activities                               | 4,933                           | (160,979)    |
| Cash flows from financing activities:                                             |                                 |              |
| Issuances of common stock under benefit plans                                     | 88,403                          | 11,249       |
| Repurchase of common stock                                                        | (10,000)                        | ) —          |
| Payments on revolving credit facility                                             | —                               | (300,000)    |
| Advance from collaborator                                                         | 2,500                           | 5,000        |
| Payments on capital lease obligations                                             | (9,197)                         | ) (4,703)    |
| Proceeds related to construction financing lease obligation                       | 9,566                           | —            |
| Repayments of advanced funding                                                    | (1,182)                         | ) (994)      |
| Other financing activities                                                        | (1,134)                         | ) (117)      |
| Net cash provided by (used in) financing activities                               | 78,956                          | (289,565)    |
| Effect of changes in exchange rates on cash                                       | 1,656                           | 1,388        |
| Net increase (decrease) in cash and cash equivalents                              | 338,202                         | (183,464)    |
| Cash, cash equivalents and restricted cash—beginning of period                    | 1,667,526                       | 1,231,707    |
| Cash, cash equivalents and restricted cash—end of period                          | \$ 2,005,728                    | \$ 1,048,243 |
| Supplemental disclosure of cash flow information:                                 |                                 |              |
| Cash paid for interest                                                            | \$ 16,825                       | \$ 17,527    |
| Cash paid for income taxes                                                        | \$ 1,897                        | \$ 1,164     |
| Capitalization of costs related to construction financing lease obligation        | \$ 3,716                        | \$ 12,549    |

|                                                                  |         |          |
|------------------------------------------------------------------|---------|----------|
| Issuances of common stock from employee benefit plans receivable | \$2,124 | \$19,847 |
|------------------------------------------------------------------|---------|----------|

The accompanying notes are an integral part of these condensed consolidated financial statements.

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VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

A. Basis of Presentation and Accounting Policies

Basis of Presentation

The accompanying condensed consolidated financial statements are unaudited and have been prepared by Vertex Pharmaceuticals Incorporated (“Vertex” or the “Company”) in accordance with accounting principles generally accepted in the United States of America (“GAAP”).

The condensed consolidated financial statements reflect the operations of (i) the Company, (ii) its wholly-owned subsidiaries and (iii) consolidated variable interest entities (VIEs). All material intercompany balances and transactions have been eliminated. The Company operates in one segment, pharmaceuticals. As of September 30, 2017, the Company deconsolidated Parion Sciences, Inc. (“Parion”), a VIE the Company had consolidated since 2015. The Company's condensed consolidated statement of operations for the interim period ended March 31, 2018 excludes Parion. Please refer to Note B, “Collaborative Arrangements and Acquisitions,” in the the Company’s Annual Report on Form 10-K for the year ended December 31, 2017 that was filed with the Securities and Exchange Commission (the “SEC”) on February 15, 2018 (the “2017 Annual Report on Form 10-K”) for further information regarding the deconsolidation of Parion.

Certain information and footnote disclosures normally included in the Company’s annual financial statements have been condensed or omitted. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the financial position and results of operations for the interim periods ended March 31, 2018 and 2017.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full fiscal year. These interim financial statements should be read in conjunction with the audited financial statements for the year ended December 31, 2017, which are contained in the 2017 Annual Report on Form 10-K.

Use of Estimates

The preparation of condensed consolidated financial statements in accordance with GAAP requires management to make certain estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements, and the amounts of revenues and expenses during the reported periods. Significant estimates in these condensed consolidated financial statements have been made in connection with the calculation of revenues, inventories, research and development expenses, stock-based compensation expense, the fair value of intangible assets, goodwill, contingent consideration, noncontrolling interest, the consolidation and deconsolidation of VIEs, leases, the fair value of cash flow hedges, deferred tax asset valuation allowances and the provision for or benefit from income taxes. The Company bases its estimates on historical experience and various other assumptions, including in certain circumstances future projections that management believes to be reasonable under the circumstances. Actual results could differ from those estimates. Changes in estimates are reflected in reported results in the period in which they become known.

Recently Adopted Accounting Standards

Revenue Recognition

In 2014, the Financial Accounting Standards Board (“FASB”) issued Accounting Standards Update (“ASU”) No. 2014-09, Revenues from Contracts with Customers (Topic 606) (“ASC 606”). The new guidance became effective January 1, 2018. ASC 606 applies a more principles-based approach to recognizing revenue. Under ASC 606, revenue is recognized when a customer obtains control of promised goods or services and is recognized in an amount that reflects the consideration that an entity expects to receive in exchange for those goods or services. In addition, the standard requires disclosure of the nature, amount, timing, and uncertainty of revenue and cash flows arising from contracts with customers. The Company adopted ASC 606 on January 1, 2018 using the modified-retrospective adoption method for all contracts that were not completed as of the date of adoption. Under the modified-retrospective method, the cumulative effect of applying the standard was recognized within “Accumulated deficit” on the condensed

consolidated balance sheet as of January 1, 2018.

For all reporting periods, the Company has not disclosed the value of unsatisfied performance obligations for all product revenue contracts with an original expected length of one year or less, which is an optional exemption that is permitted under

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## VERTEX PHARMACEUTICALS INCORPORATED

## Notes to Condensed Consolidated Financial Statements

(unaudited)

the adoption rules.

Based on the Company's review of existing customer contracts as of January 1, 2018, the Company concluded that the only significant impact that the adoption of ASC 606 had on the Company's financial statements relates to shipments of ORKAMBI under early access programs in France. Prior to the adoption of ASC 606, the Company did not recognize revenue on the proceeds received from sales of ORKAMBI under early access programs in France because the price was not fixed or determinable based on the status of ongoing pricing discussions. As of January 1, 2018, the Company recorded a cumulative effect adjustment to its accumulated deficit of \$8.3 million related to the adoption of ASC 606, which primarily represented the Company's estimated amount of consideration it expects to retain related to these shipments that will not be subject to a significant reversal in amounts recognized, net of costs previously deferred related to these shipments. Please refer to Note B, "Revenue Recognition" for further information.

The Company concluded that the remaining \$6.9 million that was recorded as deferred revenue as of December 31, 2017 related to the Company's sale of its HIV protease inhibitor royalty stream in 2008 is not subject to ASC 606 because it was initially accounted for pursuant to ASC 470, Debt, which is not under the scope of ASC 606. The Company will continue to recognize the payment received as royalty revenues over the expected life of the collaboration agreement with GlaxoSmithKline plc based on the units-of-revenue method.

The cumulative effect of applying ASC 606 to the Company's contracts with customers that were not completed as of January 1, 2018 was as follows:

|                                            | Balance as<br>of<br>December<br>31, 2017 | Adjustments | Balance as<br>of<br>January 1,<br>2018 |
|--------------------------------------------|------------------------------------------|-------------|----------------------------------------|
| Assets                                     | (in thousands)                           |             |                                        |
| Accounts receivable, net                   | \$281,343                                | \$ 29,881   | \$311,224                              |
| Inventories                                | 111,830                                  | (90 )       | 111,740                                |
| Prepaid expenses and other current assets  | 165,635                                  | (17,166 )   | 148,469                                |
| Total assets                               | \$3,546,014                              | \$ 12,625   | \$3,558,639                            |
| Liabilities and Shareholders' Equity       |                                          |             |                                        |
| Accrued expenses                           | \$443,961                                | \$ 8,586    | \$452,547                              |
| Early access sales accrual                 | 232,401                                  | (7,273 )    | 225,128                                |
| Other liabilities, current portion         | 34,373                                   | 2,083       | 36,456                                 |
| Accumulated other comprehensive loss       | (11,572 )                                | 949         | (10,623 )                              |
| Accumulated deficit                        | (5,119,723 )                             | 8,280       | (5,111,443 )                           |
| Total liabilities and shareholders' equity | \$3,546,014                              | \$ 12,625   | \$3,558,639                            |

The impact of adoption on the Company's condensed consolidated balance sheet as of March 31, 2018 was as follows:

|                                           | As of March 31, 2018            |                                               |                                       |
|-------------------------------------------|---------------------------------|-----------------------------------------------|---------------------------------------|
|                                           | As Reported<br>under ASC<br>606 | Balances<br>without<br>Adoption of<br>ASC 606 | Effect of<br>Change<br>Higher/(Lower) |
| Assets                                    | (in thousands)                  |                                               |                                       |
| Accounts receivable, net                  | \$327,294                       | \$294,142                                     | \$ 33,152                             |
| Inventories                               | 117,346                         | 117,414                                       | (68 )                                 |
| Prepaid expenses and other current assets | 109,886                         | 128,818                                       | (18,932 )                             |
| Total assets                              | \$3,952,974                     | \$3,938,822                                   | \$ 14,152                             |
| Liabilities and Shareholders' Equity      |                                 |                                               |                                       |
| Accrued expenses                          | 411,231                         | 408,014                                       | 3,217                                 |

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|                                            |              |              |           |
|--------------------------------------------|--------------|--------------|-----------|
| Early access sales accrual                 | 268,446      | 278,957      | (10,511 ) |
| Other liabilities, current portion         | 56,654       | 48,026       | 8,628     |
| Accumulated other comprehensive loss       | (39,743 )    | (41,148 )    | 1,405     |
| Accumulated deficit                        | (4,876,111 ) | (4,887,524 ) | 11,413    |
| Total liabilities and shareholders' equity | \$3,952,974  | \$3,938,822  | \$ 14,152 |

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The impact of adoption on the Company's condensed consolidated statement of operations for the three months ended March 31, 2018 was as follows:

|                                   | Three Months Ended March 31, 2018  |                                                  |                                       |
|-----------------------------------|------------------------------------|--------------------------------------------------|---------------------------------------|
|                                   | As<br>Reported<br>under<br>ASC 606 | Balances<br>without<br>Adoption<br>of ASC<br>606 | Effect of<br>Change<br>Higher/(Lower) |
|                                   | (in thousands)                     |                                                  |                                       |
| Product revenues, net             | \$637,729                          | \$633,064                                        | \$ 4,665                              |
| Cost of sales                     | 71,613                             | 70,081                                           | 1,532                                 |
| Income from operations            | 128,901                            | 125,768                                          | 3,133                                 |
| Net income attributable to Vertex | \$210,263                          | \$207,130                                        | \$ 3,133                              |

Amounts per share attributable to Vertex common shareholders:

Net income:

|         |        |        |         |
|---------|--------|--------|---------|
| Basic   | \$0.83 | \$0.82 | \$ 0.01 |
| Diluted | \$0.81 | \$0.80 | \$ 0.01 |

ASC 606 did not have an aggregate impact on the Company's net cash provided by operating activities, but resulted in offsetting changes in certain assets and liabilities presented within net cash provided by operating activities in the Company's condensed consolidated statement of cash flows, as reflected in the above tables.

Equity Investments

In 2016, the FASB issued ASU No. 2016-01, Recognition and Measurement of Financial Assets and Financial Liabilities ("ASU 2016-01"). ASU 2016-01 amended guidance related to the recording of financial assets and financial liabilities. Under the amended guidance, equity investments (except those accounted for under the equity method of accounting or those that result in consolidation of an investee) are to be measured at fair value with changes in fair value recognized in net income (loss). However, an entity has the option to measure equity investments without readily determinable fair values (i) at fair value or (ii) at cost adjusted for changes in observable prices minus impairment. Changes in measurement under either alternative will be recognized in net income (loss). The amended guidance became effective January 1, 2018 and required the modified-retrospective adoption approach. As of January 1, 2018, the Company held publicly traded equity investments and equity investments accounted for under the cost method. In the first quarter of 2018, the Company recorded a \$25.1 million cumulative effect adjustment to "Accumulated deficit" related to its publicly traded equity investments equal to the unrealized gain, net of tax, that was recorded in "Accumulated other comprehensive loss" as of December 31, 2017. The adoption of ASU 2016-01 had no effect on the Company's equity investments accounted for under the cost method because the original cost basis of these investments was recorded on the Company's condensed consolidated balance sheet as of December 31, 2017. In the three months ended March 31, 2018, the Company recorded income of \$95.5 million to "Other income (expense), net," in its condensed consolidated statement of operations related to the change in fair value of its equity investments.

Stock-Based Compensation

In 2017, the FASB issued ASU 2017-09, Compensation — Stock Compensation (Topic 718) ("ASU 2017-09") related to the scope of stock option modification accounting to reduce diversity in practice and provide clarity regarding existing guidance. The new accounting guidance was effective January 1, 2018. The Company does not expect the adoption of ASU 2017-09 to have a significant effect on its condensed consolidated financial statements in future periods and had no impact in the three months ended March 31, 2018.

Goodwill

In 2017, the FASB issued ASU 2017-04, Intangibles — Goodwill and Other (Topic 350) (“ASU 2017-04”) related to measurements of goodwill. The amended guidance modifies the concept of impairment from the condition that exists when the carrying amount of goodwill exceeds its implied fair value to the condition that exists when the carrying amount of a reporting unit exceeds its fair value, which eliminates Step 2 from the goodwill impairment test. An entity would recognize

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an impairment charge for the amount by which the carrying amount exceeds the reporting unit's fair value; however, the loss recognized should not exceed the total amount of goodwill allocated to that reporting unit. The new accounting guidance is required for annual or interim goodwill impairment tests conducted after January 1, 2020. The Company early adopted this new guidance and will utilize this approach for annual and interim goodwill impairment tests conducted after January 1, 2018. The Company does not expect the adoption of this guidance to have a significant effect on its condensed consolidated financial statements.

**Intra-Entity Transfers**

In 2016, the FASB issued ASU No. 2016-16, Intra-Entity Transfers of Assets Other Than Inventory ("ASU 2016-16"). ASU 2016-16 removes the previous exception in GAAP prohibiting entities from recognizing current and deferred income tax expenses or benefits related to the transfer of assets, other than inventory, within the consolidated entity. The exception to defer the recognition of any tax impact on the transfer of inventory within the consolidated entity until it is sold to a third party remains unaffected. The amended guidance became effective January 1, 2018. In the first quarter of 2018, upon adoption of ASU 2016-16, the Company recorded a deferred tax asset and corresponding full valuation allowance of \$204.7 million equal to the unamortized cost of intellectual property transferred to the United Kingdom in 2014 multiplied by an appropriate statutory rate. There was no cumulative effect adjustment to accumulated deficit using the modified-retrospective adoption approach.

**Cash Flows - Restricted Cash**

In 2016, the FASB issued ASU No. 2016-18, Statement of Cash Flows (Topic 230) Restricted Cash ("ASU 2016-18"). ASU 2016-18 requires that a statement of cash flows explain the change during the period in the total of cash, cash equivalents and restricted cash. Therefore, amounts described as restricted cash should be included with cash and cash equivalents when reconciling the beginning of period and end of period amounts shown on the statement of cash flows. The amended guidance became effective January 1, 2018 and is effective on a retrospective basis. The cash, cash equivalents and restricted cash at the beginning and ending of each period presented in the Company's condensed consolidated statements of cash flows for the three months ended March 31, 2018 and 2017 consisted of the following balances from the Company's condensed consolidated balance sheets:

|                                                                        | Three Months Ended<br>March 31, 2018 |                  | Three Months Ended<br>March 31, 2017 |                  |
|------------------------------------------------------------------------|--------------------------------------|------------------|--------------------------------------|------------------|
|                                                                        | Beginning<br>of period               | End of<br>period | Beginning<br>of period               | End of<br>period |
|                                                                        | (in thousands)                       |                  |                                      |                  |
| Cash and cash equivalents                                              | \$1,665,412                          | \$1,995,893      | \$1,183,945                          | \$1,003,679      |
| Restricted cash and cash equivalents (VIE)                             | 1,489                                | 9,573            | 47,762                               | 44,564           |
| Prepaid expenses and other current assets                              | 625                                  | 262              | —                                    | —                |
| Cash, cash equivalents and restricted cash per statement of cash flows | \$1,667,526                          | \$2,005,728      | \$1,231,707                          | \$1,048,243      |

The Company's restricted cash is classified in "Prepaid expenses and other current assets" in its condensed consolidated balance sheets. The Company has recorded its VIE's cash and cash equivalents as "Restricted cash and cash equivalents (VIE)" because (i) the Company does not have any interest in or control over BioAxone's cash and cash equivalents and (ii) the Company's agreement with BioAxone does not provide for BioAxone's cash and cash equivalents to be used for the development of the asset that the Company licensed from BioAxone.

**Recently Issued Accounting Standards****Derivatives and Hedging**

In 2017, the FASB issued ASU 2017-09, Derivatives and Hedging (Topic 815) ("ASU 2017-09"). ASU 2017-09 helps simplify certain aspects of hedge accounting and enables entities to more accurately present their risk management activities in their financial statements. The new guidance is effective for annual periods beginning after December 15, 2018, including interim periods within those annual periods. Early adoption is permitted. The Company is in the

process of determining the expected effect on its condensed consolidated financial statements.

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Leasing

In 2016, the FASB issued ASU No. 2016-02, Leases (“ASU 2016-02”). ASU 2016-02 is applicable to leases that will be effective for the year ending December 31, 2019. Early adoption is permitted. ASU 2016-02 requires entities to recognize assets and liabilities for leases with lease terms of more than 12 months on the balance sheet and requires a modified-retrospective adoption approach. The Company is in the process of evaluating this guidance and determining the expected effect on its condensed consolidated financial statements; however, it anticipates that the amended guidance will result in the Company recording additional assets and corresponding liabilities on its condensed consolidated balance sheets. The Company has formed a project team to review its portfolio of existing leases and current accounting policies to identify and assess the potential differences that would result from applying the requirements of the new standard.

For a discussion of other recent accounting pronouncements please refer to Note A, “Nature of Business and Accounting Policies—Recent Accounting Pronouncements,” in the 2017 Annual Report on Form 10-K.

Summary of Significant Accounting Policies

The Company’s significant accounting policies are described in Note A, “Nature of Business and Accounting Policies,” in the 2017 Annual Report on Form 10-K. The Company has included its accounting policies related to accounting guidance that became effective January 1, 2018 in this Quarterly Report on Form 10-Q. The Company’s policy related to revenue recognition that has been updated pursuant to the adoption of ASC 606 is included in Note B, “Revenue Recognition,” and its policy related to marketable and equity securities is included below.

Marketable and Equity Securities

Effective January 1, 2018, the Company measures publicly traded corporate equity investments, which have readily available prices, at fair value with changes in fair value recognized in “Other income (expense), net,” each reporting period.

Effective January 1, 2018, the Company records privately issued corporate equity investments, which do not have readily determinable fair values, at cost, and adjusts for changes in observable prices minus impairment. Each reporting period the Company adjusts the carrying value of these investments if it observes that additional shares have been issued in an orderly transaction between market participants resulting in a price increase or decrease per share. Additionally, each reporting period the Company reviews these investments for impairment considering all available information to conclude whether an impairment exists. Changes in measurement for all corporate equity investments are recognized in “Other income (expense), net.”

B. Revenue Recognition

Pursuant to ASC 606, revenue is recognized by the Company when a customer obtains control of promised goods or services. The amount of revenue that is recorded reflects the consideration that the Company expects to receive in exchange for those goods or services. The Company applies the following five-step model in order to determine this amount: (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) the Company satisfies each performance obligation.

The Company only applies the five-step model to contracts when it is probable that the entity will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. Once a contract is determined to be within the scope of ASC 606 at contract inception, the Company reviews the contract to determine which performance obligations the Company must deliver and which of these performance obligations are distinct. The Company recognizes as revenues the amount of the transaction price that is allocated to the respective performance obligation when the performance obligation is satisfied or as it is satisfied. Generally, the Company's performance obligations are transferred to customers at a point in time, typically upon delivery.

Product Revenues, Net



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The Company sells its products principally to a limited number of specialty pharmacy and specialty distributors in the United States, which account for the largest portion of our total revenues, and international sales are made primarily to specialty distributors, retail chains as well as hospitals and clinics many of which are government owned or supported (collectively, its “Customers”). The Company’s Customers in the United States subsequently resell the products to patients and health care providers. In accordance with ASC 606, the Company recognizes net revenues from product sales when the Customer obtains control of the Company’s product, which typically occurs upon delivery to the Customer. The Company’s payment terms are approximately 30 days in the United States and consistent with prevailing practice in international markets.

Revenues from product sales are recorded at the net sales price, or “transaction price,” which includes estimates of variable consideration that result from (a) trade allowances, which include invoice discounts for prompt payment and Customer fees, (b) government and private payor rebates, chargebacks, discounts and fees, (c) reserves for expected product returns and (d) costs of co-pay assistance programs for patients, as well as other incentives for certain indirect customers. Reserves are established for the estimates of variable consideration based on the amounts earned or to be claimed on the related sales. The reserves are classified as reductions to “Accounts receivable, net” if payable to a Customer or “Accrued expenses” if the amount is payable to third-party. Where appropriate, the Company utilizes the expected value method to determine the appropriate amount for estimates of variable consideration based on factors such as the Company’s historical experience, current contractual and statutory requirements, specific known market events and trends, industry data and forecasted customer buying and payment patterns. The amount of variable consideration that is included in the transaction price may be constrained, and is included in net product revenues 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 the Company’s estimates. If actual results vary from the Company’s estimates, the Company adjusts these estimates, which would affect net product revenue and earnings in the period such variances become known.

Trade Allowances: The Company generally provides invoice discounts on product sales to its Customers for prompt payment and pays fees for distribution services, such as fees for certain data that Customers provide to the Company. The Company estimates that, based on its experience, its Customers will earn these discounts and fees, and deducts the full amount of these discounts and fees from its gross product revenues and accounts receivable at the time such revenues are recognized.

Rebates, Chargebacks, Discounts and Fees: The Company contracts primarily with government agencies (its “Third-party Payors”) so that products will be eligible for purchase by, or partial or full reimbursement from, such Third-party Payors. The Company estimates the rebates, chargebacks, discounts and fees it will provide to Third-party Payors and deducts these estimated amounts from its gross product revenues at the time the revenues are recognized. For each product, the Company estimates the aggregate rebates, chargebacks and discounts that it will provide to Third-party Payors based upon (i) the Company’s contracts with these Third-party Payors, (ii) the government-mandated discounts and fees applicable to government-funded programs, (iii) information obtained from the Company’s Customers and other third-party data regarding the payor mix for such product and (iv) historical experience.

Product Returns: The Company estimates the amount of each product that will be returned and deducts these estimated amounts from its gross revenues at the time the revenues are recognized. The Company’s Customers have the right to return unopened unprescribed packages, subject to contractual limitations. To date, product returns have been minimal and, based on inventory levels held by its Customers and its distribution model, the Company believes that returns of its products will continue to be minimal.

Other Incentives: Other incentives that the Company offers include co-pay mitigation rebates provided by the Company to commercially insured patients who have coverage and who reside in states that permit co-pay mitigation programs. Based upon the terms of the Company’s co-pay mitigation programs, the Company estimates average co-pay mitigation amounts for each of its products in order to establish appropriate accruals.

The Company makes significant estimates and judgments that materially affect the Company's recognition of net product revenues. The Company adjusts its estimated rebates, chargebacks and discounts based on new information, including information regarding actual rebates, chargebacks and discounts for its products, as it becomes available. Claims by third-

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party payors for rebates, chargebacks and discounts frequently are submitted to the Company significantly after the related sales, potentially resulting in adjustments in the period in which the new information becomes known. The Company's credits to revenue related to prior period sales have typically been approximately 1% or less of gross product revenues and primarily related to U.S. rebates, chargebacks and discounts.

Taxes collected from Customers relating to product sales and remitted to governmental authorities are excluded from revenues.

**French Early Access Programs**

Pursuant to ASC 605, Revenue Recognition, which was applicable until December 31, 2017, the Company only recognized revenues from product sales if it determined that the price was fixed or determinable at the time of delivery. If the Company determined that the price was not fixed or determinable, it deferred the recognition of revenues. If the Company was able to determine that the price was fixed or determinable, it recognized the net product revenues associated with the units.

The Company began distributing ORKAMBI through early access programs in France during the fourth quarter of 2015 and is engaged in ongoing pricing discussions regarding the final price for ORKAMBI in France. The Company's ORKAMBI net product revenues for 2017, 2016 and 2015 did not include any net product revenues from sales of ORKAMBI in France because the price was not fixed or determinable. The Company expects that the difference between the amounts collected based on the invoiced price and the final price for ORKAMBI in France will be returned to the French government.

As of March 31, 2018 and December 31, 2017, the Company's condensed consolidated balance sheet included \$268.4 million and \$232.4 million, respectively, classified as "Early access sales accrual" related to amounts collected in France as payment for shipments of ORKAMBI under the early access programs, which is considered to be a refund liability pursuant to ASC 606.

Upon adopting ASC 606 in the first quarter of 2018, the Company recorded an \$8.3 million cumulative effect adjustment to "Accumulated deficit" primarily related to shipments of ORKAMBI under early access programs in France. The Company determined the amount of the adjustment based upon (i) the status of pricing discussions in France upon adoption, (ii) the Company's estimate of the amount of consideration it expects to retain related to ORKAMBI sales in France that occurred on or prior to December 31, 2017 that will not be subject to a significant reversal in amounts recognized and (iii) recognition of costs previously deferred related to the ORKAMBI sales in France. For ORKAMBI sales in France that occurred after December 31, 2017 under the early access programs, the Company has recognized net product revenues based on the estimate of consideration it expects to retain that will not be subject to a significant reversal in amounts recognized.

In periods after the first quarter of 2018, if the Company's estimate regarding the amounts it will receive for ORKAMBI supplied pursuant to these early access programs changes, the Company will reflect the effect of the change in estimate in net product revenues in the period in which the change in estimate occurs and will include adjustments to all prior sales of ORKAMBI under the early access programs.

**Collaborative Revenues**

The Company recognizes collaborative revenues generated through collaborative research, development and/or commercialization agreements. The terms of these agreements typically include payment to the Company of one or more of the following: nonrefundable, upfront license fees; development and commercial milestone payments; funding of research and/or development activities; and royalties on net sales of licensed products. Each of these types of payments results in collaborative revenues except for revenues from royalties on net sales of licensed products, which are classified as royalty revenues. Revenue is recognized upon satisfaction of a performance obligation by transferring control of a good or service to the collaborator.

For each collaborative research, development and/or commercialization agreement that result in revenues, the Company identifies all material performance obligations, which may include a license to intellectual property and know-how, research and development activities and/or transition activities. In order to determine the transaction price,

in addition to any upfront payment, the Company estimates the amount of variable consideration at the outset of the contract either utilizing the

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expected value or most likely amount method, depending on the facts and circumstances relative to the contract. The Company constrains (reduces) the estimates of variable consideration such that it is probable that a significant reversal of previously recognized revenue will not occur throughout the life of the contract. When determining if variable consideration should be constrained, management considers whether there are factors outside the Company's control that could result in a significant reversal of revenue. In making these assessments, the Company considers the likelihood and magnitude of a potential reversal of revenue. These estimates are re-assessed each reporting period as required.

Once the estimated transaction price is established, amounts are allocated to the performance obligations that have been identified. The transaction price is generally allocated to each separate performance obligation on a relative standalone selling price basis. The Company must develop assumptions that require judgment to determine the standalone selling price in order to account for these agreements. To determine the standalone selling price the Company's assumptions may include (i) assumptions regarding the probability of obtaining marketing approval for the drug candidate, (ii) estimates regarding the timing of and the expected costs to develop and commercialize the drug candidate, (iii) estimates of future cash flows from potential product sales with respect to the drug candidate and (iv) appropriate discount and tax rates. Standalone selling prices used to perform the initial allocation are not updated after contract inception. The Company does not include a financing component to its estimated transaction price at contract inception unless it estimates that certain performance obligations will not be satisfied within one year.

**Upfront License Fees:** If a license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from nonrefundable, upfront license fees based on the relative value prescribed to the license compared to the total value of the arrangement. The revenue is recognized when the license is transferred to the collaborator and the collaborator is able to use and benefit from the license. For licenses that are not distinct from other obligations identified in the arrangement, the Company utilizes 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. If the combined performance obligation is satisfied over time, the Company applies an appropriate method of measuring progress for purposes of recognizing revenue from nonrefundable, upfront license fees. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition.

**Development and Regulatory Milestone Payments:** Depending on facts and circumstances, the Company may conclude that it is appropriate to include the milestone in the estimated transaction price or that it is appropriate to fully constrain the milestone. A milestone payment is included in the transaction price in the reporting period that the Company concludes that it is probable that recording revenue in the period will not result in a significant reversal in amounts recognized in future periods. The Company may record revenues from certain milestones in a reporting period before the milestone is achieved if the Company concludes that achievement of the milestone is probable and that recognition of revenue related to the milestone will not result in a significant reversal in amounts recognized in future periods. The Company records a corresponding contract asset when this conclusion is reached. Milestone payments that have not been included in the transaction price to date are fully constrained. These milestones remain fully constrained until the Company concludes that achievement of the milestone is probable and that recognition of revenue related to the milestone will not result in a significant reversal in amounts recognized in future periods. The Company re-evaluates the probability of achievement of such development milestones and any related constraint each reporting period. The Company adjusts its estimate of the overall transaction price, including the amount of collaborative revenue that it has recorded, if necessary.

**Research and Development Activities/Transition Services:** If the Company is entitled to reimbursement from its collaborators for specified research and development expenses, the Company accounts for them as separate performance obligations if it determines that these services represent a material right. The Company also determines whether the research and development funding would result in collaborative revenues or an offset to research and development expenses in accordance with provisions of gross or net revenue presentation. The corresponding

revenues or offset to research and development expenses are recognized as the related performance obligations are satisfied.

Sales-based Milestone and Royalty Payments: The Company's collaborators may be required to pay the Company sales-based milestone payments or royalties on future sales of commercial products. The Company recognizes revenues related to sales-based milestone and royalty payments upon the later to occur of (i) achievement of the collaborator's

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underlying sales or (ii) satisfaction of any performance obligation(s) related to these sales, in each case assuming the license to the Company's intellectual property is deemed to be the predominant item to which the sales-based milestones and/or royalties relate.

**Contract Liabilities**

The following table summarizes changes in the Company's contract liabilities for the three months ended March 31, 2018:

|  | Balance<br>at<br>January<br>1,<br>2018<br>(ASC<br>606<br>adoption)<br>(in thousands) | Balance<br>at<br>March<br>31,<br>2018 |
|--|--------------------------------------------------------------------------------------|---------------------------------------|
|  | Additions                                                                            | Deductions                            |

**Three Months Ended March 31, 2018****Contract liabilities:**

|                                    |         |          |    |           |
|------------------------------------|---------|----------|----|-----------|
| Other liabilities, current portion | \$1,654 | \$12,983 | \$ | —\$14,637 |
|------------------------------------|---------|----------|----|-----------|

The Company's contract liabilities relate to contracts with government-owned and supported customers in international markets limiting the amount of annual reimbursement the Company can receive. These contracts, which are classified as "Other liabilities, current portion," include upfront payments and fees. Upon exceeding the annual reimbursement amount, products are provided free of charge, which is a material right pursuant to ASC 606. The deferred portion is recognized as revenue when the free products are shipped. The Company's product revenue contracts include performance obligations that are one year or less.

During the three months ended March 31, 2018, the Company did not recognize any revenues related to its contract liability balance as of January 1, 2018 or revenues related to performance obligations satisfied in previous periods.

**Disaggregation of Revenue****Revenues by Product**

Product revenues, net consisted of the following:

|                             | Three Months<br>Ended March 31,<br>2018 (as<br>reported<br>under<br>ASC<br>606)<br>(in thousands) | 2017 (as<br>reported<br>under<br>ASC<br>605)<br>(in thousands) |
|-----------------------------|---------------------------------------------------------------------------------------------------|----------------------------------------------------------------|
| KALYDECO                    | \$249,539                                                                                         | \$185,715                                                      |
| ORKAMBI                     | 354,066                                                                                           | 294,861                                                        |
| SYMDEKO                     | 34,124                                                                                            | —                                                              |
| Other                       | —                                                                                                 | 46                                                             |
| Total product revenues, net | \$637,729                                                                                         | \$480,622                                                      |



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## Revenues by Geographic Location

Net product revenues are attributed to countries based on the location of the customer. Collaborative and royalty revenues are attributed to countries based on the location of the Company's subsidiary associated with the collaborative arrangement related to such revenues. Total revenues from external customers and collaborators by geographic region consisted of the following:

|                                             | Three Months<br>Ended March 31,<br>2018 (as reported<br>under ASC<br>606)<br>(in thousands) |  | 2017 (as<br>reported<br>under<br>ASC<br>605) |
|---------------------------------------------|---------------------------------------------------------------------------------------------|--|----------------------------------------------|
| United States                               | \$482,667                                                                                   |  | \$599,126                                    |
| Outside of the United States                |                                                                                             |  |                                              |
| Europe                                      | 131,895                                                                                     |  | 92,358                                       |
| Other                                       | 26,237                                                                                      |  | 23,234                                       |
| Total revenues outside of the United States | 158,132                                                                                     |  | 115,592                                      |
| Total revenues                              | \$640,799                                                                                   |  | \$714,718                                    |

In the three months ended March 31, 2018 and 2017, revenues attributable to Germany and the United Kingdom contributed the largest amounts to the Company's European revenues.

## C. Collaborative Arrangements and Acquisitions

## Cystic Fibrosis Foundation Therapeutics Incorporated

The Company has a research, development and commercialization agreement with Cystic Fibrosis Foundation Therapeutics Incorporated ("CFFT") that was originally entered into in May 2004, and was most recently amended in October 2016 (the "2016 Amendment"). Pursuant to the agreement, as amended, the Company has agreed to pay royalties ranging from low-single digits to mid-single digits on potential sales of certain compounds first synthesized and/or tested between March 1, 2014 and August 31, 2016, including VX-659 and VX-445, and tiered royalties ranging from single digits to sub-teens on any approved drugs first synthesized and/or tested during a research term on or before February 28, 2014, including KALYDECO (ivacaftor), ORKAMBI (lumacaftor in combination with ivacaftor) and SYMDEKO (tezacaftor in combination with ivacaftor). For combination products, such as ORKAMBI and SYMDEKO, sales are allocated equally to each of the active pharmaceutical ingredients in the combination product. The Company previously made certain commercial milestone payments to CFFT but there are no remaining commercial milestone payments payable by the Company to CFFT pursuant to the agreement.

Pursuant to the 2016 Amendment, the CFFT provided the Company an upfront payment of \$75.0 million and agreed to provide development funding to the Company of up to \$6.0 million annually. The upfront payment plus any future development funding represent a form of financing pursuant to ASC 730, Research and Development, and thus the amounts are recorded as a liability on the condensed consolidated balance sheet, primarily reflected in "Advance from collaborator, excluding current portion". The liability is reduced over the estimated royalty term of the agreement. Reductions in the liability are reflected as an offset to "Cost of sales" and as "Interest expense, net".

The Company began marketing KALYDECO in 2012 and began marketing ORKAMBI in 2015. The Company received approval for SYMDEKO in the United States in February 2018 and has submitted a Marketing Authorization Application ("MAA") to the European Medicines Agency ("EMA") seeking approval for tezacaftor in combination with ivacaftor in the European Union. The Company expects the EMA to complete its review of the MAA in the second half of 2018. The Company has royalty obligations to CFFT for ivacaftor, lumacaftor and tezacaftor until the expiration of patents covering those compounds. The Company has patents in the United States and European Union

covering the composition-of-matter of ivacaftor that expire in 2027 and 2025, respectively, subject to potential patent extensions. The Company has patents in the United States and European Union covering the composition-of-matter of lumacaftor that expire in 2030 and 2026, respectively, subject to potential extension. The Company has patents in the United States and European Union covering the composition-of-matter of tezacaftor that expire in 2027 and 2028, respectively, subject to potential extension.

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CRISPR Therapeutics AG

In 2015, the Company entered into a strategic collaboration, option and license agreement (the “CRISPR Agreement”) with CRISPR Therapeutics AG and its affiliates (“CRISPR”) to collaborate on the discovery and development of potential new treatments aimed at the underlying genetic causes of human diseases using CRISPR-Cas9 gene editing technology. The Company has the exclusive right to license up to six CRISPR-Cas9-based targets, including targets for the potential treatment of sickle cell disease. In connection with the CRISPR Agreement, the Company made an upfront payment to CRISPR of \$75.0 million and a \$30.0 million investment in CRISPR pursuant to a convertible loan agreement that subsequently converted into common shares of CRISPR and was recorded on the Company’s condensed consolidated balance sheet. The Company has made several additional investments in CRISPR’s common shares, including a \$21.5 million investment in January 2018. As of March 31, 2018, the Company recorded the fair value of its investment in CRISPR common shares of \$188.9 million in “Marketable securities” on its condensed consolidated balance sheet.

The Company funds all of the discovery activities conducted pursuant to the CRISPR Agreement. For targets that the Company elects to license, other than hemoglobinopathy treatments, the Company would lead all development and global commercialization activities. For each target that the Company elects to license, other than hemoglobinopathy targets, CRISPR has the potential to receive up to \$420.0 million in development, regulatory and commercial milestones and royalties on net product sales. As part of the collaboration, the Company and CRISPR share equally all development costs and potential worldwide revenues related to potential hemoglobinopathy treatments, including treatments for beta-thalassemia and sickle cell disease.

The Company may terminate the CRISPR Agreement upon 90 days’ notice to CRISPR prior to any product receiving marketing approval or upon 270 days’ notice after a product has received marketing approval. The CRISPR Agreement also may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, the CRISPR Agreement will continue in effect until the expiration of the Company’s payment obligations under the CRISPR Agreement.

In the fourth quarter of 2017, the Company entered into a co-development and co-commercialization agreement with CRISPR pursuant to the terms of the CRISPR Agreement, under which the Company and CRISPR will co-develop and co-commercialize CTX001 for the treatment of hemoglobinopathy treatments, including treatments for sickle cell disease and beta-thalassemia.

Merck KGaA

In January 2017, the Company entered into a strategic collaboration and license agreement (the “Merck KGaA Agreement”) with Merck KGaA, Darmstadt, Germany (“Merck KGaA”). Pursuant to the Merck KGaA Agreement, the Company granted Merck KGaA an exclusive worldwide license to research, develop and commercialize four oncology research and development programs. Under the Merck KGaA Agreement, the Company granted Merck KGaA exclusive, worldwide rights to two clinical-stage programs targeting DNA damage repair: its ataxia telangiectasia and Rad3-related protein inhibitor program, including VX-970 and VX-803, and its DNA-dependent protein kinase inhibitor program, including VX-984. In addition, the Company granted Merck KGaA exclusive, worldwide rights to two pre-clinical programs.

The Merck KGaA Agreement provided for an upfront payment from Merck KGaA to the Company of \$230.0 million. A portion of the upfront payment that was remitted to the German tax authorities in 2017 was refunded to the Company in February 2018. In addition to the upfront payment, the Company will receive tiered royalties on potential sales of licensed products, calculated as a percentage of net sales, that range from (i) mid-single digits to mid-twenties for clinical-stage programs and (ii) mid-single digits to high single digits for the pre-clinical research programs. Merck KGaA has assumed full responsibility for development and commercialization costs for all programs.

The Company evaluated the deliverables, primarily consisting of a license to the four programs and the obligation to complete certain fully-reimbursable research and development and transition activities as directed by Merck KGaA, pursuant to the Merck KGaA Agreement, under the multiple element arrangement accounting guidance that was

applicable in 2017. The Company concluded that the license had stand-alone value from the research and development and transition activities based on the resources and know-how possessed by Merck KGaA, and thus concluded that there are two units of accounting in the arrangement. The Company determined the relative selling price of the units of accounting based on the Company's

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best estimate of selling price. The Company utilized key assumptions to determine the best estimate of selling price for the license, which included future potential net sales of licensed products, development timelines, reimbursement rates for personnel costs, discount rates, and estimated third-party development costs. The Company utilized a discounted cash flow model to determine its best estimate of selling price for the license and determined the best estimate of selling price for the research and development and transition activities based on what it would sell the services for separately. Given the significance of the best estimate of selling price for the license as compared to the best estimate of selling price for the research and development and transition services, reasonable changes in the assumptions used in the discounted cash flow model would not have a significant impact on the relative selling price allocation. Based on this analysis, the Company recognized the \$230.0 million upfront payment upon delivery of the license as well as research and development and transition activities during the first quarter of 2017. The Company records the reimbursement for the research and development and transition activities in its condensed consolidated statements of operations as collaborative revenue primarily due to the fact that it is the primary obligor in the arrangement. As of December 31, 2017, the Company's activities related to research and development and transition activities under the Merck KGaA Agreement were substantially complete.

Merck KGaA may terminate the Merck KGaA Agreement or any individual program by providing 90 days' notice, or, in the case of termination of a program with a product that has received marketing approval, 180 days' notice. The Merck KGaA Agreement also may be terminated by either party for a material breach by the other party, subject to notice and cure provisions. Unless earlier terminated, the Merck KGaA Agreement will continue in effect until the date on which the royalty term and all payment obligations with respect to all products in all countries have expired.

Variable Interest Entities (VIEs)

The Company has entered into several agreements pursuant to which it has licensed rights to certain drug candidates from third-party collaborators, resulting in the consolidation of the third parties' financial statements into the Company's condensed consolidated financial statements as VIEs. In order to account for the fair value of the contingent payments, which could consist of milestone, royalty and option payments, related to these collaborations under GAAP, the Company uses present-value models based on assumptions regarding the probability of achieving the relevant milestones, estimates regarding the timing of achieving the milestones, estimates of future product sales and the appropriate discount rates. The Company bases its estimates of the probability of achieving the relevant milestones on industry data for similar assets and its own experience. The discount rates used in the valuation model represent a measure of credit risk and market risk associated with settling the liabilities. Significant judgment is used in determining the appropriateness of these assumptions at each reporting period. Changes in these assumptions could have a material effect on the fair value of the contingent payments. The following collaborations have been reflected in the Company's financial statements as consolidated VIEs for portions or all of the periods presented:

Parion Sciences, Inc.

In 2015, the Company entered into a strategic collaboration and license agreement (the "Parion Agreement") with Parion to collaborate with Parion to develop investigational epithelial sodium channel ("ENaC") inhibitors, including VX-371 (formerly P-1037) and VX-551 (formerly P-1055), for the potential treatment of CF and all other pulmonary diseases. The Company is responsible for all costs, subject to certain exceptions, related to development and commercialization of the compounds.

Pursuant to the Parion Agreement, the Company has worldwide development and commercial rights to Parion's lead investigational ENaC inhibitors, VX-371 and VX-551, for the potential treatment of CF and all other pulmonary diseases and has the option to select additional compounds discovered in Parion's research program. To date Parion received \$85.0 million in upfront and milestone payments under the Parion Agreement. Parion has the potential to receive up to an additional (i) \$485.0 million in development and regulatory milestone payments for development of ENaC inhibitors in CF, including \$360.0 million related to global filing and approval milestones, (ii) \$370.0 million in development and regulatory milestones for VX-371 and VX-551 in non-CF pulmonary indications and (iii) \$230.0 million in development and regulatory milestones should the Company elect to develop an additional ENaC inhibitor

from Parion's research program. The Company agreed to pay Parion tiered royalties that range from the low double digits to mid-teens as a percentage of potential sales of licensed products.

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The Company may terminate the Parion Agreement upon 90 days' notice to Parion prior to any licensed product receiving marketing approval or upon 180 days' notice after a licensed product has received marketing approval. If the Company experiences a change of control prior to the initiation of the first Phase 3 clinical trial for a licensed product, Parion may terminate the Parion Agreement upon 30 days' notice, subject to the Company's right to receive specified royalties on any subsequent commercialization of licensed products. The Parion Agreement also may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, the Parion Agreement will continue in effect until the expiration of the Company's royalty obligations, which expire on a country-by-country basis on the later of (i) the date the last-to-expire patent covering a licensed product expires or (ii) ten years after the first commercial sale in the country.

Following execution of the Parion Agreement, the Company determined that it had a variable interest in Parion via the Parion Agreement, and that the variable interest represented a variable interest in Parion as a whole because the fair value of the ENaC inhibitors represented more than half of the total fair value of Parion's assets. The Company also concluded that it was the primary beneficiary as it had the power to direct the activities that most significantly affect the economic performance of Parion and that it had the obligation to absorb losses and right to receive benefits that potentially could be significant to Parion. Accordingly, the Company consolidated Parion's financial statements beginning in June 2015. Notwithstanding the applicable accounting treatment, the Company's interests in Parion have been and continue to be limited to those accorded to the Company in the Parion Agreement.

As of September 30, 2017, the Company determined that the fair value of Parion's pulmonary ENaC platform had declined significantly based on data received in September 2017 from a Phase 2 clinical trial of VX-371 that did not meet its primary efficacy endpoint. After evaluating the results of the clinical trial and based on the decrease in the fair value of Parion's pulmonary ENaC platform relative to Parion's other activities, the Company determined that it was no longer the primary beneficiary of Parion as it no longer had the power to direct the significant activities of Parion. Accordingly, the Company deconsolidated Parion as of September 30, 2017. Please refer to Note B, "Collaborative Arrangements and Acquisitions," in the 2017 Annual Report on Form 10-K for further information regarding the deconsolidation of Parion.

**BioAxxone Biosciences, Inc.**

In 2014, the Company entered into a license and collaboration agreement (the "BioAxxone Agreement") with BioAxxone Biosciences, Inc. ("BioAxxone"), which resulted in the consolidation of BioAxxone as a VIE beginning on October 1, 2014. The Company recorded an in-process research and development intangible asset of \$29.0 million for VX-210 and a corresponding deferred tax liability of \$11.3 million attributable to BioAxxone. The Company made an initial payment to BioAxxone of \$10.0 million in 2014. In the first quarter of 2018, the Company's option to purchase BioAxxone expired and the Company paid a \$10.0 million license continuation fee to BioAxxone. BioAxxone has the potential to receive up to \$80.0 million in milestones, including development and regulatory milestone payments. As of March 31, 2018, the Company continues to conclude that it is the primary beneficiary of BioAxxone and continues to consolidate BioAxxone as a VIE.

**Aggregate VIE Financial Information**

An aggregate summary of net income attributable to noncontrolling interest related to the Company's VIEs for the three months ended March 31, 2018 and 2017 is as follows:

|                                                                                                                                 | Three Months<br>Ended March 31,<br>2018      2017<br>(in thousands) |          |
|---------------------------------------------------------------------------------------------------------------------------------|---------------------------------------------------------------------|----------|
| Loss attributable to noncontrolling interest before provision for income taxes and changes in fair value of contingent payments | \$557                                                               | \$1,547  |
| Provision for income taxes                                                                                                      | 6,405                                                               | 391      |
| Increase in fair value of contingent payments                                                                                   | (24,000 )                                                           | (3,730 ) |

Net income attributable to noncontrolling interest \$(17,038) \$(1,792)

The increase in the noncontrolling interest holders' claim to net assets with respect to the fair value of the contingent payments for the three months ended March 31, 2018 was primarily due to the expiration of the Company's option to

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purchase BioAxone that increased the probability related to the \$10.0 million license continuation fee for VX-210 and the probability that additional milestone and royalty payments related to the BioAxone Agreement will be achieved. The increase in the noncontrolling interest holders' claim to net assets with respect to the fair value of the contingent payments for the three months ended March 31, 2017 was primarily due to changes in market interest rates and the time value of money. The fair value of the contingent payments payable by the Company to BioAxone was \$32.9 million and \$18.9 million as of March 31, 2018 and December 31, 2017, respectively. During the three months ended March 31, 2018 and 2017, the increases in the fair value of the contingent payments related to the Company's VIEs were as follows:

|          | Three<br>Months<br>Ended<br>March 31,<br>2018 | 2017      |
|----------|-----------------------------------------------|-----------|
|          | (in<br>thousands)                             |           |
| Parion   | \$—                                           | \$(2,830) |
| BioAxone | (24,900)                                      | ( )       |

Significant amounts related to the Company's consolidation of BioAxone as a VIE included in the Company's condensed consolidated balance sheets as of the dates set forth in the table were as follows:

|                                            | March<br>31,<br>2018 | December<br>31, 2017 |
|--------------------------------------------|----------------------|----------------------|
|                                            | (in thousands)       |                      |
| Restricted cash and cash equivalents (VIE) | \$9,573              | \$ 1,489             |
| Intangible assets                          | 29,000               | 29,000               |
| Deferred tax liability                     | 9,636                | 4,756                |
| Noncontrolling interest                    | 29,765               | 13,727               |

The Company has recorded BioAxone's cash and cash equivalents as "Restricted cash and cash equivalents (VIE)" because (i) the Company does not have any interest in or control over BioAxone's cash and cash equivalents and (ii) the Company's agreement with BioAxone does not provide for BioAxone's cash and cash equivalents to be used for the development of the asset that the Company licensed from BioAxone. Assets recorded as a result of consolidating BioAxone's financial condition into the Company's balance sheets do not represent additional assets that could be used to satisfy claims against the Company's general assets.

**Other Collaborations**

The Company has entered into various agreements pursuant to which it collaborates with third parties, including inlicensing and outlicensing arrangements. Although the Company does not consider any of these arrangements to be material, the most notable of these arrangements are described below.

**Moderna Therapeutics, Inc.**

In 2016, the Company entered into a strategic collaboration and licensing agreement (the "Moderna Agreement") with Moderna Therapeutics, Inc. ("Moderna") pursuant to which the parties are seeking to identify and develop messenger Ribonucleic Acid ("mRNA") therapeutics for the treatment of CF. In connection with the Moderna Agreement, the Company made an upfront payment to Moderna of \$20.0 million and a \$20.0 million investment in Moderna pursuant to a convertible promissory note that converted into preferred stock in 2016. Moderna has the potential to receive future development and regulatory milestones of up to \$275.0 million, including \$220.0 million in approval and reimbursement milestones, as well as tiered royalty payments on future sales. The carrying value of our

investment in Moderna was \$23.0 million as of March 31, 2018.

Under the terms of the Moderna Agreement, Moderna leads discovery efforts and the Company leads all preclinical, development and commercialization activities associated with the advancement of mRNA Therapeutics that result from this collaboration and will fund all expenses related to the collaboration.

The Company may terminate the Moderna Agreement by providing advance notice to Moderna, with the required length of notice dependent on whether any product developed under the Moderna Agreement has received marketing approval. The

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Moderna Agreement also may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, the Moderna Agreement will continue in effect until the expiration of the Company's payment obligations under the Moderna Agreement.

Janssen Pharmaceuticals, Inc.

In 2014, the Company entered into an agreement (the "Janssen Agreement") with Janssen Pharmaceuticals, Inc. ("Janssen"). Pursuant to the agreement, Janssen has an exclusive worldwide license to develop and commercialize certain drug candidates for the treatment of influenza, including pimodivir (formerly VX-787). The Company received non-refundable payments of \$35.0 million from Janssen in 2014 and recognized a \$25.0 million milestone in the fourth quarter of 2017. The milestone, which was achieved based on the Phase 3 clinical trial Janssen initiated in the fourth quarter of 2017, was collected in the first quarter of 2018. The Company has the potential to receive additional regulatory and commercial milestone payments as well as royalties on future product sales, if any. Janssen is responsible for costs related to the development and commercialization of the compounds. Janssen may terminate the Janssen Agreement, subject to certain exceptions, upon 6 months' notice.

Asset Acquisition

Concert Pharmaceuticals

In July 2017, the Company acquired certain CF assets including VX-561 (formerly CTP-656) (the "Concert Assets") from Concert Pharmaceuticals Inc. ("Concert") pursuant to an asset purchase agreement that was entered into in March 2017 (the "Concert Agreement"). VX-561 is an investigational CFTR potentiator that has the potential to be used as part of combination regimens of CFTR modulators to treat CF. Pursuant to the Concert Agreement, Vertex paid Concert \$160.0 million in cash for the Concert Assets. If VX-561 is approved as part of a combination regimen to treat CF, Concert could receive up to an additional \$90.0 million in milestones based on regulatory approval in the United States and reimbursement in the United Kingdom, Germany or France. The Company determined that substantially all of the fair value of the Concert Agreement was attributable to a single in-process research and development asset, VX-561, which did not constitute a business. The Company concluded that it did not have any alternative future use for the acquired in-process research and development asset. Thus, the Company recorded the \$160.0 million upfront payment as a research and development expense in the third quarter of 2017. The total cost of the transaction was \$165.1 million including \$5.1 million of transaction costs that were recorded as sales, general and administrative expenses. If the Company achieves regulatory approval and reimbursement milestones, the Company will record the value of the milestone as an intangible asset and will begin amortizing the asset in cost of sales in the period that the relevant milestone is achieved.

D. Earnings Per Share

Basic net income per share attributable to Vertex common shareholders is based upon the weighted-average number of common shares outstanding during the period, excluding restricted stock, restricted stock units and performance-based restricted stock units, or "PSUs," that have been issued but are not yet vested. Diluted net income per share attributable to Vertex common shareholders is based upon the weighted-average number of common shares outstanding during the period plus additional weighted-average common equivalent shares outstanding during the period when the effect is dilutive.

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The following table sets forth the computation of basic and diluted net income per share for the periods ended:

|                                                                       | Three Months Ended<br>March 31,             |           |
|-----------------------------------------------------------------------|---------------------------------------------|-----------|
|                                                                       | 2018                                        | 2017      |
|                                                                       | (in thousands, except<br>per share amounts) |           |
| Basic net income attributable to Vertex per common share calculation: |                                             |           |
| Net income attributable to Vertex common shareholders                 | \$210,263                                   | \$247,756 |
| Less: Undistributed earnings allocated to participating securities    | (99 )                                       | (406 )    |
| Net income attributable to Vertex common shareholders—basic           | \$210,164                                   | \$247,350 |
| Basic weighted-average common shares outstanding                      | 253,231                                     | 246,024   |
| Basic net income attributable to Vertex per common share              | \$0.83                                      | \$1.01    |

Diluted net income attributable to Vertex per common share calculation:

|                                                                    |           |           |
|--------------------------------------------------------------------|-----------|-----------|
| Net income attributable to Vertex common shareholders              | \$210,263 | \$247,756 |
| Less: Undistributed earnings allocated to participating securities | (97 )     | (401 )    |
| Net income attributable to Vertex common shareholders—diluted      | \$210,166 | \$247,355 |

|                                                                             |         |         |
|-----------------------------------------------------------------------------|---------|---------|
| Weighted-average shares used to compute basic net income per common share   | 253,231 | 246,024 |
| Effect of potentially dilutive securities:                                  |         |         |
| Stock options                                                               | 3,248   | 2,037   |
| Restricted stock and restricted stock units (including PSUs)                | 2,013   | 627     |
| Employee stock purchase program                                             | 34      | 12      |
| Weighted-average shares used to compute diluted net income per common share | 258,526 | 248,700 |
| Diluted net income attributable to Vertex per common share                  | \$0.81  | \$0.99  |

The Company did not include the securities in the following table in the computation of the net income per share attributable to Vertex common shareholders calculations because the effect would have been anti-dilutive during each period:

|                                                                       | Three<br>Months<br>Ended<br>March 31, |       |
|-----------------------------------------------------------------------|---------------------------------------|-------|
|                                                                       | 2018                                  | 2017  |
|                                                                       | (in<br>thousands)                     |       |
| Stock options                                                         | 1,633                                 | 8,303 |
| Unvested restricted stock and restricted stock units (including PSUs) | 4                                     | 807   |

**E. Fair Value Measurements**

The fair value of the Company's financial assets and liabilities reflects the Company's estimate of amounts that it would have received in connection with the sale of the assets or paid in connection with the transfer of the liabilities in an orderly transaction between market participants at the measurement date. In connection with measuring the fair value of its assets and liabilities, the Company seeks to maximize the use of observable inputs (market data obtained from sources independent from the Company) and to minimize the use of unobservable inputs (the Company's assumptions about how market participants would price assets and liabilities). The following fair value hierarchy is used to classify assets and liabilities based on the observable inputs and unobservable inputs used in order to value the assets and

liabilities:

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Quoted prices in active markets for identical assets or liabilities. An active market for an asset or liability is a Level 1: market in which transactions for the asset or liability occur with sufficient frequency and volume to provide pricing information on an ongoing basis.

Observable inputs other than Level 1 inputs. Examples of Level 2 inputs include quoted prices in active Level 2: markets for similar assets or liabilities and quoted prices for identical assets or liabilities in markets that are not active.

Unobservable inputs based on the Company's assessment of the assumptions that market participants would Level 3: use in pricing the asset or liability.

The Company's investment strategy is focused on capital preservation. The Company invests in instruments that meet the credit quality standards outlined in the Company's investment policy. This policy also limits the amount of credit exposure to any one issue or type of instrument. As of March 31, 2018, the Company's investments were primarily in money market funds, government-sponsored enterprise securities, corporate equity securities, corporate debt securities and commercial paper. Additionally, the Company utilizes foreign currency forward contracts intended to mitigate the effect of changes in foreign exchange rates on its condensed consolidated statement of operations.

As of March 31, 2018, all of the Company's financial assets and liabilities that were subject to fair value measurements were valued using observable inputs. The Company's financial assets valued based on Level 1 inputs consisted of money market funds, government-sponsored enterprise securities and corporate equity securities. The Company's financial assets and liabilities valued based on Level 2 inputs consisted of corporate debt securities and commercial paper, which consisted of investments in highly-rated investment-grade corporations, and foreign currency forward contracts with reputable and creditworthy counterparties. During the three months ended March 31, 2018 and 2017, the Company did not record any other-than-temporary impairment charges related to its financial assets.

The following table sets forth the Company's financial assets and liabilities (excluding VIE cash and cash equivalents) subject to fair value measurements:

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## Fair Value Measurements as of March 31, 2018

| Total          | Fair Value Hierarchy |         |         |
|----------------|----------------------|---------|---------|
|                | Level 1              | Level 2 | Level 3 |
| (in thousands) |                      |         |         |

## Financial instruments carried at fair value (asset position):

## Cash equivalents:

## Money market funds

|           |           |     |     |
|-----------|-----------|-----|-----|
| \$921,007 | \$921,007 | \$— | \$— |
|-----------|-----------|-----|-----|

## Government-sponsored enterprise securities

|       |       |   |   |
|-------|-------|---|---|
| 4,989 | 4,989 | — | — |
|-------|-------|---|---|

## Corporate debt securities

|       |   |       |   |
|-------|---|-------|---|
| 7,927 | — | 7,927 | — |
|-------|---|-------|---|

## Commercial paper

|        |   |        |   |
|--------|---|--------|---|
| 50,669 | — | 50,669 | — |
|--------|---|--------|---|

## Marketable securities:

## Corporate equity securities

|         |         |   |   |
|---------|---------|---|---|
| 188,857 | 188,857 | — | — |
|---------|---------|---|---|

## Government-sponsored enterprise securities

|       |       |   |   |
|-------|-------|---|---|
| 4,995 | 4,995 | — | — |
|-------|-------|---|---|

## Corporate debt securities

|         |   |         |   |
|---------|---|---------|---|
| 218,213 | — | 218,213 | — |
|---------|---|---------|---|

## Commercial paper

|        |   |        |   |
|--------|---|--------|---|
| 69,059 | — | 69,059 | — |
|--------|---|--------|---|

## Prepaid and other current assets:

## Foreign currency forward contracts

|     |   |     |   |
|-----|---|-----|---|
| 540 | — | 540 | — |
|-----|---|-----|---|

## Other assets:

## Foreign currency forward contracts

|    |   |    |   |
|----|---|----|---|
| 31 | — | 31 | — |
|----|---|----|---|

## Total financial assets

|             |             |           |     |
|-------------|-------------|-----------|-----|
| \$1,466,287 | \$1,119,848 | \$346,439 | \$— |
|-------------|-------------|-----------|-----|

## Financial instruments carried at fair value (liability position):

## Other liabilities, current portion:

## Foreign currency forward contracts

|             |     |             |     |
|-------------|-----|-------------|-----|
| \$(15,370 ) | \$— | \$(15,370 ) | \$— |
|-------------|-----|-------------|-----|

## Other liabilities, excluding current portion:

## Foreign currency forward contracts

|        |   |        |   |
|--------|---|--------|---|
| (850 ) | — | (850 ) | — |
|--------|---|--------|---|

## Total financial liabilities

|             |     |             |     |
|-------------|-----|-------------|-----|
| \$(16,220 ) | \$— | \$(16,220 ) | \$— |
|-------------|-----|-------------|-----|

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|                                                                   | Fair Value Measurements as of<br>December 31, 2017 |           |             |         |
|-------------------------------------------------------------------|----------------------------------------------------|-----------|-------------|---------|
|                                                                   | Fair Value Hierarchy                               |           |             |         |
|                                                                   | Total                                              | Level 1   | Level 2     | Level 3 |
| (in thousands)                                                    |                                                    |           |             |         |
| Financial instruments carried at fair value (asset position):     |                                                    |           |             |         |
| Cash equivalents:                                                 |                                                    |           |             |         |
| Money market funds                                                | \$614,951                                          | \$614,951 | \$—         | \$ —    |
| Government-sponsored enterprise securities                        | 12,678                                             | 12,678    | —           | —       |
| Commercial paper                                                  | 57,357                                             | —         | 57,357      | —       |
| Marketable securities:                                            |                                                    |           |             |         |
| Corporate equity securities                                       | 74,821                                             | 74,821    | —           | —       |
| Government-sponsored enterprise securities                        | 2,303                                              | 2,303     | —           | —       |
| Corporate debt securities                                         | 265,867                                            | —         | 265,867     | —       |
| Commercial paper                                                  | 80,263                                             | —         | 80,263      | —       |
| Prepaid and other current assets:                                 |                                                    |           |             |         |
| Foreign currency forward contracts                                | 13                                                 | —         | 13          | —       |
| Total financial assets                                            | \$1,108,253                                        | \$704,753 | \$403,500   | \$ —    |
| Financial instruments carried at fair value (liability position): |                                                    |           |             |         |
| Other liabilities, current portion:                               |                                                    |           |             |         |
| Foreign currency forward contracts                                | \$(13,642 )                                        | \$—       | \$(13,642 ) | \$ —    |
| Other liabilities, excluding current portion:                     |                                                    |           |             |         |
| Foreign currency forward contracts                                | (866 )                                             | —         | (866 )      | —       |
| Total financial liabilities                                       | \$(14,508 )                                        | \$—       | \$(14,508 ) | \$ —    |

Please refer to Note F, "Marketable Securities and Equity Investments," for the carrying amount and related unrealized gains (losses) by type of the Company's financial assets and liabilities.

The Company's VIE invested in cash equivalents consisting of money market funds of \$8.8 million as of March 31, 2018, which are valued based on Level 1 inputs. These cash equivalents are not included in the table above. The Company's noncontrolling interest related to the Company's VIE includes the fair value of the contingent payments, which could consist of milestone, royalty and option payments, which are valued based on Level 3 inputs. Please refer to Note C, "Collaborative Arrangements and Acquisitions," for further information.

**F. Marketable Securities and Equity Investments**

Pursuant to the adoption of ASU 2016-01 on January 1, 2018, the Company began recording changes in the fair value of its investments in corporate equity securities to "Other income (expense), net" in the Company's condensed consolidated statements of operations. Prior to its adoption of ASU 2016-01, the Company recorded changes in the fair value of its investments in corporate equity securities to "Accumulated other comprehensive loss" on its condensed consolidated balance sheet until the related gains or losses were realized. The Company continues to record unrealized gains (losses) on available-for-sale debt securities as a component of accumulated other comprehensive income (loss) until such gains and losses are realized.

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A summary of the Company's cash equivalents and marketable securities is shown below:

|                                                                    | Amortized<br>Cost | Gross<br>Unrealized<br>Gains | Gross<br>Unrealized<br>Losses | Fair Value    |
|--------------------------------------------------------------------|-------------------|------------------------------|-------------------------------|---------------|
|                                                                    | (in thousands)    |                              |                               |               |
| As of March 31, 2018                                               |                   |                              |                               |               |
| Available-for-sale debt securities                                 |                   |                              |                               |               |
| Cash equivalents:                                                  |                   |                              |                               |               |
| Money market funds                                                 | \$921,007         | \$ —                         | \$ —                          | \$921,007     |
| Government-sponsored enterprise securities                         | 4,990             | —                            | (1                            | ) 4,989       |
| Corporate debt securities                                          | 7,930             | —                            | (3                            | ) 7,927       |
| Commercial paper                                                   | 50,680            | —                            | (11                           | ) 50,669      |
| Total cash equivalents                                             | 984,607           | —                            | (15                           | ) 984,592     |
| Marketable securities:                                             |                   |                              |                               |               |
| Government-sponsored enterprise securities (matures within 1 year) | 4,995             | —                            | —                             | 4,995         |
| Corporate debt securities (matures within 1 year)                  | 184,698           | —                            | (648                          | ) 184,050     |
| Corporate debt securities (matures after 1 year through 5 years)   | 34,383            | —                            | (220                          | ) 34,163      |
| Commercial paper (matures within 1 year)                           | 69,230            | —                            | (171                          | ) 69,059      |
| Total marketable debt securities                                   | 293,306           | —                            | (1,039                        | ) 292,267     |
| Total available-for-sale debt securities                           | 1,277,913         | —                            | (1,054                        | ) 1,276,859   |
| Corporate equity securities                                        | 64,713            | 124,144                      | —                             | 188,857       |
| Total cash equivalents and marketable securities                   | \$1,342,626       | \$ 124,144                   | \$ (1,054                     | ) \$1,465,716 |

As of December 31, 2017

Available-for-sale debt securities

Cash equivalents:

|                                            |           |      |      |           |
|--------------------------------------------|-----------|------|------|-----------|
| Money market funds                         | \$614,951 | \$ — | \$ — | \$614,951 |
| Government-sponsored enterprise securities | 12,679    | —    | (4   | ) 12,678  |
| Commercial paper                           | 57,371    | —    | (14  | ) 57,357  |
| Total cash equivalents                     | 685,001   | —    | (15  | ) 684,986 |

Marketable securities:

|                                                                    |             |           |         |               |
|--------------------------------------------------------------------|-------------|-----------|---------|---------------|
| Government-sponsored enterprise securities (matures within 1 year) | 2,304       | —         | (1      | ) 2,303       |
| Corporate debt securities (matures within 1 year)                  | 215,639     | —         | (363    | ) 215,276     |
| Corporate debt securities (matures after 1 year through 5 years)   | 50,697      | —         | (106    | ) 50,591      |
| Commercial paper (matures within 1 year)                           | 80,372      | —         | (109    | ) 80,263      |
| Total marketable debt securities                                   | 349,012     | —         | (579    | ) 348,433     |
| Total available-for-sale debt securities                           | 1,034,013   | —         | (594    | ) 1,033,419   |
| Available-for-sale corporate equity securities                     | 43,213      | 31,608    | —       | 74,821        |
| Total cash equivalents and marketable securities                   | \$1,077,226 | \$ 31,608 | \$ (594 | ) \$1,108,240 |

The Company has a limited number of available-for-sale debt securities in insignificant loss positions as of March 31, 2018, which the Company does not intend to sell and has concluded it will not be required to sell before recovery of the amortized costs for the investments at maturity. There were no charges recorded for other-than-temporary declines in the fair value of available-for-sale debt securities nor gross realized gains or losses recognized in the three months ended March 31, 2018 and 2017, respectively.

The Company maintains strategic investments separately from the investment policy that governs its other cash, cash equivalents and marketable securities. During the three months ended March 31, 2018, the Company recorded an

aggregate unrealized gain of \$95.5 million related to its investments in corporate equity securities, as follows:

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\$92.5 million related to its equity investment in CRISPR, a publicly traded company. The CRISPR common stock held by the Company has a readily determinable fair value that is recorded in “Marketable securities” on the Company’s condensed consolidated balance sheets. In January 2018, the Company purchased an additional \$21.5 million of CRISPR’s common shares.

\$2.9 million related to its equity investment in Moderna, which is not a publicly traded company that has a readily determinable fair value for its stock. The Company increased the carrying value of its investment in Moderna, which is recorded in “Other assets” on its condensed consolidated balance sheets, to \$23.0 million as of March 31, 2018 based on an observable price increase for additional shares privately issued by Moderna in an orderly transaction between market participants.

G. Accumulated Other Comprehensive  
Income (Loss)

The following table summarizes the changes in accumulated other comprehensive income (loss) by component:

|                                                                                             | Unrealized Holding Gains<br>(Losses), Net of Tax |                                        |                                        |                                                   |            |
|---------------------------------------------------------------------------------------------|--------------------------------------------------|----------------------------------------|----------------------------------------|---------------------------------------------------|------------|
|                                                                                             | Foreign<br>Currency<br>Translation<br>Adjustment | On<br>Available-<br>Debt<br>Securities | On<br>For-Sale<br>Equity<br>Securities | On<br>Foreign<br>Currency<br>Forward<br>Contracts | Total      |
|                                                                                             | (in thousands)                                   |                                        |                                        |                                                   |            |
| Balance at December 31, 2017                                                                | \$(21,031)                                       | \$(594 )                               | 25,069                                 | \$(15,016)                                        | \$(11,572) |
| Other comprehensive loss before reclassifications                                           | (2,729 )                                         | (460 )                                 | —                                      | (7,639 )                                          | (10,828 )  |
| Amounts reclassified from accumulated other comprehensive income (loss)                     | —                                                | —                                      | —                                      | 6,777                                             | 6,777      |
| Net current period other comprehensive (loss) income                                        | \$(2,729 )                                       | \$(460 )                               | \$ —                                   | \$(862 )                                          | \$(4,051 ) |
| Amounts reclassified to accumulated deficit pursuant to adoption of new accounting standard | 949                                              | —                                      | (25,069)                               | —                                                 | (24,120 )  |
| Balance at March 31, 2018                                                                   | \$(22,811)                                       | \$(1,054)                              | \$ —                                   | \$(15,878)                                        | \$(39,743) |

|                                                                         | Unrealized Holding Gains<br>(Losses), Net of Tax |                                        |                                        |                                                   |            |
|-------------------------------------------------------------------------|--------------------------------------------------|----------------------------------------|----------------------------------------|---------------------------------------------------|------------|
|                                                                         | Foreign<br>Currency<br>Translation<br>Adjustment | On<br>Available-<br>Debt<br>Securities | On<br>For-Sale<br>Equity<br>Securities | On<br>Foreign<br>Currency<br>Forward<br>Contracts | Total      |
|                                                                         | (in thousands)                                   |                                        |                                        |                                                   |            |
| Balance at December 31, 2016                                            | \$(7,862)                                        | \$(10 )                                | 17,531                                 | \$ 11,514                                         | \$ 21,173  |
| Other comprehensive (loss) income before reclassifications              | (2,001 )                                         | (251 )                                 | 3,785                                  | (2,802 )                                          | (1,269 )   |
| Amounts reclassified from accumulated other comprehensive income (loss) | —                                                | —                                      | —                                      | (3,879 )                                          | (3,879 )   |
| Net current period other comprehensive (loss) income                    | \$(2,001)                                        | \$(251)                                | \$ 3,785                               | \$(6,681 )                                        | \$(5,148 ) |
| Balance at March 31, 2017                                               | \$(9,863)                                        | \$(261)                                | \$ 21,316                              | \$ 4,833                                          | \$ 16,025  |

## H. Hedging

The Company maintains a hedging program intended to mitigate the effect of changes in foreign exchange rates for a portion of the Company’s forecasted product revenues denominated in certain foreign currencies. The program includes



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foreign currency forward contracts that are designated as cash flow hedges under GAAP having contractual durations from one to eighteen months.

The Company formally documents the relationship between foreign currency forward contracts (hedging instruments) and forecasted product revenues (hedged items), as well as the Company's risk management objective and strategy for undertaking various hedging activities, which includes matching all foreign currency forward contracts that are designated as cash flow hedges to forecasted transactions. The Company also formally assesses, both at the hedge's inception and on an ongoing basis, whether the foreign currency forward contracts are highly effective in offsetting changes in cash flows of hedged items on a prospective and retrospective basis. If the Company determines that a (i) foreign currency forward contract is not highly effective as a cash flow hedge, (ii) foreign currency forward contract has ceased to be a highly effective hedge or (iii) forecasted transaction is no longer probable of occurring, the Company would discontinue hedge accounting treatment prospectively. The Company measures effectiveness based on the change in fair value of the forward contracts and the fair value of the hypothetical foreign currency forward contracts with terms that match the critical terms of the risk being hedged. As of March 31, 2018, all hedges were determined to be highly effective, and the Company had not recorded any ineffectiveness related to the hedging program.

The following table summarizes the notional amount of the Company's outstanding foreign currency forward contracts designated as cash flow hedges under GAAP:

|                                          | As of<br>March<br>31, 2018 | As of<br>December<br>31, 2017 |
|------------------------------------------|----------------------------|-------------------------------|
| Foreign Currency                         | (in thousands)             |                               |
| Euro                                     | \$326,024                  | \$257,230                     |
| British pound sterling                   | 78,066                     | 77,481                        |
| Australian dollar                        | 33,134                     | 30,501                        |
| Canadian dollar                          | 21,104                     | —                             |
| Total foreign currency forward contracts | \$458,328                  | \$365,212                     |

The following table summarizes the fair value of the Company's outstanding foreign currency forward contracts designated as cash flow hedges under GAAP included on the Company's condensed consolidated balance sheets:

As of March 31, 2018

| Assets                           |            | Liabilities                                  |            |
|----------------------------------|------------|----------------------------------------------|------------|
| Classification                   | Fair Value | Classification                               | Fair Value |
| (in thousands)                   |            |                                              |            |
| Prepaid and other current assets | \$ 540     | Other liabilities, current portion           | \$(15,370) |
| Other assets                     | 31         | Other liabilities, excluding current portion | (850 )     |
| Total assets                     | \$ 571     | Total liabilities                            | \$(16,220) |

As of December 31, 2017

| Assets                                    |            | Liabilities                                  |            |
|-------------------------------------------|------------|----------------------------------------------|------------|
| Classification                            | Fair Value | Classification                               | Fair Value |
| (in thousands)                            |            |                                              |            |
| Prepaid expenses and other current assets | \$ 13      | Other liabilities, current portion           | \$(13,642) |
| Other assets                              | —          | Other liabilities, excluding current portion | (866 )     |
| Total assets                              | \$ 13      | Total liabilities                            | \$(14,508) |

As of March 31, 2018, the Company expects amounts recorded in "Prepaid expenses and other current assets" and "Other liabilities, current portion" to be reclassified to earnings within twelve months.

The following table summarizes the potential effect of offsetting derivatives by type of financial instrument designated as cash flow hedges under GAAP on the Company's condensed consolidated balance sheets:

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|                                                   | As of March 31, 2018           |                            |                               |                                   |                 |
|---------------------------------------------------|--------------------------------|----------------------------|-------------------------------|-----------------------------------|-----------------|
|                                                   | Gross<br>Amounts<br>Recognized | Gross<br>Amounts<br>Offset | Gross<br>Amounts<br>Presented | Gross<br>Amounts<br>Not<br>Offset | Legal<br>Offset |
| Foreign currency forward contracts (in thousands) |                                |                            |                               |                                   |                 |
| Total assets                                      | \$571                          | \$                         | —\$571                        | \$ (571 )                         | \$—             |
| Total liabilities                                 | \$(16,220)                     | \$                         | —\$(16,220)                   | \$ 571                            | \$(15,649)      |

|                                                   | As of December 31, 2017        |                            |                               |                                   |                 |
|---------------------------------------------------|--------------------------------|----------------------------|-------------------------------|-----------------------------------|-----------------|
|                                                   | Gross<br>Amounts<br>Recognized | Gross<br>Amounts<br>Offset | Gross<br>Amounts<br>Presented | Gross<br>Amounts<br>Not<br>Offset | Legal<br>Offset |
| Foreign currency forward contracts (in thousands) |                                |                            |                               |                                   |                 |
| Total assets                                      | \$13                           | \$                         | —\$13                         | \$ (13 )                          | \$—             |
| Total liabilities                                 | \$(14,508)                     | \$                         | —\$(14,508)                   | \$ 13                             | \$(14,495)      |

The Company also enters into foreign exchange forward contracts with contractual maturities of less than one month designed to mitigate the effect of changes in foreign exchange rates on monetary assets and liabilities including intercompany balances. The Company recognized losses of \$1.5 million and \$3.5 million recorded in “Other income (expense), net”, for the three months ended March 31, 2018 and 2017, respectively, related to foreign exchange contracts, which are not designated as hedging instruments under GAAP.

As of March 31, 2018, the notional amount of foreign exchange contracts where hedge accounting under GAAP is not applied was \$88.9 million. The following table summarizes the fair value of the Company’s outstanding foreign currency forward contracts not designated for hedge accounting included on the Company’s condensed consolidated balance sheets:

|                                           | As<br>of<br>March<br>31,<br>2018 | As of<br>December<br>31, 2017 |
|-------------------------------------------|----------------------------------|-------------------------------|
|                                           | (in thousands)                   |                               |
| Prepaid expenses and other current assets | \$115                            | \$ —                          |
| Other liabilities, current portion        | \$—                              | \$ (684 )                     |

## I. Inventories

Inventories consisted of the following:

|                 | As of<br>March<br>31, 2018 | As of<br>December<br>31, 2017 |
|-----------------|----------------------------|-------------------------------|
|                 | (in thousands)             |                               |
| Raw materials   | \$17,587                   | \$20,924                      |
| Work-in-process | 78,232                     | 74,237                        |
| Finished goods  | 21,527                     | 16,669                        |
| Total           | \$117,346                  | \$111,830                     |

## J. Intangible Assets and Goodwill

## Intangible Assets

As of March 31, 2018 and December 31, 2017, the Company had \$29.0 million of in-process research and development intangible assets recorded on the Company's condensed consolidated balance sheet related to VX-210, that is licensed by BioAxone to the Company.

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Goodwill

As of March 31, 2018 and December 31, 2017, goodwill of \$50.4 million was recorded on the Company's condensed consolidated balance sheet.

K. Long-term Obligations

Fan Pier Leases

In 2011, the Company entered into two lease agreements, pursuant to which the Company leases approximately 1.1 million square feet of office and laboratory space in two buildings (the "Fan Pier Buildings") at Fan Pier in Boston, Massachusetts (the "Fan Pier Leases"). The Company commenced lease payments in December 2013, and will make lease payments pursuant to the Fan Pier Leases through December 2028. The Company has an option to extend the term of the Fan Pier Leases for an additional ten years.

Because the Company was involved in the construction project, the Company was deemed for accounting purposes to be the owner of the Fan Pier Buildings during the construction period and recorded project construction costs incurred by the landlord. Upon completion of the Fan Pier Buildings, the Company evaluated the Fan Pier Leases and determined that the Fan Pier Leases did not meet the criteria for "sale-leaseback" treatment. Accordingly, the Company began depreciating the asset and incurring interest expense related to the financing obligation in 2013. The Company bifurcates its lease payments pursuant to the Fan Pier Leases into (i) a portion that is allocated to the Buildings and (ii) a portion that is allocated to the land on which the Fan Pier Buildings were constructed. The portion of the lease obligations allocated to the land is treated as an operating lease that commenced in 2011.

Property and equipment, net, included \$472.4 million and \$475.7 million as of March 31, 2018 and December 31, 2017, respectively, related to construction costs for the Fan Pier Buildings. The carrying value of the construction financing lease obligation related to the Fan Pier Buildings, which excludes interest that will be imputed over the course of the Company's lease agreement for the Buildings was \$471.9 million and \$472.1 million as of March 31, 2018 and December 31, 2017, respectively.

San Diego Lease

On December 2, 2015, the Company entered into a lease agreement for 3215 Merryfield Row, San Diego, California with ARE-SD Region No. 23, LLC (the "San Diego Building"). Pursuant to this agreement, the Company agreed to lease approximately 170,000 square feet of office and laboratory space in a building under construction in San Diego, California ("San Diego Lease") for a term of 16 years. The Company expects base rent payments will commence in the second quarter of 2019. Pursuant to the San Diego Lease, during the initial 16-year term, the Company will pay an average of approximately \$10.2 million per year in aggregate rent, exclusive of operating expenses. The Company has the option to extend the lease term for up to two additional five-year terms.

Because the Company is involved in the construction project, the Company is deemed for accounting purposes to be the owner of the San Diego Building during the construction period and recorded project construction costs incurred by the landlord. The Company bifurcates its lease payments pursuant to the San Diego Lease into (i) a portion that is allocated to the San Diego Building and (ii) a portion that is allocated to the land on which the San Diego Building was constructed. Although the Company will not begin making lease payments pursuant to the San Diego Lease until the commencement date, the portion of the lease obligation allocated to the land is treated for accounting purposes as an operating lease that commenced in the fourth quarter of 2016. Upon completion of the San Diego Building, the Company will evaluate the San Diego Lease and determine if the San Diego Lease meets the criteria for "sale-leaseback" treatment. If the San Diego Lease meets the "sale-leaseback" criteria, the Company will remove the asset and the related liability from its condensed consolidated balance sheet and treat the San Diego Lease as either an operating or a capital lease based on the Company's assessment of the accounting guidance. The Company expects that upon completion of construction of the San Diego Building the San Diego Lease will not meet the "sale-leaseback" criteria. If the San Diego Lease does not meet "sale-leaseback" criteria, the Company will treat the San Diego Lease as a financing obligation and will depreciate the asset over its estimated useful life.



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Property and equipment, net, included \$105.7 million and \$94.6 million as of March 31, 2018 and December 31, 2017, respectively, related to construction costs for the San Diego building. The carrying value of the construction financing lease obligation for the San Diego building was \$91.1 million and \$87.4 million as of March 31, 2018 and December 31, 2017, respectively.

Revolving Credit Facility

In October 2016, the Company entered into a Credit Agreement (the "Credit Agreement") with Bank of America, N.A., as administrative agent and the lenders referred to therein. The Credit Agreement provides for a \$500.0 million revolving facility, \$300.0 million of which was drawn at closing (the "Loans") and was repaid in February 2017. The Credit Agreement also provides that, subject to satisfaction of certain conditions, the Company may request that the borrowing capacity under the Credit Agreement be increased by an additional \$300.0 million. The Credit Agreement matures on October 13, 2021.

The Loans will bear interest, at the Company's option, at either a base rate or a Eurodollar rate, in each case plus an applicable margin. Under the Credit Agreement, the applicable margins on base rate loans range from 0.75% to 1.50% and the applicable margins on Eurodollar loans range from 1.75% to 2.50%, in each case based on the Company's consolidated leverage ratio (the ratio of the Company's total consolidated debt to the Company's trailing twelve-month EBITDA).

The Loans are guaranteed by certain of the Company's domestic subsidiaries and secured by substantially all of the Company's assets and the assets of the Company's domestic subsidiaries (excluding intellectual property, owned and leased real property and certain other excluded property) and by the equity interests of the Company's subsidiaries, subject to certain exceptions. Under the terms of the Credit Agreement, the Company must maintain, subject to certain limited exceptions, a consolidated leverage ratio of 3.00 to 1.00 and consolidated EBITDA of at least \$200.0 million, in each case to be measured on a quarterly basis.

The Credit Agreement contains customary representations and warranties and usual and customary affirmative and negative covenants. The Credit Agreement also contains customary events of default. In the case of a continuing event of default, the administrative agent would be entitled to exercise various remedies, including the acceleration of amounts due under outstanding loans.

L. Stock-based Compensation Expense and Share RepurchaseStock-based compensation expense

During the three months ended March 31, 2018 and 2017, the Company recognized the following stock-based compensation expense:

|                                                               | Three Months<br>Ended March 31,<br>2018      2017<br>(in thousands) |          |
|---------------------------------------------------------------|---------------------------------------------------------------------|----------|
| Stock-based compensation expense by type of award:            |                                                                     |          |
| Stock options                                                 | \$26,055                                                            | \$26,981 |
| Restricted stock and restricted stock units (including PSUs)  | 50,418                                                              | 40,745   |
| ESPP share issuances                                          | 2,128                                                               | 2,064    |
| Stock-based compensation expense related to inventories       | (465)                                                               | (351)    |
| Total stock-based compensation included in costs and expenses | \$78,136                                                            | \$69,439 |
| Stock-based compensation expense by line item:                |                                                                     |          |
| Cost of sales                                                 | \$813                                                               | \$457    |
| Research and development expenses                             | 48,488                                                              | 44,837   |
| Sales, general and administrative expenses                    | 28,835                                                              | 24,145   |
| Total stock-based compensation included in costs and expenses | \$78,136                                                            | \$69,439 |



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The following table sets forth the Company's unrecognized stock-based compensation expense as of March 31, 2018, by type of award and the weighted-average period over which that expense is expected to be recognized:

|                                                              | As of March 31, 2018                           |
|--------------------------------------------------------------|------------------------------------------------|
|                                                              | Unrecognized Expense (in thousands)            |
|                                                              | Weighted-average Recognition Period (in years) |
| Type of award:                                               |                                                |
| Stock options                                                | \$203,271 2.79                                 |
| Restricted stock and restricted stock units (including PSUs) | \$386,253 2.76                                 |
| ESPP share issuances                                         | \$2,117 0.43                                   |

The following table summarizes information about stock options outstanding and exercisable as of March 31, 2018:

| Range of Exercise Prices | Options Outstanding |                                                        | Weighted-average Exercise Price (per share) | Options Exercisable |                                             |
|--------------------------|---------------------|--------------------------------------------------------|---------------------------------------------|---------------------|---------------------------------------------|
|                          | Number Outstanding  | Weighted-average Remaining Contractual Life (in years) |                                             | Number Exercisable  | Weighted-average Exercise Price (per share) |
|                          | (in thousands)      |                                                        |                                             | (in thousands)      |                                             |
| \$26.73–\$40.00          | 726                 | 1.63                                                   | \$ 34.46                                    | 726                 | \$ 34.46                                    |
| \$40.01–\$60.00          | 588                 | 4.24                                                   | \$ 49.43                                    | 588                 | \$ 49.43                                    |
| \$60.01–\$80.00          | 644                 | 5.96                                                   | \$ 75.25                                    | 627                 | \$ 75.28                                    |
| \$80.01–\$100.00         | 3,791               | 7.85                                                   | \$ 89.21                                    | 1,357               | \$ 89.45                                    |
| \$100.01–\$120.00        | 972                 | 6.80                                                   | \$ 109.28                                   | 622                 | \$ 109.15                                   |
| \$120.01–\$140.00        | 1,138               | 7.35                                                   | \$ 130.20                                   | 732                 | \$ 129.98                                   |
| \$140.01–\$160.00        | 1,496               | 9.86                                                   | \$ 155.57                                   | 10                  | \$ 155.57                                   |
| \$160.01–\$163.74        | 600                 | 9.25                                                   | \$ 162.94                                   | 76                  | \$ 162.94                                   |
| Total                    | 9,955               | 7.30                                                   | \$ 103.03                                   | 4,738               | \$ 84.36                                    |

## Share repurchase program

The Board of Directors has authorized approved a share repurchase program, pursuant to which the Company is authorized to repurchase up to \$500.0 million of its common stock between February 1, 2018 and December 31, 2019. During the three months ended March 31, 2018, the Company repurchased 67,084 shares of its common stock under the share repurchase program for an aggregate of \$11.3 million (of which \$1.3 million was accrued as of March 31, 2018), including commissions and fees. Under the share repurchase program, the Company is authorized to purchase shares from time to time through open market or privately negotiated transactions and may be made pursuant to Rule 10b5-1 plans or other means as determined by the Company's management and in accordance with the requirements of the Securities and Exchange Commission.

The Company expects to fund any further repurchases of its common stock through a combination of cash on hand and cash generated by operations.

## M. Income Taxes

The Company is subject to U.S. federal, state, and foreign income taxes. For the three months ended March 31, 2018, the Company recorded a benefit from income taxes of \$12.7 million, which included a benefit from income taxes of \$21.9 million from excess tax benefits related to stock-based compensation partially offset by provisions for income taxes of \$6.4 million attributable to noncontrolling interest as a result of an increase in the fair value of the contingent payments payable by the Company to BioAxone and the Company's U.S. state and foreign taxes. The Company has no liability for taxes payable



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by BioAxe and the income tax provision and related liability have been allocated to noncontrolling interest. For the three months ended March 31, 2017, the Company recorded a provision for income taxes of \$4.0 million, which included a provision for income taxes of \$0.4 million related to the Company's VIEs' income tax provision.

As of March 31, 2018 and December 31, 2017, the Company has unrecognized tax benefits of \$4.8 million and \$3.8 million, respectively. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of March 31, 2018, no interest and penalties have been accrued. The Company does not expect that its unrecognized tax benefits will materially increase within the next twelve months. The Company did not recognize any material interest or penalties related to uncertain tax positions as of March 31, 2018 and December 31, 2017.

The Company maintains a valuation allowance on the majority of its net operating losses and other deferred tax assets. Accordingly, the Company has not reported any benefits from income taxes relating to the remaining NOLs and income tax credit carryforwards that will be utilized in future periods in these jurisdictions. On a periodic basis, the Company reassesses the valuation allowance on its deferred income tax assets weighing positive and negative evidence to assess the recoverability of the deferred tax assets. In 2017, the Company reassessed the valuation allowance and considered negative evidence, including its cumulative losses over the three years ended December 31, 2017, and positive evidence, including its income during the year ended December 31, 2017. After assessing both the negative evidence and the positive evidence, the Company concluded that it should continue to maintain the valuation allowance on its net operating losses and the majority of its other deferred tax assets as of December 31, 2017. Based on the Company's recent financial performance and its future projections, it could record a reversal of all, or a portion of the valuation allowance associated with U.S. deferred tax assets in future periods. However, any such change is subject to actual performance and other considerations that may present positive or negative evidence at the time of the assessment. The Company's total deferred tax asset balance subject to the valuation allowance was approximately \$1.6 billion at December 31, 2017.

As described in Note A, "Basis of Presentation and Accounting Policies," the Company adopted amended guidance, effective January 1, 2018. In connection with the adoption of this new standard, the Company recorded a deferred tax asset and corresponding full valuation allowance of \$204.7 million upon adoption of this new guidance equal to the unamortized cost of intellectual property transferred to the United Kingdom in 2014 multiplied by an appropriate statutory rate. As a result, there was no cumulative effect adjustment to accumulated deficit using the modified-retrospective adoption approach.

The Company files U.S. federal income tax returns and income tax returns in various state, local and foreign jurisdictions. The Company is no longer subject to any tax assessment from an income tax examination in the U.S. or any other major taxing jurisdiction for years before 2014, except where the Company has net operating losses or tax credit carryforwards that originate before 2014. The Company currently is under examination in Canada for 2011 through 2013, Germany for 2012 through 2015 and Italy for 2015 and 2016. No adjustments have been reported. The Company is not under examination by any other jurisdictions for any tax year.

In December 2017, the SEC staff issued SAB 118 to address the application of GAAP in situations when a registrant does not have the necessary information available, prepared, or analyzed (including computations) in reasonable detail to complete the accounting for certain income tax effects of H.R.1. The Company has recognized the provisional tax impacts related to deemed repatriated earnings and the revaluation of deferred tax assets and liabilities and included these amounts in its consolidated financial statements for the year ended December 31, 2017. The Company has an accumulated deficit from its foreign operations and does not have an associated liability from the repatriation tax on accumulated earnings in H.R.1. The ultimate impact may differ from these provisional amounts, possibly materially, due to, among other things, additional analysis, changes in interpretations and assumptions the Company has made, additional regulatory guidance that may be issued, and actions the Company may take as a result of H.R.1. The Company's accounting treatment is expected to be complete when the 2017 U.S. corporate income tax return is filed in the second half of 2018.

At March 31, 2018, foreign earnings, which were not significant, have been retained indefinitely by foreign subsidiary companies for reinvestment. Upon repatriation of those earnings, in the form of dividends or otherwise, the Company could be subject to withholding taxes payable to the various foreign countries.

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VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

N. Restructuring Liabilities

The Company has adopted several plans to restructure its facilities and operations for which it has incurred restructuring expenses. During the three months ended March 31, 2018, the Company's restructuring expenses were not significant. During the three months ended March 31, 2017, the Company's restructuring expenses primarily related to its decision to consolidate its research activities into its Boston, Milton Park and San Diego locations commencing in February 2017. The Company closed its research site in Canada as a result of this decision affecting approximately 70 positions. The Company recorded a restructuring credit of \$0.5 million and a restructuring charge of \$9.2 million related to this restructuring event during the three months ended March 31, 2018 and 2017, respectively. As of March 31, 2018, the restructuring liability associated with this event relates to the lease for the research site in Canada that terminates in October 2018 and is not material to the Company's condensed consolidated balance sheet. The Company does not anticipate any significant additional charges related to this restructuring event in the future.

O. Commitments and Contingencies

Guaranties and Indemnifications

As permitted under Massachusetts law, the Company's Articles of Organization and By-laws provide that the Company will indemnify certain of its officers and directors for certain claims asserted against them in connection with their service as an officer or director. The maximum potential amount of future payments that the Company could be required to make under these indemnification provisions is unlimited. However, the Company has purchased directors' and officers' liability insurance policies that could reduce its monetary exposure and enable it to recover a portion of any future amounts paid. No indemnification claims currently are outstanding, and the Company believes the estimated fair value of these indemnification arrangements is minimal.

The Company customarily agrees in the ordinary course of its business to indemnification provisions in agreements with clinical trial investigators and sites in its drug development programs, sponsored research agreements with academic and not-for-profit institutions, various comparable agreements involving parties performing services for the Company and its real estate leases. The Company also customarily agrees to certain indemnification provisions in its drug discovery, development and commercialization collaboration agreements. With respect to the Company's clinical trials and sponsored research agreements, these indemnification provisions typically apply to any claim asserted against the investigator or the investigator's institution relating to personal injury or property damage, violations of law or certain breaches of the Company's contractual obligations arising out of the research or clinical testing of the Company's compounds or drug candidates. With respect to lease agreements, the indemnification provisions typically apply to claims asserted against the landlord relating to personal injury or property damage caused by the Company, to violations of law by the Company or to certain breaches of the Company's contractual obligations. The indemnification provisions appearing in the Company's collaboration agreements are similar to those for the other agreements discussed above, but in addition provide some limited indemnification for its collaborator in the event of third-party claims alleging infringement of intellectual property rights. In each of the cases above, the indemnification obligation generally survives the termination of the agreement for some extended period, although the Company believes the obligation typically has the most relevance during the contract term and for a short period of time thereafter. The maximum potential amount of future payments that the Company could be required to make under these provisions is generally unlimited. The Company has purchased insurance policies covering personal injury, property damage and general liability that reduce its exposure for indemnification and would enable it in many cases to recover all or a portion of any future amounts paid. The Company has never paid any material amounts to defend lawsuits or settle claims related to these indemnification provisions. Accordingly, the Company believes the estimated fair value of these indemnification arrangements is minimal.

Other Contingencies

The Company has certain contingent liabilities that arise in the ordinary course of its business activities. The Company accrues a reserve for contingent liabilities when it is probable that future expenditures will be made and such expenditures can be reasonably estimated. There were no material contingent liabilities accrued as of March 31,

2018 or December 31, 2017.

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### Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations

#### OVERVIEW

We invest in scientific innovation to create transformative medicines for serious diseases. Our business is focused on developing and commercializing therapies for the treatment of cystic fibrosis, or CF, and advancing our research and development programs in other diseases. Our marketed products are KALYDECO (ivacaftor), ORKAMBI (lumacaftor in combination with ivacaftor) and SYMDEKO (tezacaftor in combination with ivacaftor), which are collectively approved to treat approximately 45% of the 75,000 CF patients in North America, Europe and Australia.

#### Cystic Fibrosis

##### Current Medicines

KALYDECO is approved for the treatment of approximately 6,000 CF patients who have the G551D mutation or other specified mutations in their cystic fibrosis transmembrane conductance regulator, or CFTR, gene. ORKAMBI is approved as a treatment for approximately 28,000 patients who have two copies of the F508del mutation, or F508del homozygous, in their CFTR gene. SYMDEKO was approved by the United States Food and Drug Administration, or FDA, in February 2018 for the treatment of patients with CF twelve years of age and older who are F508del homozygous or who have at least one mutation that is responsive to tezacaftor/ivacaftor. SYMDEKO provides an additional treatment option to CF patients who were already eligible for either KALYDECO or ORKAMBI. We expect the European Medicines Agency, or EMA, to complete its review of the Marketing Authorization Application, or MAA, we submitted for tezacaftor in combination with ivacaftor in the second half of 2018.

We continuously seek to increase the number of patients eligible to receive our medicines through label expansions.

Activities in support of this effort include the following:

We have submitted a supplemental new drug application, or sNDA, to the FDA and an MAA line extension to the EMA for ivacaftor in patients with CF 12 months to two years of age. The target date for the FDA to complete its review of the sNDA under the Prescription Drug User Fee Act, or PDUFA, is August 15, 2018. We expect the EMA to complete its review in the first half of 2019.

- We have submitted a new drug application, or NDA, to the FDA and an MAA line extension to the EMA for lumacaftor in combination with ivacaftor in patients with CF two to five years of age. The target date for the FDA to complete its review of the NDA under PDUFA is August 7, 2018. We expect the EMA to complete its review in the first half of 2019.

We have completed enrollment in a Phase 3 clinical trial evaluating tezacaftor in combination with ivacaftor in patients with CF six to eleven years of age who are F508del homozygous or who have at least one mutation in their CFTR gene that is responsive to tezacaftor/ivacaftor. We expect to receive data from this clinical trial in the second half of 2018.

• We are evaluating ivacaftor in a Phase 3 clinical trial in patients with CF less than 12 months of age.

• We expect to commence a Phase 3 clinical trial evaluating lumacaftor in combination with ivacaftor in patients with CF 12 months to two years of age in the second half of 2018.

##### Next-generation CFTR Corrector Compounds

We have initiated Phase 3 clinical trials for two next-generation corrector compounds, VX-659 and VX-445, as part of separate triple combination regimens with tezacaftor and ivacaftor. The Phase 3 development program for VX-659, which was initiated in February 2018, and the Phase 3 development program for VX-445, which was initiated in April 2018, are each comprised of two clinical trials. The first clinical trial in each program will enroll approximately 360 patients with CF who have one copy of the F508del mutation in their CFTR gene and a second mutation that results in minimal CFTR function, who we refer to as F508del/Min patients. The primary efficacy endpoint of the first clinical trial in each program is the mean absolute change from baseline in percent predicted forced expiratory volume in one second, or ppFEV1, at week four of treatment with the triple combination regimen versus placebo. The second clinical trial in each program will enroll approximately 100 F508del homozygous patients. The primary efficacy endpoint of the second clinical trial in each program is the mean absolute change from baseline in ppFEV1 at week four of treatment with the triple combination regimen compared to tezacaftor in combination with ivacaftor. The clinical trials are designed to support the approval for the respective triple combination regimen in the respective patient populations using data from the primary efficacy endpoints and 12- or 24-week safety data.



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We believe the triple combination regimens we are evaluating could potentially provide benefits to all CF patients who have at least one F508del mutation in their CFTR gene (approximately 90% of all CF patients). This would include (i) the first treatment option that treats the underlying cause of CF for F508del/Min patients, and (ii) an additional treatment option for patients with CF who are eligible for KALYDECO, ORKAMBI and/or SYMDEKO. In the second quarter of 2018, we announced positive clinical data from (i) a Phase 2 clinical trial arm evaluating VX-561 in combination with ivacaftor and VX-659 and (ii) a Phase 2 clinical trial arm evaluating VX-561 in combination with ivacaftor and VX-445. After discussing this data with the FDA, we are planning to conduct additional dose-ranging with VX-561 to support potential late-stage development of future once-daily triple combination regimens.

### Research and Development

We have a number of ongoing research and development programs in other diseases that we are conducting independently or in collaboration with third parties. We are developing VX-150 and VX-128 as treatments for pain, co-developing CTX001, an investigational gene editing treatment, for the treatment of beta-thalassemia and sickle cell disease, with CRISPR Therapeutics AG, or CRISPR, and developing VX-210 as a treatment for acute spinal cord injury. We plan to continue investing in our research programs and fostering scientific innovation in order to identify and develop transformative medicines for people with serious diseases. In addition to continuing our research in cystic fibrosis, pain and hemoglobinopathies, our current internal research programs include programs targeting alpha-1 antitrypsin deficiency and polycystic kidney disease. To supplement our internal research programs, we seek to collaborate with biopharmaceutical and technology companies, leading academic research institutions, government laboratories, foundations and other organizations as needed to advance research in our areas of therapeutic interest and to access technologies needed to execute on our strategy. We believe that pursuing research in diverse areas allows us to balance the risks inherent in drug development and may provide drug candidates that will form our pipeline in future years.

### Drug Discovery and Development

Discovery and development of a new pharmaceutical product is a difficult and lengthy process that requires significant financial resources along with extensive technical and regulatory expertise and can take 10 to 15 years or more. Potential drug candidates are subjected to rigorous evaluations, driven in part by stringent regulatory considerations, designed to generate information concerning efficacy, side-effects, proper dosage levels and a variety of other physical and chemical characteristics that are important in determining whether a drug candidate should be approved for marketing as a pharmaceutical product. Most chemical compounds that are investigated as potential drug candidates never progress into development, and most drug candidates that do advance into development never receive marketing approval. Because our investments in drug candidates are subject to considerable risks, we closely monitor the results of our discovery, research, clinical trials and nonclinical studies and frequently evaluate our drug development programs in light of new data and scientific, business and commercial insights, with the objective of balancing risk and potential. This process can result in abrupt changes in focus and priorities as new information becomes available and as we gain additional understanding of our ongoing programs and potential new programs, as well as those of our competitors.

If we believe that data from a completed registration program support approval of a drug candidate, we submit an NDA to the FDA requesting approval to market the drug candidate in the United States and seek analogous approvals from comparable regulatory authorities in foreign jurisdictions. To obtain approval, we must, among other things, demonstrate with evidence gathered in nonclinical studies and well-controlled clinical trials that the drug candidate is safe and effective for the disease it is intended to treat and that the manufacturing facilities, processes and controls for the manufacture of the drug candidate are adequate. The FDA and foreign regulatory authorities have substantial discretion in deciding whether or not a drug candidate should be granted approval based on the benefits and risks of the drug candidate in the treatment of a particular disease, and could delay, limit or deny regulatory approval. If regulatory delays are significant or regulatory approval is limited or denied altogether, our financial results and the commercial prospects for the drug candidate involved will be harmed.

### Regulatory Compliance

Our marketing of pharmaceutical products is subject to extensive and complex laws and regulations. We have a corporate compliance program designed to actively identify, prevent and mitigate risk through the implementation of compliance policies and systems, and through the promotion of a culture of compliance. Among other laws, regulations and standards, we are subject to various U.S. federal and state laws, and comparable foreign laws, pertaining to health care fraud and abuse, including anti-kickback and false claims laws, and laws prohibiting the promotion of drugs for unapproved or off-label uses. Anti-kickback laws make it illegal for a prescription drug manufacturer to solicit, offer, receive or pay any remuneration to induce the referral of business, including the purchase or prescription of a particular drug that is reimbursed by a state or federal program. False claims laws prohibit anyone from knowingly or willfully presenting for payment to third-party payors, including Medicare and Medicaid, claims for reimbursed drugs or services that are false or fraudulent, claims for items or

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services not provided as claimed, or claims for medically unnecessary items or services. We are subject to laws and regulations that regulate the sales and marketing practices of pharmaceutical manufacturers, as well as laws such as the U.S. Foreign Corrupt Practices Act that govern our international business practices with respect to payments to government officials. We expect to continue to devote substantial resources to maintain, administer and expand these compliance programs globally.

### Reimbursement

Sales of our products depend, to a large degree, on the extent to which our products are covered by third-party payors, such as government health programs, commercial insurance and managed health care organizations. We dedicate substantial management and other resources in order to obtain and maintain appropriate levels of reimbursement for our products from third-party payors, including governmental organizations in the United States and ex-U.S. markets. In the United States, we continue to engage in discussions with numerous commercial insurers and managed health care organizations, along with government health programs that are typically managed by authorities in the individual states. In Europe and other ex-U.S. markets, we work to obtain government reimbursement for ORKAMBI on a country-by-country basis, because in many foreign countries patients are unable to access prescription pharmaceutical products that are not reimbursed by their governments.

In the United States, we worked successfully with third party payors in order to promptly obtain appropriate levels of reimbursement for KALYDECO and ORKAMBI and are beginning that process for SYMDEKO. We also successfully obtained reimbursement for KALYDECO in each significant ex-U.S. market within two years of approval. Since we obtained approval for ORKAMBI in 2015, we have experienced significant challenges in obtaining reimbursement for ORKAMBI in ex-U.S. markets, to date, having reached a pricing and reimbursement agreement for ORKAMBI in several European countries, including Germany, Ireland and Italy, but remain in negotiations with a number of other European countries, including the United Kingdom and France, which represent significant potential markets for ORKAMBI. While we have innovative reimbursement arrangements in place in certain ex-U.S. jurisdiction such as Ireland that will allow rapid access to tezacaftor in combination with ivacaftor, if approved, and ORKAMBI for younger patients, in most significant markets we will need to obtain country-by-country reimbursement for each new medicine and each label expansion for a current medicine.

### Collaboration Arrangements and Strategic Investments

#### In-License Agreements

We have entered into collaborations with biotechnology and pharmaceutical companies in order to acquire rights or to license drug candidates or technologies that enhance our pipeline and/or our research capabilities. Over the last several years, we entered into collaboration agreements with:

- CRISPR, pursuant to which we are collaborating on the discovery and development of potential new treatments aimed at the underlying genetic causes of human diseases using CRISPR-Cas9 gene editing technology;

- Moderna Therapeutics, Inc., or Moderna, pursuant to which we are seeking to identify and develop messenger ribonucleic acid, or mRNA therapeutics for the treatment of CF;

- BioAxone Biosciences, Inc., or BioAxone, pursuant to which we are evaluating VX-210 as a potential treatment for patients who have spinal cord injuries; and

- Parion Sciences, Inc., or Parion, pursuant to which we are developing epithelial sodium channel, or ENaC, inhibitors for the treatment of pulmonary diseases.

Generally, when we in-license a technology or drug candidate, we make upfront payments to the collaborator, assume the costs of the program and agree to make contingent payments, which could consist of milestone, royalty and option payments. Depending on many factors, including the structure of the collaboration, the significance of the drug candidate that we license to the collaborator's operations and the other activities in which our collaborators are engaged, the accounting for these transactions can vary significantly. For example, the upfront payments and expenses incurred in connection with our CRISPR and Moderna collaborations are being expensed as research expenses because the collaboration represents a small portion of these collaborators overall business. CRISPR and Moderna's activities unrelated to our collaborations have no effect on our consolidated financial statements. Parion and BioAxone have historically been accounted for as variable interest entities, or VIEs, and historically have been included in our consolidated financial statements due to (i) the significance of the respective licensed programs to

Parion and BioAxone as a whole, (ii) our power to control the significant activities under each collaboration and (iii) our obligation to absorb losses and right to receive benefits that potentially could be significant. As of September 30, 2017, we determined that the above conditions were no longer satisfied with respect to Parion following the

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results of a Phase 2 clinical trial of VX-371 that did not meet its primary efficacy endpoint. As a result, we no longer account for Parion as a VIE and have deconsolidated Parion from our consolidated financial statements as of September 30, 2017. BioAxone continues to be accounted for as a VIE and remains included in our consolidated financial statements as of March 31, 2018.

Collaborators we account for as a VIE may engage in activities unrelated to our collaboration. The revenues and expenses unrelated to the programs we in-license from our VIEs have historically been immaterial to our consolidated financial statements. With respect to each of Parion, prior to its deconsolidation as of September 30, 2017, and BioAxone, the activities unrelated to our collaborations with these entities have represented approximately 2% or less of our total revenues and total expenses on an annual basis. As a result of the deconsolidation of Parion, we expect these amounts to decrease in future periods. For any consolidated VIEs, we evaluate the fair value of the contingent payments payable by us on a quarterly basis. Changes in the fair value of these contingent future payments affect net income attributable to Vertex on a dollar-for-dollar basis, with increases in the fair value of contingent payments payable by us to a VIE resulting in a decrease in net income attributable to Vertex (or an increase in net loss attributable to Vertex) and decreases in the fair value of contingent payments payable by us to a VIE resulting in an increase in net income attributable to Vertex (or decrease in net loss attributable to Vertex). For additional information regarding our VIEs see Note C, "Collaborative Arrangements and Acquisitions," and our critical accounting policies in our 2017 Annual Report on Form 10-K.

### Out-License Agreements

We also out-licensed internally developed programs to collaborators who are leading the development of these programs. These outlicense arrangements include our collaboration agreements with:

- Janssen Pharmaceuticals, Inc., which is developing pimodivir (formerly VX-787) for the treatment of influenza; and
- Merck KGaA, which licensed four oncology research and development programs from us in early 2017.

Pursuant to these out-licensing arrangements, our collaborators are responsible for the research, development and commercialization costs associated with these programs and we are entitled to receive contingent milestone and/or royalty payments. As a result, we do not expect to incur significant expenses in connection with these programs and have the potential for future collaborative and/or royalty revenues resulting from these programs.

### Strategic Investments

In connection with our business development activities, we have periodically made equity investments in our collaborators. As of March 31, 2018 and December 31, 2017, we held strategic equity investments in CRISPR, a public company, and Moderna, a private company, and we may make additional strategic investments in the future. While our general investment strategy is focused on capital preservation, our strategic investments are maintained and managed separately from our other cash, cash equivalents and marketable securities.

Until December 31, 2017, changes in the fair value of these strategic investments were reflected on our balance sheet, but did not affect our net income until the related gains or losses were realized. As a result of new accounting guidance, effective January 1, 2018, any changes in the fair value of equity investments with readily determinable fair values (including publicly traded securities such as CRISPR) are recorded to other income (expense), net in our condensed consolidated statement of operations. For equity investments without readily determinable fair values (including private equity investments such as Moderna), each reporting period we are required to re-evaluate the carrying value of the investment, which may result in other income (expense).

In the first quarter of 2018, we recorded other income, net of \$95.5 million related to the increase in the fair value of our investments in CRISPR and Moderna, which is included in net income attributable to Vertex. To the extent that we continue to hold strategic investments and in particular strategic investments in publicly traded companies, we will record on a quarterly basis other income (expense) related to these strategic investments. Due to the high volatility of stocks in the biotechnology industry, we expect the value of these strategic investments to fluctuate and that the increases or decreases in the fair value of these strategic investments may have a material impact on our net income (expense) and our profitability under GAAP on a quarterly and/or annual basis.



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## RESULTS OF OPERATIONS

|                                   | Three Months Ended March 31, |            | Increase/(Decrease) |        |
|-----------------------------------|------------------------------|------------|---------------------|--------|
|                                   | 2018                         | 2017       | \$                  | %      |
|                                   | (in thousands)               |            |                     |        |
| Revenues                          | \$ 640,799                   | \$ 714,718 | \$ (73,919 )        | (10 )% |
| Operating costs and expenses      | 511,898                      | 443,876    | 68,022              | 15 %   |
| Other items, net                  | 81,362                       | (23,086 )  | 104,448             | n/a    |
| Net income attributable to Vertex | \$ 210,263                   | \$ 247,756 | \$ (37,493 )        | (15 )% |

|                                                                         |         |         |
|-------------------------------------------------------------------------|---------|---------|
| Net income per diluted share attributable to Vertex common shareholders |         |         |
| Diluted shares used in per share calculations                           |         |         |
|                                                                         | \$ 0.81 | \$ 0.99 |

## Net Income Attributable to Vertex

Net income attributable to Vertex was \$210.3 million in the first quarter of 2018 as compared to net income attributable to Vertex of \$247.8 million in the first quarter of 2017. Net income attributable to Vertex in the first quarter of 2018 reflected increases in CF net product revenues of \$157.2 million from sales of ORKAMBI, KALYDECO and SYMDEKO as compared to the first quarter of 2017. Our total revenues decreased in the first quarter of 2018 as compared to the first quarter of 2017 primarily due to \$230.0 million in one-time collaborative revenues in the first quarter of 2017 related to an upfront payment from Merck KGaA. The increase in operating costs and expenses in the first quarter of 2018 as compared to the first quarter of 2017 included increases in cost of sales, research and development expenses and sales, general and administrative expenses offset by a decrease in restructuring expenses.

Other items, net, in the first quarter of 2018 primarily related to a \$92.5 million unrealized gain associated with an increase in the fair value of our investment in CRISPR. This unrealized gain is included in other items, net due to new accounting guidance that became effective on January 1, 2018. Other items, net, in the first quarter of 2017 primarily related to interest expense.

## Diluted Net Income Per Share Attributable to Vertex Common Shareholders

Diluted net income per share attributable to Vertex common shareholders was \$0.81 in the first quarter of 2018 as compared to diluted net income per share attributable to Vertex common shareholders of \$0.99 in the first quarter of 2017.

## Revenues

|                        | Three Months Ended March 31, |           | Increase/(Decrease) |        |
|------------------------|------------------------------|-----------|---------------------|--------|
|                        | 2018                         | 2017      | \$                  | %      |
|                        | (in thousands)               |           |                     |        |
| Product revenues, net  | \$637,729                    | \$480,622 | \$ 157,107          | 33 %   |
| Royalty revenues       | 1,356                        | 1,551     | (195 )              | (13 )% |
| Collaborative revenues | 1,714                        | 232,545   | (230,831 )          | (99 )% |
| Total revenues         | \$640,799                    | \$714,718 | \$ (73,919 )        | (10 )% |
| Product Revenues, Net  |                              |           |                     |        |

Increase/(Decrease)

|                                | Three Months<br>Ended March 31, |           |            |      |
|--------------------------------|---------------------------------|-----------|------------|------|
|                                | 2018                            | 2017      | \$         | %    |
|                                | (in thousands)                  |           |            |      |
| KALYDECO                       | \$249,539                       | \$185,715 | \$ 63,824  | 34 % |
| ORKAMBI                        | 354,066                         | 294,861   | 59,205     | 20 % |
| SYMDEKO                        | 34,124                          | —         | 34,124     | n/a  |
| Total CF product revenues, net | \$637,729                       | \$480,576 | \$ 157,153 | 33 % |

In the first quarter of 2018, our total CF net product revenues increased by \$157.2 million as compared to the first quarter of 2017. The increase in total CF net product revenues was due to (i) increased KALYDECO net product revenues, (ii) increased ORKAMBI net product revenues and (ii) net product revenues from sales of SYMDEKO, which was approved by the FDA in February 2018. We believe that our total CF net product revenues will continue to increase on a quarterly basis during the remainder of 2018.

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KALYDECO net product revenues increased in the first quarter of 2018 as compared to the first quarter of 2017 primarily due to additional patients being treated with KALYDECO as we completed reimbursement discussions in various ex-U.S. jurisdictions and as we increased the number of patients eligible to receive KALYDECO through label expansions. In the first quarter of 2018 and 2017, we recognized approximately \$86.2 million and \$84.2 million, respectively, in ex-U.S. KALYDECO net product revenues.

In the first quarter of 2018, ORKAMBI net product revenues increased to \$354.1 million from \$294.9 million in first quarter of 2017, due to increases in U.S. and ex-U.S. ORKAMBI net products. In the first quarter of 2018 and 2017, we recognized approximately \$71.8 million and \$31.4 million, respectively, in ex-U.S. ORKAMBI net product revenues. Our condensed consolidated balance sheet includes \$268.4 million collected as of March 31, 2018 in France related to ORKAMBI supplied under early access programs at the invoiced price. Pursuant to new revenue recognition guidance that became effective under GAAP on January 1, 2018, we have recognized limited net product revenues to date on sales of ORKAMBI in France due to ongoing pricing discussions regarding the reimbursement rate for ORKAMBI. Please refer to Note A, "Basis of Presentation and Accounting Policies," for a discussion of the application of the new revenue recognition guidance and to Note B, "Revenue Recognition," for a discussion of our accounting treatment for our early access programs for ORKAMBI in France.

SYMDEKO was approved in the United States in February 2018. We expect SYMDEKO net product revenues to increase on a quarterly basis for the remainder of 2018 as additional patients initiate treatment with SYMDEKO. SYMDEKO provides an additional treatment option to CF patients in the United States who were already eligible for either ORKAMBI or KALYDECO. Accordingly, patients who initiate treatment with SYMDEKO include both patients who were not previously receiving treatment with one of our CF medicines and patients who are switching from either ORKAMBI or KALYDECO to SYMDEKO. While patients switching from our other CF medicines to SYMDEKO is not expected to have a significant effect on our total CF net product revenues, patients who switch will have a negative effect on the net product revenues from ORKAMBI and/or KALYDECO, and that effect may be substantial in future periods.

**Royalty Revenues**

Our royalty revenues were \$1.4 million in the first quarter of 2018 as compared to \$1.6 million in the first quarter of 2017. Our royalty revenues consist of revenues related to a cash payment we received in 2008 when we sold our rights to certain HIV royalties. We expect to continue to record royalty revenues related to this payment through the first half of 2019. Other future royalty revenues will be dependent on if, and when, our collaborators, including Janssen, Inc. and Merck KGaA, are able to successfully develop drug candidates that we have outlicensed to them.

**Collaborative Revenues**

Our collaborative revenues were \$1.7 million in the first quarter of 2018 as compared to \$232.5 million in the first quarter of 2017. The decrease in our collaborative revenues during the first quarter of 2018 as compared to the first quarter of 2017 was primarily due to revenue recognized related to the one-time upfront payment earned in the first quarter of 2017 from Merck KGaA. Our collaborative revenues have historically fluctuated significantly from one period to another and may continue to fluctuate in the future.

**Operating Costs and Expenses**

|                                            | Three Months Ended |           | Increase/(Decrease) |      |
|--------------------------------------------|--------------------|-----------|---------------------|------|
|                                            | March 31,          |           |                     |      |
|                                            | 2018               | 2017      | \$                  | %    |
|                                            | (in thousands)     |           |                     |      |
| Cost of sales                              | \$71,613           | \$46,988  | \$ 24,625           | 52 % |
| Research and development expenses          | 310,553            | 273,563   | 36,990              | 14 % |
| Sales, general and administrative expenses | 129,808            | 113,326   | 16,482              | 15 % |
| Restructuring (income) expenses            | (76)               | 9,999     | (10,075)            | n/a  |
| Total costs and expenses                   | \$511,898          | \$443,876 | \$ 68,022           | 15 % |

**Cost of Sales**

Our cost of sales primarily consists of the cost of producing inventories that corresponded to product revenues for the reporting period, plus the third-party royalties payable on our net sales of our products. Cost of sales also includes a

small subroyalty payable to a third party related to royalty revenues that we historically classified under “Royalty Expenses.” Pursuant to our agreement with Cystic Fibrosis Foundation Therapeutics Incorporated, or CFFT, our tiered third-party royalties on sales of KALYDECO, ORKAMBI and SYMDEKO, calculated as a percentage of net sales, range from the single

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digits to the sub-teens. As a result of the tiered royalty rate, which resets annually, our cost of sales as a percentage of CF net product revenues are lower at the beginning of each calendar year.

Our cost of sales have been increasing due primarily to increased net product revenues. In 2018, we expect our cost of sales as a percentage of total CF net product revenues to be similar to the cost of sales as a percentage of total CF net product revenues in 2017.

## Research and Development Expenses

|                                         | Three Months<br>Ended March 31, |           | Increase/(Decrease) |      |
|-----------------------------------------|---------------------------------|-----------|---------------------|------|
|                                         | 2018                            | 2017      | \$                  | %    |
|                                         | (in thousands)                  |           |                     |      |
| Research expenses                       | \$77,942                        | \$73,056  | \$ 4,886            | 7 %  |
| Development expenses                    | 232,611                         | 200,507   | 32,104              | 16 % |
| Total research and development expenses | \$310,553                       | \$273,563 | \$ 36,990           | 14 % |

Our research and development expenses include internal and external costs incurred for research and development of our drugs and drug candidates. We do not assign our internal costs, such as salary and benefits, stock-based compensation expense, laboratory supplies and other direct expenses and infrastructure costs, to individual drugs or drug candidates, because the employees within our research and development groups typically are deployed across multiple research and development programs. These internal costs are significantly greater than our external costs, such as the costs of services provided to us by clinical research organizations and other outsourced research, which we allocate by individual program. All research and development costs for our drugs and drug candidates are expensed as incurred.

Since January 2015, we have incurred \$3.7 billion in research and development expenses associated with drug discovery and development. The successful development of our drug candidates is highly uncertain and subject to a number of risks. In addition, the duration of clinical trials may vary substantially according to the type, complexity and novelty of the drug candidate and the disease indication being targeted. The FDA and comparable agencies in foreign countries impose substantial requirements on the introduction of therapeutic pharmaceutical products, typically requiring lengthy and detailed laboratory and clinical testing procedures, sampling activities and other costly and time-consuming procedures. Data obtained from nonclinical and clinical activities at any step in the testing process may be adverse and lead to discontinuation or redirection of development activities. Data obtained from these activities also are susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The duration and cost of discovery, nonclinical studies and clinical trials may vary significantly over the life of a project and are difficult to predict. Therefore, accurate and meaningful estimates of the ultimate costs to bring our drug candidates to market are not available.

In 2017 and the first quarter of 2018, costs related to our CF programs represented the largest portion of our development costs. Any estimates regarding development and regulatory timelines for our drug candidates are highly subjective and subject to change. We expect the EMA to complete its review of our MAA for tezacaftor in combination with ivacaftor in the second half of 2018. We cannot make a meaningful estimate when, if ever, our other clinical development programs will generate revenues and cash flows.

## Research Expenses

|                                               | Three Months<br>Ended March 31, |          | Increase/(Decrease) |      |
|-----------------------------------------------|---------------------------------|----------|---------------------|------|
|                                               | 2018                            | 2017     | \$                  | %    |
|                                               | (in thousands)                  |          |                     |      |
| Research Expenses:                            |                                 |          |                     |      |
| Salary and benefits                           | \$24,088                        | \$21,533 | \$ 2,555            | 12 % |
| Stock-based compensation expense              | 14,760                          | 13,691   | 1,069               | 8 %  |
| Laboratory supplies and other direct expenses | 10,916                          | 11,365   | (449)               | (4)% |
| Outsourced services                           | 7,897                           | 7,337    | 560                 | 8 %  |
| Collaboration and asset acquisition payments  | 308                             | —        | 308                 | n/a  |

|                         |          |          |          |   |   |
|-------------------------|----------|----------|----------|---|---|
| Infrastructure costs    | 19,973   | 19,130   | 843      | 4 | % |
| Total research expenses | \$77,942 | \$73,056 | \$ 4,886 | 7 | % |

We maintain a substantial investment in research activities. Our research expenses increased by 7% in the first quarter of 2018 as compared to the first quarter of 2017 due primarily to increases in salary and benefits. We expect to continue to invest in our research programs with a focus on identifying drug candidates with the goal of creating transformative medicines.

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## Development Expenses

|                                               | Three Months<br>Ended March 31, |           | Increase/(Decrease) |     |   |
|-----------------------------------------------|---------------------------------|-----------|---------------------|-----|---|
|                                               | 2018                            | 2017      | \$                  | %   |   |
|                                               | (in thousands)                  |           |                     |     |   |
| Development Expenses:                         |                                 |           |                     |     |   |
| Salary and benefits                           | \$57,002                        | \$48,971  | \$ 8,031            | 16  | % |
| Stock-based compensation expense              | 33,728                          | 31,146    | 2,582               | 8   | % |
| Laboratory supplies and other direct expenses | 14,473                          | 14,013    | 460                 | 3   | % |
| Outsourced services                           | 84,719                          | 73,185    | 11,534              | 16  | % |
| Collaboration and asset acquisition payments  | 250                             | 250       | —                   | —   | % |
| Drug supply costs                             | 8,421                           | 1,949     | 6,472               | 332 | % |
| Infrastructure costs                          | 34,018                          | 30,993    | 3,025               | 10  | % |
| Total development expenses                    | \$232,611                       | \$200,507 | \$ 32,104           | 16  | % |

Our development expenses increased by 16% in the first quarter of 2018 as compared to the first quarter of 2017, primarily due to increased outsourced services and drug supply expenses related to ongoing clinical trials, including trials involving our next-generation CFTR corrector compounds that we are evaluating as part of triple combination treatment regimens, as well as an increase in salary and benefits associated with an increase in our headcount. We expect our development expenses to increase on a quarterly basis during the remainder of 2018 as compared to 2017 due to expenses related to the ongoing Phase 3 development of our triple combination regimens.

## Sales, General and Administrative Expenses

|  | Three Months<br>Ended March 31, |      | Increase/(Decrease) |   |  |
|--|---------------------------------|------|---------------------|---|--|
|  | 2018                            | 2017 | \$                  | % |  |
|  | (in thousands)                  |      |                     |   |  |

|                                            |           |           |           |    |   |
|--------------------------------------------|-----------|-----------|-----------|----|---|
| Sales, general and administrative expenses | \$129,808 | \$113,326 | \$ 16,482 | 15 | % |
|--------------------------------------------|-----------|-----------|-----------|----|---|

Sales, general and administrative expenses increased by 15% in the first quarter of 2018 as compared to the first quarter of 2017, primarily due to increased global support for KALYDECO and ORKAMBI and costs incurred to prepare for the launch of SYMDEKO in the United States.

## Restructuring Expenses

We recorded restructuring credits of \$0.1 million in the first quarter of 2018 as compared to restructuring expenses of \$10.0 million in the first quarter of 2017. The restructuring expenses in the first quarter of 2017 primarily related to our decision to consolidate our research activities into our Boston, Milton Park and San Diego locations and to close our research site in Canada.

## Other Items, Net

## Interest Expense, Net

Our interest expense, net relates primarily to interest expenses associated with our real estate leases and outstanding debt, if any, partially offset by interest income from the investment of our cash, cash equivalents and marketable securities. Interest expense, net was \$11.1 million in the first quarter of 2018 as compared to \$16.8 million in the first quarter of 2017. The decrease in interest expense, net in the first quarter of 2018 as compared to the first quarter of 2017 was primarily due to (i) a \$4.3 million increase in our interest income in the first quarter of 2018 as compared to the first quarter of 2017 resulting from an increase in our cash, cash equivalents and marketable securities and (ii) a decrease in interest expense due to the repayment of \$300.0 million outstanding under our revolving credit facility in February 2017. For the remainder of 2018, we expect to incur approximately \$50 million in interest expenses related to our real estate leases and that our interest expense related to our outstanding debt will be dependent on whether, and to what extent, we reborrow amounts under our credit facility.



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## Other Income (Expense), Net

Other income (expense), net was income of \$96.8 million in the first quarter of 2018 as compared to expense of \$0.5 million in the first quarter of 2017. Other income (expense), net in the first quarter of 2018 was primarily related to an increase in fair value of our equity investment in CRISPR of \$92.5 million in the first quarter of 2018. New accounting guidance, which became effective January 1, 2018, requires that changes in the fair value of our equity investments are recorded in other income (expense), net in our condensed consolidated statement of operations. In prior periods, changes in the fair value of our equity investments were not reflected in our condensed consolidated statement of operations and a gain or loss was only recognized upon a sale of the underlying securities. As a result of the new guidance, our other income (expense), net will fluctuate based on increases or decreases in the fair value of these investments.

Other income (expense), net in the first quarter of 2017 was primarily due to foreign exchange losses.

## Income Taxes

We recorded a benefit from income taxes of \$12.7 million in the first quarter of 2018 as compared to a provision for income taxes of \$4.0 million in the first quarter of 2017. The benefit from income taxes in the first quarter of 2018 was primarily related to a benefit from income taxes of \$21.9 million related to excess tax benefits related to stock-based compensation offset by provisions for income taxes of \$6.4 million attributable to noncontrolling interest as a result of an increase in the fair value of the contingent payments payable by us to BioAxone and our U.S. state and foreign taxes. We expect to record excess tax benefits from income taxes of a similar nature in the second and third quarter of the 2018. In the fourth quarter of 2018, we expect to record a provision for income taxes equal to the cumulative benefits recorded through the first three quarters of 2018. As a result, these excess tax benefits and provisions recorded on a quarterly basis are not expected to have any effect on our annual provision for (benefit from) income taxes. The provision for income taxes in the first quarter of 2017 was primarily related to our U.S. state and foreign taxes.

As discussed in Note M, "Income Taxes," we continue to maintain a valuation allowance on the majority of our net operating losses and other deferred tax assets.

## Noncontrolling Interest (VIEs)

The net (income) loss attributable to noncontrolling interest (VIEs) recorded on our condensed consolidated statements of operations reflects BioAxone's net (income) loss for the reporting period, adjusted for any changes in the noncontrolling interest holders' claim to net assets, including contingent milestone, royalty and option payments. A summary of net income attributable to noncontrolling interest related to our VIEs for the first quarter of 2018 and 2017 is as follows:

|                                                                                                                                 | Three Months<br>Ended March 31, |           |
|---------------------------------------------------------------------------------------------------------------------------------|---------------------------------|-----------|
|                                                                                                                                 | 2018                            | 2017      |
| Loss attributable to noncontrolling interest before provision for income taxes and changes in fair value of contingent payments | \$557                           | \$1,547   |
| Provision for income taxes                                                                                                      | 6,405                           | 391       |
| Increase in fair value of contingent payments                                                                                   | (24,000 )                       | (3,730 )  |
| Net income attributable to noncontrolling interest                                                                              | \$(17,038)                      | \$(1,792) |

The net income attributable to noncontrolling interest in the first quarter of 2018 was primarily due to an increase in the fair value of contingent payments related to the expiration of our option to purchase BioAxone that increased the probability related to a \$10.0 million license continuation fee for VX-210 that was paid in the first quarter of 2018 and the probability that additional milestone and royalty payments related to the BioAxone Agreement will be achieved, partially offset by a provision for income taxes related to this increase. The net income attributable to noncontrolling interest in the first quarter of 2017 was primarily due to an increase in the fair value of contingent payments related to changes in market interest rates and the time value of money.



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## LIQUIDITY AND CAPITAL RESOURCES

The following table summarizes the components of our financial condition as of March 31, 2018 and December 31, 2017:

|                                                  | March 31,<br>2018<br>(in thousands) | December<br>31,<br>2017 | Increase/(Decrease) |      |
|--------------------------------------------------|-------------------------------------|-------------------------|---------------------|------|
|                                                  |                                     |                         | \$                  | %    |
| Cash, cash equivalents and marketable securities | \$2,477,017                         | \$2,088,666             | \$ 388,351          | 19 % |
| Working Capital                                  |                                     |                         |                     |      |
| Total current assets                             | 3,041,116                           | 2,648,963               | 392,153             | 15 % |
| Total current liabilities                        | 827,312                             | 807,260                 | 20,052              | 2 %  |
| Total working capital                            | \$2,213,804                         | \$1,841,703             | \$ 372,101          | 20 % |

As of March 31, 2018, we had cash, cash equivalents and marketable securities of \$2.5 billion, which represented an increase of \$388 million from \$2.1 billion as of December 31, 2017. In the first quarter of 2018, our cash, cash equivalents and marketable securities balance increased due to cash receipts from product sales, a \$92.5 million increase in the fair value of our investment in CRISPR based on an increase in the price of CRISPR's common stock and \$88.4 million cash received from issuances of common stock under our employee benefit plans partially offset by cash expenditures. We expect that our future cash flows will be substantially dependent on CF product sales.

As of March 31, 2018, total working capital was \$2.2 billion, which represented an increase of \$372 million from \$1.8 billion as of December 31, 2017. The most significant items that increased total working capital in the first quarter of 2018 were \$252.7 million cash provided by operations, a \$92.5 million increase in the fair value of our investment in CRISPR based on an increase in the price of CRISPR's common stock and \$88.4 million cash received from issuances of common stock under our employee benefit plans.

Sources of Liquidity

We intend to rely on our existing cash, cash equivalents and marketable securities together with cash flows from product sales as our primary source of liquidity. We are receiving cash flows from sales of ORKAMBI, KALYDECO and SYMDEKO from the United States and ORKAMBI and KALYDECO from ex-U.S. markets. We expect the EMA to complete its review of the MAA we submitted for tezacaftor in combination with ivacaftor in the second half of 2018. Future net product revenues for ORKAMBI and, if approved, tezacaftor in combination with ivacaftor, from ex-U.S. markets will be dependent on, among other things, the timing of and ability to complete reimbursement discussions in European countries.

We may borrow up to \$500.0 million pursuant to a revolving credit facility that we entered into in October 2016. We may repay and reborrow amounts under the revolving credit agreement without penalty. Subject to certain conditions, we may request that the borrowing capacity under this credit agreement be increased by an additional \$300.0 million. In the first quarter of 2018, we received significant proceeds from the issuance of common stock under our employee benefit plans, but the amount and timing of future proceeds from employee benefits plans is uncertain. In the first quarter of 2018, the value of our strategic investment in CRISPR increased significantly, but the future value of this strategic investment is uncertain. Other possible sources of liquidity include strategic collaborative agreements that include research and/or development funding, commercial debt, public and private offerings of our equity and debt securities, development milestones and royalties on sales of products, software and equipment leases, strategic sales of assets or businesses and financial transactions. Negative covenants in our credit agreement may prohibit or limit our ability to access these sources of liquidity.

Future Capital Requirements

We incur substantial operating expenses to conduct research and development activities and to operate our organization. We have substantial facility and capital lease obligations, including leases for two buildings in Boston, Massachusetts that continue through 2028 and capital expenditures for our building under construction in San Diego, California. As of March 31, 2018, we have accrued approximately \$268.4 million from ORKAMBI early access programs in France. We expect we will be required to repay a portion of the collected amounts to the French government based on the difference between the invoiced price of ORKAMBI and the final price for ORKAMBI in

France once we conclude our ongoing pricing

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discussions with the French government. To the extent we borrow amounts under the credit agreement we entered into in October 2016, we would be required to repay any outstanding principal amounts in 2021.

In addition, we have entered into certain collaboration agreements with third parties that include the funding of certain research, development and commercialization efforts with the potential for future milestone and royalty payments by us upon the achievement of pre-established developmental and regulatory targets and/or commercial targets and we may enter into additional business development transactions that require additional capital. Our board of directors also has authorized a share repurchase program to repurchase up to \$500.0 million of shares of our common stock through December 31, 2019. As of March 31, 2018, \$488.8 million remained available to fund repurchases under the share repurchase program.

We expect that cash flows from ORKAMBI, KALYDECO and SYMDEKO, together with our current cash, cash equivalents and marketable securities will be sufficient to fund our operations for at least the next twelve months. The adequacy of our available funds to meet our future operating and capital requirements will depend on many factors, including the amounts of future revenues generated by ORKAMBI, KALYDECO and SYMDEKO and the potential introduction of one or more of our other drug candidates to the market, the level of our business development activities and the number, breadth, cost and prospects of our research and development programs.

### Financing Strategy

We have a \$500.0 million revolving credit facility that we entered into in October 2016. We may repay and reborrow amounts under the revolving credit agreement without penalty. In addition, subject to certain conditions, we may request that the borrowing capacity under this credit agreement be increased by an additional \$300.0 million. We may raise additional capital through public offerings or private placements of our securities or securing new collaborative agreements or other methods of financing. We will continue to manage our capital structure and will consider all financing opportunities, whenever they may occur, that could strengthen our long-term liquidity profile. There can be no assurance that any such financing opportunities will be available on acceptable terms, if at all.

### CONTRACTUAL COMMITMENTS AND OBLIGATIONS

Our commitments and obligations were reported in our Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the Securities and Exchange Commission, or SEC, on February 15, 2018. There have been no material changes from the contractual commitments and obligations previously disclosed in that Annual Report on Form 10-K.

### CRITICAL ACCOUNTING POLICIES AND ESTIMATES

Our discussion and analysis of our financial condition and results of operations is based upon our condensed consolidated financial statements prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make certain estimates and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of revenues and expenses during the reported periods. These items are monitored and analyzed by management for changes in facts and circumstances, and material changes in these estimates could occur in the future. Changes in estimates are reflected in reported results for the period in which the change occurs. We base our estimates on historical experience and various other assumptions that we believe to be reasonable under the circumstances. Actual results may differ from our estimates if past experience or other assumptions do not turn out to be substantially accurate. During the three months ended March 31, 2018, there were no material changes to our critical accounting policies as reported in our Annual Report on Form 10-K for the year ended December 31, 2017, which we filed with the SEC on February 15, 2018, except as set forth below:

#### Product Revenues - Early Access Programs

We began distributing ORKAMBI in France in 2015 through early access programs and are engaged in ongoing pricing discussions regarding the final price for ORKAMBI in France. We did not recognize any revenues from these product sales through December 31, 2017 based on accounting guidance in effect during this time because the price was not fixed or determinable. As of March 31, 2018 and December 31, 2017, the Company's condensed consolidated balance sheet includes \$268.4 million and \$232.4 million, respectively, under the caption "Early access sales accrual" related to amounts accrued in France as payment for shipments of ORKAMBI under the early access programs. We expect to return the difference between the amounts collected based on the invoiced price and the final price for

ORKAMBI in France to the French government.

Upon adopting ASC 606 in the first quarter of 2018, the Company recorded a \$8.3 million cumulative effect adjustment to “Accumulated deficit” primarily related to shipments of ORKAMBI under early access programs in France. The

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Company determined the amount of the adjustment based upon (i) the status of pricing discussions in France upon adoption and (ii) the Company's estimate of the amount of consideration it expects to retain related to ORKAMBI sales in France that occurred on or prior to December 31, 2017 that will not be subject to a significant reversal in amounts recognized. For ORKAMBI sales in France that occurred after December 31, 2017 under the early access programs, the Company has recognized net product revenues based on the estimate of consideration it expects to retain that will not be subject to a significant reversal in amounts recognized. In periods after the first quarter of 2018, if the Company's estimate regarding the amounts it will receive for ORKAMBI supplied pursuant to these early access programs changes, the Company will reflect the effect of the change in estimate in net product revenues in the period in which the change in estimate occurs and will include adjustments to all prior sales of ORKAMBI under the early access programs. Depending on the final price of ORKAMBI and because the current estimate is based on the amount that will not be subject to a significant reversal in amounts recognized this adjustment could be material. For more information regarding the new guidance please see Note B, "Revenue Recognition."

### RECENT ACCOUNTING PRONOUNCEMENTS

For a discussion of recent accounting pronouncements, please refer to Note A, "Basis of Presentation and Accounting Policies—Recent Accounting Pronouncements."

### Item 3. Quantitative and Qualitative Disclosures About Market Risk

As part of our investment portfolio, we own financial instruments that are sensitive to market risks. The investment portfolio is used to preserve our capital until it is required to fund operations, including our research and development activities. None of these market risk-sensitive instruments are held for trading purposes. We do not have derivative financial instruments in our investment portfolio.

#### Interest Rate Risk

We invest our cash in a variety of financial instruments, principally securities issued by the U.S. government and its agencies, investment-grade corporate bonds and commercial paper, and money market funds. These investments are denominated in U.S. Dollars. All of our interest-bearing securities are subject to interest rate risk and could decline in value if interest rates fluctuate. Substantially all of our investment portfolio consists of marketable securities with active secondary or resale markets to help ensure portfolio liquidity, and we have implemented guidelines limiting the term-to-maturity of our investment instruments. Due to the conservative nature of these instruments, we do not believe that we have a material exposure to interest rate risk. If interest rates were to increase or decrease by 1%, the fair value of our investment portfolio would increase or decrease by an immaterial amount.

In October 2016, we entered into a credit agreement. Loans under the credit agreement bear interest, at our option, at either a base rate or a Eurodollar rate, in each case plus an applicable margin. The applicable margin on base rate loans ranges from 0.75% to 1.50% and the applicable margin on Eurodollar loans ranges from 1.75% to 2.50%, in each case, based on our consolidated leverage ratio (as defined in the credit agreement). We do not believe that changes in interest rates related to the credit agreement would have a material effect on our financial statements. As of March 31, 2018, we had no principal or interest outstanding. A portion of our interest expense, net in 2018 will be dependent on whether, and to what extent, we reborrow amounts under the existing facility.

#### Foreign Exchange Market Risk

As a result of our foreign operations, we face exposure to movements in foreign currency exchange rates, primarily the Euro and British Pound against the U.S. Dollar. The current exposures arise primarily from cash, accounts receivable, intercompany receivables and payables, payables and accruals and inventories. Both positive and negative affects to our net revenues from international product sales from movements in foreign currency exchange rates are partially mitigated by the natural, opposite affect that foreign currency exchange rates have on our international operating costs and expenses.

We have a foreign currency management program with the objective of reducing the effect of exchange rate fluctuations on our operating results and forecasted revenues and expenses denominated in foreign currencies. We currently have cash flow hedges for the Euro, British Pound, Canadian Dollar and Australian Dollar related to forecasted product revenues that qualify for hedge accounting treatment under U.S. GAAP. We do not seek hedge accounting treatment for our forward contracts related to monetary assets and liabilities that impact our operating results. As of March 31, 2018, we held foreign



exchange forward contracts with notional amounts totaling \$547.3 million. As of March 31, 2018, our outstanding foreign exchange forward contracts had a net fair value of \$(15.5) million.

Based on our foreign currency exchange rate exposures at March 31, 2018, a hypothetical 10% adverse fluctuation in exchange rates would decrease the fair value of our foreign exchange forward contracts that are designated as cash flow hedges by approximately \$45.8 million at March 31, 2018. The resulting loss on these forward contracts would be offset by the gain on the underlying transactions and therefore would have minimal impact on future anticipated earnings and cash flows. Similarly, adverse fluctuations in exchange rates that would decrease the fair value of our foreign exchange forward contracts that are not designated as hedge instruments would be offset by a positive impact of the underlying monetary assets and liabilities.

#### Item 4. Controls and Procedures

##### Evaluation of Disclosure Controls and Procedures

Our chief executive officer and chief financial officer, after evaluating the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended) as of the end of the period covered by this Quarterly Report on Form 10-Q, have concluded that, based on such evaluation, as of March 31, 2018 our disclosure controls and procedures were effective and designed to provide reasonable assurance that the information required to be disclosed is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. In designing and evaluating our disclosure controls and procedures, our management recognized that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives, and our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

##### Changes in Internal Controls Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Securities Exchange Act of 1934, as amended) occurred during the three months ended March 31, 2018 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

We implemented internal controls to ensure we adequately evaluated our contracts and properly assessed the impact of ASC 606, Revenue from Contracts with Customers, to facilitate its adoption on January 1, 2018. There were no significant changes to our internal control over financial reporting due to the adoption of this standard.

#### PART II. Other Information

##### Item 1. Legal Proceedings

We are not currently subject to any material legal proceedings.

##### Item 1A. Risk Factors

Information regarding risk factors appears in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the SEC on February 15, 2018. There have been no material changes from the risk factors previously disclosed in the Annual Report on Form 10-K.

##### SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q and, in particular, our Management's Discussion and Analysis of Financial Condition and Results of Operations set forth in Part I-Item 2, contain or incorporate a number of forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, including statements regarding:

- our expectations regarding the amount of, timing of and trends with respect to our revenues, costs and expenses and other gains and losses, including those related to CF net product revenues;

our expectations regarding clinical trials, development timelines, timing of our receipt of data from our ongoing and planned clinical trials and regulatory authority filings and submissions for ivacaftor, lumacaftor, tezacaftor, VX-659, VX-445, VX-561, VX-150, VX-128 and VX-210 and the MAA for tezacaftor in combination with ivacaftor; our ability to obtain reimbursement for ORKAMBI in ex-U.S. markets and our ability to otherwise successfully market KALYDECO, ORKAMBI and SYMDEKO or any of our other drug candidates for which we obtain regulatory approval;

our expectations regarding the timing and structure of clinical trials of our drugs and drug candidates, including ivacaftor, lumacaftor, tezacaftor, VX-659, VX-445, VX-561, VX-150, VX-128 and VX-210, and the expected timing of our receipt of data from our ongoing and planned clinical trials;

the data that will be generated by ongoing and planned clinical trials and the ability to use that data to advance compounds, continue development or support regulatory filings;

our beliefs regarding the support provided by clinical trials and preclinical and nonclinical studies of our drug candidates for further investigation, clinical trials or potential use as a treatment;

our plan to continue investing in our research and development programs and our strategy to develop our drug candidates, alone or with third party-collaborators;

the establishment, development and maintenance of collaborative relationships;

potential business development activities;

our post-closing integration of the assets acquired from Concert;

potential fluctuations in foreign currency exchange rates;

our ability to use our research programs to identify and develop new drug candidates to address serious diseases and significant unmet medical needs; and

our liquidity and our expectations regarding the possibility of raising additional capital.

Any or all of our forward-looking statements in this Quarterly Report on Form 10-Q may turn out to be wrong. They can be affected by inaccurate assumptions or by known or unknown risks and uncertainties. Many factors mentioned in this Quarterly Report on Form 10-Q will be important in determining future results. Consequently, no forward-looking statement can be guaranteed. Actual future results may vary materially from expected results. We also provide a cautionary discussion of risks and uncertainties under “Risk Factors” in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the SEC on February 15, 2018. These are factors and uncertainties that we think could cause our actual results to differ materially from expected results. Other factors and uncertainties besides those listed there could also adversely affect us.

Without limiting the foregoing, the words “believes,” “anticipates,” “plans,” “intends,” “expects” and similar expressions are intended to identify forward-looking statements. There are a number of factors and uncertainties that could cause actual events or results to differ materially from those indicated by such forward-looking statements, many of which are beyond our control. In addition, the forward-looking statements contained herein represent our estimate only as of the date of this filing and should not be relied upon as representing our estimate as of any subsequent date. While we may elect to update these forward-looking statements at some point in the future, we specifically disclaim any obligation to do so to reflect actual results, changes in assumptions or changes in other factors affecting such forward-looking statements.

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

### Issuer Repurchases of Equity Securities

The table set forth below shows all repurchases of securities by us during the three months ended March 31, 2018:

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| Period                                | Total Number of Shares Purchased (1) | Average Price Paid per Share | Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs (2) | Approximate Dollar Value of Shares that May Yet be Purchased Under the Plans or Programs (2) |
|---------------------------------------|--------------------------------------|------------------------------|--------------------------------------------------------------------------------------|----------------------------------------------------------------------------------------------|
| January 1, 2018 to January 31, 2018   | 4,445                                | \$0.01                       | —                                                                                    | 500,000,000                                                                                  |
| February 1, 2018 to February 28, 2018 | 22,043                               | \$85.06                      | 11,164                                                                               | 498,125,176                                                                                  |
| March 1, 2018 to March 31, 2018       | 63,886                               | \$146.73                     | 55,920                                                                               | 488,751,049                                                                                  |
| Total                                 | 90,374                               | \$124.47                     | 67,084                                                                               | 488,751,049                                                                                  |

(1) Consists of 67,084 shares repurchased pursuant to our share repurchase program (described in footnote 2 below) at an average price per share of \$167.68 and 23,290 restricted shares repurchased for \$0.01 per share from our employees pursuant to our equity plans. While we have restricted shares that are continuing to vest under our equity plans that are subject to repurchase rights upon termination of service, we have transitioned our equity program to granting restricted stock units. Unvested restricted stock units are forfeited upon termination of service and do not result in an issuer repurchase that would be reflected in the preceding table.

(2) Our Board of Directors has approved a share repurchase program pursuant to which we are authorized to repurchase up to \$500.0 million of our common stock by December 2019, which was announced on January 31, 2018. Under the share repurchase program, we are authorized to purchase shares from time to time through open market or privately negotiated transactions and such purchases may be made pursuant to Rule 10b5-1 plans or other means as determined by our management and in accordance with the requirements of the Securities and Exchange Commission. The approximate dollar value of shares that may yet be repurchased is based solely on shares that may be repurchased under the share repurchase program and excludes any shares that may be repurchased under our employee equity programs.

## Item 6. Exhibits

| Exhibit Number | Exhibit Description                                                                                                                      |
|----------------|------------------------------------------------------------------------------------------------------------------------------------------|
| 31.1           | <u>Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.</u>                                 |
| 31.2           | <u>Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.</u>                                 |
| 32.1           | <u>Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.</u> |
| 101.INS        | XBRL Instance                                                                                                                            |

|         |                                      |
|---------|--------------------------------------|
| 101.SCH | XBRL Taxonomy Extension Schema       |
| 101.CAL | XBRL Taxonomy Extension Calculation  |
| 101.LAB | XBRL Taxonomy Extension Labels       |
| 101.PRE | XBRL Taxonomy Extension Presentation |
| 101.DEF | XBRL Taxonomy Extension Definition   |

**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.

Vertex Pharmaceuticals Incorporated

April 27, 2018 By: /s/ Thomas Graney

Thomas Graney

Senior Vice President and Chief Financial Officer

(principal financial officer and  
duly authorized officer)