

VERTEX PHARMACEUTICALS INC / MA

Form 10-Q

July 28, 2017

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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 JUNE 30, 2017

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 252,118,869

Class

Outstanding at July 21, 2017

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FORM 10-Q  
FOR THE QUARTER ENDED JUNE 30, 2017

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“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.

“Vertex,” “KALYDECO” and “ORKAMBI” 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.

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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 June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Revenues:				
Product revenues, net	\$513,988	\$425,651	\$994,610	\$820,061
Royalty revenues	2,861	5,282	4,412	8,878
Collaborative revenues	27,286	675	259,831	749
Total revenues	544,135	431,608	1,258,853	829,688
Costs and expenses:				
Cost of product revenues	70,535	44,154	116,777	93,943
Royalty expenses	670	1,098	1,416	1,958
Research and development expenses	289,451	271,008	563,014	526,868
Sales, general and administrative expenses	127,249	111,652	240,575	216,866
Restructuring expenses, net	3,523	343	13,522	1,030
Total costs and expenses	491,428	428,255	935,304	840,665
Income (loss) from operations	52,707	3,353	323,549	(10,977 )
Interest expense, net	(14,664 )	(20,155 )	(31,429 )	(40,853 )
Other (expenses) income, net	(2,537 )	(1,219 )	(3,081 )	3,192
Income (loss) before provision for income taxes	35,506	(18,021 )	289,039	(48,638 )
Provision for income taxes	4,337	18,130	8,322	23,615
Net income (loss)	31,169	(36,151 )	280,717	(72,253 )
Income attributable to noncontrolling interest	(13,173 )	(28,374 )	(14,965 )	(33,903 )
Net income (loss) attributable to Vertex	\$17,996	\$(64,525 )	\$265,752	\$(106,156)

Amounts per share attributable to Vertex common shareholders:

Net income (loss):				
Basic	\$0.07	\$(0.26 )	\$1.08	\$(0.43 )
Diluted	\$0.07	\$(0.26 )	\$1.06	\$(0.43 )
Shares used in per share calculations:				
Basic	247,521	244,482	246,782	244,124
Diluted	251,635	244,482	250,199	244,124

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 (Loss)

(unaudited)

(in thousands)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Net income (loss)	\$31,169	\$(36,151)	\$280,717	\$(72,253)
Changes in other comprehensive income (loss):				
Unrealized holding (losses) gains on marketable securities, net of tax of \$1.0 million, zero, zero and zero, respectively	(17,281)	(29)	(13,747)	200
Unrealized (losses) gains on foreign currency forward contracts, net of tax of \$1.1 million, \$0.2 million, \$2.0 million and \$(0.6) million, respectively	(15,245)	4,999	(21,926)	(213)
Foreign currency translation adjustment	(5,252)	(3,461)	(7,253)	(5,201)
Total changes in other comprehensive (loss) income	(37,778)	1,509	(42,926)	(5,214)
Comprehensive (loss) income	(6,609)	(34,642)	237,791	(77,467)
Comprehensive income attributable to noncontrolling interest	(13,173)	(28,374)	(14,965)	(33,903)
Comprehensive (loss) income attributable to Vertex	\$(19,782)	\$(63,016)	\$222,826	\$(111,370)

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)

	June 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	\$1,223,130	\$1,183,945
Marketable securities, available for sale	445,520	250,612
Restricted cash and cash equivalents (VIE)	64,628	47,762
Accounts receivable, net	247,949	201,083
Inventories	92,263	77,604
Prepaid expenses and other current assets	107,082	70,534
Total current assets	2,180,572	1,831,540
Property and equipment, net	740,103	698,362
Intangible assets	284,340	284,340
Goodwill	50,384	50,384
Cost method investments	20,252	20,276
Other assets	9,943	11,885
Total assets	\$3,285,594	\$2,896,787
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$75,941	\$61,451
Accrued expenses	345,062	315,249
Deferred revenues, current portion	7,277	6,005
Accrued restructuring expenses, current portion	6,491	6,047
Capital lease obligations, current portion	18,179	19,426
Customer deposits	147,686	73,416
Credit facility	—	300,000
Other liabilities, current portion	24,770	10,943
Total current liabilities	625,406	792,537
Deferred revenues, excluding current portion	4,161	6,632
Accrued restructuring expenses, excluding current portion	527	1,907
Capital lease obligations, excluding current portion	25,346	34,976
Deferred tax liability	136,649	134,063
Construction financing lease obligation, excluding current portion	525,019	486,359
Advance from collaborator	76,034	73,423
Other liabilities, excluding current portion	25,221	28,699
Total liabilities	1,418,363	1,558,596
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; 250,769,906 and 248,300,517 shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively	2,479	2,450
Additional paid-in capital	6,808,002	6,506,795
Accumulated other comprehensive (loss) income	(21,753)	21,173

Accumulated deficit	(5,117,455 )	(5,373,836 )
Total Vertex shareholders' equity	1,671,273	1,156,582
Noncontrolling interest	195,958	181,609
Total shareholders' equity	1,867,231	1,338,191
Total liabilities and shareholders' equity	\$3,285,594	\$2,896,787

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 Shares	Amount	Additional Paid-in Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Vertex Shareholders' Equity	Noncontrolling Interest	Total Shareholders' Equity
Balance at December 31, 2015	246,307	\$ 2,427	\$ 6,197,500	\$ 1,824	\$(5,261,784)	\$ 939,967	\$ 153,661	\$ 1,093,628
Other comprehensive loss, net of tax	—	—	—	(5,214 )	—	(5,214 )	—	(5,214 )
Net loss	—	—	—	—	(106,156 )	(106,156 )	33,903	(72,253 )
Issuance of common stock under benefit plans	1,397	13	33,557	—	—	33,570	—	33,570
Stock-based compensation expense	—	—	119,187	—	—	119,187	(73 )	119,114
Balance at June 30, 2016	247,704	\$ 2,440	\$ 6,350,244	\$ (3,390 )	\$(5,367,940)	\$ 981,354	\$ 187,491	\$ 1,168,845
Balance at December 31, 2016	248,301	\$ 2,450	\$ 6,506,795	\$ 21,173	\$(5,373,836)	\$ 1,156,582	\$ 181,609	\$ 1,338,191
Cumulative effect adjustment for adoption of new accounting guidance	—	—	9,371	—	(9,371 )	—	—	—
Other comprehensive loss, net of tax	—	—	—	(42,926 )	—	(42,926 )	—	(42,926 )
Net income	—	—	—	—	265,752	265,752	14,965	280,717
Issuance of common stock under benefit plans	2,469	29	147,979	—	—	148,008	—	148,008
Stock-based compensation expense	—	—	143,857	—	—	143,857	—	143,857
Other	—	—	—	—	—	—	(616 )	(616 )
Balance at June 30, 2017	250,770	\$ 2,479	\$ 6,808,002	\$ (21,753 )	\$(5,117,455)	\$ 1,671,273	\$ 195,958	\$ 1,867,231

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)

	Six Months Ended June 30,	
	2017	2016
Cash flows from operating activities:		
Net income (loss)	\$280,717	\$(72,253 )
Adjustments to reconcile net income (loss) to net cash provided by operating activities:		
Stock-based compensation expense	141,564	117,414
Depreciation and amortization expense	29,740	31,378
Write-downs of inventories to net realizable value	9,479	—
Deferred income taxes	4,626	22,858
Impairment of property and equipment	1,946	—
Other non-cash items, net	(4,834 )	3,436
Changes in operating assets and liabilities:		
Accounts receivable, net	(41,450 )	(12,954 )
Inventories	(22,028 )	(7,779 )
Prepaid expenses and other assets	(47,848 )	(7,971 )
Accounts payable	14,047	(23,821 )
Accrued expenses and other liabilities	83,643	(14,562 )
Accrued restructuring expense	(1,058 )	(2,892 )
Deferred revenues	(1,199 )	(7,131 )
Net cash provided by operating activities	447,345	25,723
Cash flows from investing activities:		
Purchases of marketable securities	(377,667 )	(470,077 )
Maturities of marketable securities	168,882	332,316
Expenditures for property and equipment	(28,866 )	(27,892 )
(Increase) decrease in restricted cash and cash equivalents (VIE)	(16,865 )	8,397
Investment in CRISPR Series B preferred stock	—	(3,075 )
Decrease (increase) in other assets	388	(159 )
Net cash used in investing activities	(254,128 )	(160,490 )
Cash flows from financing activities:		
Issuances of common stock under benefit plans	147,887	33,702
Payments on revolving credit facility	(300,000 )	—
Advance from collaborator	7,500	—
Payments on capital lease obligations	(10,637 )	(7,538 )
Payments on construction financing lease obligation	(238 )	(209 )
Repayments of advanced funding	(2,044 )	—
Net cash (used in) provided by financing activities	(157,532 )	25,955
Effect of changes in exchange rates on cash	3,500	(90 )
Net increase (decrease) in cash and cash equivalents	39,185	(108,902 )
Cash and cash equivalents—beginning of period	1,183,945	714,768
Cash and cash equivalents—end of period	\$1,223,130	\$605,866
Supplemental disclosure of cash flow information:		
Cash paid for interest	\$35,003	\$41,325
Cash paid for income taxes	\$2,218	\$1,237

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Capitalization of costs related to construction financing lease obligation	\$38,930	\$—
Issuances of common stock from employee benefit plans receivable	\$188	\$161

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

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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.

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 June 30, 2017 and 2016.

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, 2016, which are contained in the Company’s Annual Report on Form 10-K for the year ended December 31, 2016 that was filed with the Securities and Exchange Commission (the “SEC”) on February 23, 2017 (the “2016 Annual Report on Form 10-K”).

Use of Estimates and Summary of Significant Accounting Policies

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, restructuring expense, the fair value of intangible assets, goodwill, contingent consideration, noncontrolling interest, the consolidation of VIEs, leases, the fair value of cash flow hedges 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.

The Company’s significant accounting policies are described in Note A, “Nature of Business and Accounting Policies,” in the 2016 Annual Report on Form 10-K.

Recent Accounting Pronouncements

In 2014, the Financial Accounting Standards Board (“FASB”) issued new guidance applicable to revenue recognition that will be effective January 1, 2018. Early adoption was permitted for the year-ending December 31, 2017. The new guidance applies a more principles based approach to recognizing revenue. Under the new guidance, 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 new guidance must be adopted using either a modified retrospective approach or a full retrospective approach for all periods presented. Under the modified retrospective method, the cumulative effect of applying the standard would be recognized at the date of initial application within retained earnings. Under the full retrospective approach, the standard would be applied to each prior reporting period presented. Upon adoption, the Company will use the modified retrospective method. The Company continued its evaluation of the new guidance and the effect of adoption on the condensed consolidated financial statements. The Company’s project team progressed its

review of existing customer contracts and current accounting policies to identify and assess the potential differences that would result from applying the requirements of the new standard. Based on the Company's assessment performed to date, the new guidance could impact the Company's accounting for product shipments

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VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

to certain countries through early access programs, including the French early access programs, whereby the associated product has received regulatory approval but the reimbursement rate has not been finalized, and could impact the Company's accounting for certain reimbursement agreements that the Company plans to negotiate in the second half of 2017. The Company is also in the process of implementing appropriate changes to its controls to support revenue recognition and additional revenue-related disclosures under the new standard.

In 2016, the FASB issued amended guidance applicable to share-based compensation to employees that simplifies the accounting for employee share-based payment transactions, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. The amended guidance became effective for the Company during the first quarter of 2017. The amended guidance eliminates the requirement that excess tax benefits be realized as a reduction in current taxes payable before the associated tax benefit can be recognized as an increase in additional paid-in capital. This created approximately \$410.8 million of deferred tax asset ("DTA") relating to federal and state net operating losses ("NOLs") that are fully reserved by an equal increase in valuation allowance. The Company recorded DTAs of approximately \$404.7 million relating to Federal NOLs and approximately \$6.1 million relating to State NOLs, both of which are offset by a full valuation allowance. Upon adoption, the Company also elected to change its accounting policy to account for forfeitures of options and awards as they occur. The change was applied on a modified retrospective basis with a cumulative effect adjustment to the Company's accumulated deficit of \$9.4 million, which increased the accumulated deficit as of January 1, 2017. This change also resulted in an increase to the DTA of \$3.4 million, which is offset by a full valuation allowance. As a result, there was no cumulative-effect adjustment to accumulated deficit. The provisions related to the recognition of excess tax benefits in the income statement and classification in the statement of cash flows were adopted prospectively, and as such, the prior periods were not retrospectively adjusted.

In 2016, the FASB issued 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 the investee) are to be measured at fair value with changes in fair value recognized in net income. However, an entity has the option to either measure equity investments without readily determinable fair values at fair value or at cost adjusted for changes in observable prices minus impairment. Changes in measurement under either alternative will be recognized in net income. The amended guidance is effective for the year-ending December 31, 2018. Early adoption is permitted. The Company expects the implementation of this standard to have an impact on its consolidated financial statements and related disclosures, as the Company held publicly traded equity investments as of June 30, 2017 as well as equity investments accounted for under the cost method. A cumulative-effect adjustment to the balance sheet will be recorded as of the beginning of the fiscal year of adoption. The implementation of this amended guidance is expected to increase volatility in net income as the volatility currently recorded in other comprehensive income related to changes in the fair market value of available-for-sale equity investments will be reflected in net income after adoption.

In 2016, the FASB issued amended guidance applicable to leases that will be effective for the year ending December 31, 2019. Early adoption is permitted. This guidance requires entities to recognize assets and liabilities for leases with lease terms of more than 12 months on the balance sheet. The Company is in the process of evaluating this guidance and determining the expected effect on its condensed consolidated financial statements.

In 2016, the FASB issued amended guidance related to intra-entity transfers other than inventory. This guidance removes the current exception in GAAP prohibiting entities from recognizing current and deferred income tax expenses or benefits related to transfer of assets, other than inventory, within the consolidated entity. The current 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 is effective for the year ending December 31, 2018. Early adoption is permitted. The Company is in the process of evaluating this guidance and determining the expected

effect on its condensed consolidated financial statements.

In 2017, the FASB issued amended guidance related to business combinations. The amended guidance clarifies the definition of a business with the objective of adding guidance to assist entities with evaluating whether transactions should be accounted for as acquisitions (or disposals) of assets or businesses. The new accounting guidance is effective for annual periods beginning after December 15, 2017, including interim periods within those periods. Early adoption is permitted. The Company early adopted this new guidance as of January 1, 2017 and will apply this new guidance to future acquisitions.

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(unaudited)

In 2017, the FASB issued amended guidance related to measurements of goodwill. The amended guidance eliminates a step from the goodwill impairment test. Under the amended guidance, an entity should perform its annual or interim goodwill impairment test by comparing the fair value of a reporting unit with its carrying amount. An entity would recognize 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 amended guidance is effective for the year-ending December 31, 2020. Early adoption is permitted. The Company does not expect a significant effect on its condensed consolidated financial statements upon adoption of this new guidance.

In 2017, the FASB issued amended guidance related to the scope of stock option modification accounting, to reduce diversity in practice and provide clarity regarding existing guidance. The new accounting guidance is effective for annual periods beginning after December 15, 2017, including interim periods within those periods. Early adoption is permitted. The Company does not expect the adoption of this guidance to have a material effect on its condensed consolidated financial statements and related disclosures.

For a discussion of other recent accounting pronouncements please refer to Note A, "Nature of Business and Accounting Policies—Recent Accounting Pronouncements," in the 2016 Annual Report on Form 10-K.

**B. Product Revenues, Net**

The Company sells its products principally to a limited number of specialty pharmacy providers in North America as well as government-owned and supported customers in international markets (collectively, its "Customers"). The Company's Customers in North America subsequently resell the products to patients and health care providers. The Company recognizes net revenues from product sales upon delivery to the Customer as long as (i) there is persuasive evidence that an arrangement exists between the Company and the Customer, (ii) collectibility is reasonably assured and (iii) the price is fixed or determinable.

In order to conclude that the price is fixed or determinable, the Company must be able to (i) calculate its gross product revenues from sales to Customers and (ii) reasonably estimate its net product revenues upon delivery to its Customers' locations. The Company calculates gross product revenues based on the price that the Company charges its Customers. The Company estimates its net product revenues by deducting from its gross product revenues (a) trade allowances, such as invoice discounts for prompt payment and Customer fees, (b) estimated government and private payor rebates, chargebacks and discounts, (c) estimated reserves for expected product returns and (d) estimated costs of co-pay assistance programs for patients, as well as other incentives for certain indirect customers.

The Company makes significant estimates and judgments that materially affect the Company's recognition of net product revenues. In certain instances, the Company may be unable to reasonably conclude that the price is fixed or determinable at the time of delivery, in which case it defers the recognition of revenues. Once the Company is able to determine that the price is fixed or determinable, it recognizes the revenues associated with the units in which revenue recognition was deferred. ORKAMBI net product revenues do not include any revenues from product sales in France. The Company began distributing ORKAMBI through early access programs in France during the fourth quarter of 2015. The Company's condensed consolidated balance sheet includes \$147.7 million collected as of June 30, 2017 in France related to ORKAMBI that is classified as Customer deposits. The Company currently expects that revenues from these early access programs and deferred expenses associated with these revenues will be recognized in the period that a formal reimbursement agreement in France is reached based on the terms of such agreement.





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## VERTEX PHARMACEUTICALS INCORPORATED

## Notes to Condensed Consolidated Financial Statements

(unaudited)

The following table summarizes activity in each of the product revenue allowance and reserve categories for the six months ended June 30, 2017:

	Trade Allowances and Discounts	Rebates, Chargebacks	Product Returns	Other Incentives	Total
	(in thousands)				
Balance at December 31, 2016	\$2,568	\$ 81,927	\$3,492	\$ 1,214	\$89,201
Provision related to current period sales	11,941	69,669	1,777	9,224	92,611
Adjustments related to prior period sales	(194 )	(3,268 )	(48 )	(145 )	(3,655 )
Credits/payments made	(11,683)	(58,121 )	(631 )	(6,966 )	(77,401 )
Balance at June 30, 2017	\$2,632	\$ 90,207	\$4,590	\$ 3,327	\$100,756

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VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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 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 (i) KALYDECO (ivacaftor) and ORKAMBI (lumacaftor in combination with ivacaftor), which are the Company’s current products and (ii) tezacaftor in combination with ivacaftor. For combination products, such as ORKAMBI, sales will be allocated equally to each of the active pharmaceutical ingredients in the combination product.

In the first quarter of 2016, CFFT earned a commercial milestone payment of \$13.9 million from the Company upon achievement of certain sales levels of lumacaftor. There are no additional 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 program award of \$75.0 million and agreed to provide development funding to the Company of up to \$6.0 million annually. The program award plus any future development funding represent a form of financing pursuant to Accounting Standards Codification (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. The liability is reduced over the estimated royalty term of the agreement. Reductions in the liability are reflected as an offset to cost of product revenues and as interest expense.

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.

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 converted into preferred stock in January 2016. The Company expensed \$75.0 million to research and development, and the \$30.0 million investment was recorded at cost and was classified as a long-term asset on the Company’s condensed consolidated balance sheets. In the second quarter of 2016, the Company made an additional preferred stock investment in CRISPR of approximately \$3.1 million. In connection with CRISPR’s initial public offering in October 2016, the Company purchased \$10 million of common shares at public offering price and the Company’s preferred stock investments in CRISPR converted into common shares. As of June 30, 2017, the Company recorded the CRISPR common shares it holds at fair value and included the \$51.0 million fair value of the common shares in its marketable securities and the 7.8 million unrecognized gain related to these common shares in accumulated other comprehensive income (loss) on the condensed consolidated balance sheet.

The Company will fund all of the discovery activities conducted pursuant to the CRISPR Agreement. For potential hemoglobinopathy treatments, including treatments for sickle cell disease, the Company and CRISPR will share

equally all research and development costs and worldwide revenues. For other targets that the Company elects to license, the Company would lead all development and global commercialization activities. For each of up to six targets that the Company elects to

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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.

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.

Merck KGaA

On January 10, 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 up-front payment from Merck KGaA to the Company of \$230.0 million. During the first quarter of 2017, the Company received \$193.6 million of the up-front payment and the remaining \$36.4 million was remitted to the German tax authorities. Pursuant to a tax treaty between the United States and Germany, the Company filed a refund application for the tax withholding and expects to receive the refund in the second half of 2017. The income tax receivable is included in Prepaid expenses and other current assets at June 30, 2017. In addition to the up-front 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. The Company concluded that the license has 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 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. Based on this analysis, the Company recognized approximately \$231.7 million in collaborative revenues related to the up-front payment upon delivery of the license and to the research and development and transition activities provided during the first quarter of 2017. During the three and six months ended June 30, 2017, the Company recorded the reimbursement for the research and development and transition activities of \$6.1 million and \$7.6 million, respectively, as revenue in the Company's consolidated statements of operations primarily due to the fact that the Company is the primary obligor in the arrangement. The Company is providing research and development and transition activities and will recognize the revenues and associated expenses as the services are provided.

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.

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Variable Interest Entities

The Company has entered into several agreements pursuant to which it has licensed rights to certain drug candidates from third-party collaborators, which has resulted 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 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 estimate 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 are reflected in the Company's financial statements as consolidated VIEs:

Parion Sciences, Inc.

In June 2015, the Company entered into a strategic collaboration and license agreement (the "Parion Agreement") with Parion Sciences, Inc. ("Parion"). Pursuant to the agreement, the Company is collaborating 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 leading development activities for VX-371 and VX-551 and 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. Parion received an \$80.0 million up-front payment and has the potential to receive up to an additional (i) \$490.0 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 has 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.

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.

The Company determined that it has a variable interest in Parion via the Parion Agreement, and that the variable interest represents a variable interest in Parion as a whole since the fair value of the ENaC inhibitors represents more than half of the total fair value of Parion's assets. The Company also concluded that it is the primary beneficiary as it has the power to direct the activities that most significantly affect the economic performance of Parion and it has 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 on June 4, 2015. However, the Company's interests in Parion are limited to those accorded to the Company in the Parion Agreement.



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While there was a transfer of \$80.0 million to Parion, the cash remained within the Company's condensed consolidated balance sheet since Parion is part of the consolidated entity. The cash received, net of any cash spend by Parion, is classified as restricted cash and cash equivalents (VIE) within the condensed consolidated balance sheet as it is attributed to the noncontrolling interest holders of Parion. When determining the valuation of goodwill, the fair value of consideration for the license is zero since there was no consideration transferred outside the condensed consolidated financial statements. The Company recorded \$255.3 million of intangible assets on the Company's condensed consolidated balance sheet for Parion's in-process research and development assets. These in-process research and development assets relate to Parion's pulmonary ENaC platform, including the intellectual property related to VX-371 and VX-551, that are licensed by Parion to the Company. The Company also recorded the fair value of the net assets attributable to noncontrolling interest of \$164.3 million, deferred tax liability of \$91.0 million resulting from a basis difference in the intangible assets and certain other net liabilities held by Parion of \$10.5 million. The difference between the fair values of the consideration and noncontrolling interest and the fair value of Parion's net assets was recorded as goodwill.

In the second quarter of 2017, Parion signed a license agreement with an affiliate of Shire plc related to the development of a drug candidate for the potential treatment of dry eye disease. The Company evaluated the license agreement entered into by Parion as a reconsideration event to determine whether it should continue to consolidate Parion as a variable interest entity into its condensed consolidated financial statements. The Company determined that there was no substantive change in the design of Parion subsequent to Parion's agreement with Shire. Additionally, the Company concluded that it is appropriate to continue to consolidate the financial results of Parion because it continues to have (i) the power to direct the activities that most significantly affect the economic performance of Parion and (ii) the obligation to absorb losses and right to receive benefits that potentially could be significant to Parion. Based on the consolidation of Parion's financial statements, in the three and six months ended June 30, 2017, the Company recognized (i) \$20.0 million of collaborative revenues and (ii) a tax provision of \$7.4 million, both of which were attributable to noncontrolling interest related to an upfront payment that Parion received from Shire in the second quarter of 2017. The Company has no interest in Parion's license agreement with Shire, including the economic benefits and/or obligations derived therefrom.

BioAxone Biosciences, Inc.

In October 2014, the Company entered into a license and collaboration agreement (the "BioAxone Agreement") with BioAxone Biosciences, Inc. ("BioAxone"), which resulted in the consolidation of BioAxone as a VIE beginning on October 1, 2014. The Company paid BioAxone initial payments of \$10.0 million in the fourth quarter of 2014. BioAxone has the potential to receive up to \$90.0 million in milestones and fees, including development, regulatory and milestone payments and a license continuation fee. In addition, BioAxone would receive royalties and commercial milestones on future net product sales of VX-210, if any. 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 BioAxone. The Company holds an option to purchase BioAxone at a predetermined price. The option expires on the earliest of (a) the day the FDA accepts the Biologics License Application submission for VX-210, (b) the day the Company elects to continue the license instead of exercising the option to purchase BioAxone and (c) March 15, 2018, subject to the Company's option to extend this date by one year.

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## Aggregate VIE Financial Information

An aggregate summary of net income attributable to noncontrolling interest related to the Company's VIEs for the three and six months ended June 30, 2017 and 2016 is as follows:

	Three Months Ended June 30, 2017		Six Months Ended June 30, 2017	
	2016	2016	2016	2016
	(in thousands)			
(Income) loss attributable to noncontrolling interest before provision for income taxes and changes in fair value of contingent payments	\$(18,045)	\$2,835	\$(16,498)	\$3,674
Provision for income taxes	8,132	17,511	8,523	20,573
Increase in fair value of contingent payments	(3,260 )	(48,720 )	(6,990 )	(58,150 )
Net income attributable to noncontrolling interest	\$(13,173)	\$(28,374)	\$(14,965)	\$(33,903)

The increases in the noncontrolling interest holders' claim to net assets with respect to the fair value of the contingent payments in the three and six months ended June 30, 2017 were primarily due to changes in market interest rates and the time value of money. The increases in the fair value of the contingent milestone and royalty payments in the three and six months ended June 30, 2016 were primarily due to a Phase 2 clinical trial of VX-371, a compound being developed pursuant to the Parion Agreement, achieving its primary safety endpoint in the second quarter of 2016. During the three and six months ended June 30, 2017 and 2016, the increases in the fair value of the contingent payments related to the Company's VIEs was as follows:

	Three Months Ended June 30, 2017		Six Months Ended June 30, 2017	
	2016	2016	2016	2016
	(in thousands)			
Parion	\$3,260	\$48,400	\$6,090	\$57,400
BioAxone	—	320	900	750

The fair value of the contingent payments related to the Parion Agreement and the BioAxone Agreement as of the dates set forth in the table:

	June 30, 2017	December 31, 2016
	(in thousands)	
Parion	\$244,890	\$238,800
BioAxone	18,900	18,000

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The following table summarizes items related to the Company's VIEs included in the Company's condensed consolidated balance sheets as of the dates set forth in the table:

	June 30, December	
	2017	31, 2016
	(in thousands)	
Restricted cash and cash equivalents (VIE)	\$64,628	\$ 47,762
Prepaid expenses and other current assets	1,198	6,812
Intangible assets	284,340	284,340
Goodwill	19,391	19,391
Other assets	752	399
Accounts payable	702	415
Accrued expenses	4,118	1,330
Other liabilities, current portion	1,610	2,137
Deferred tax liability	134,305	131,446
Other liabilities, excluding current portion	300	300
Noncontrolling interest	195,958	181,609

The Company has recorded the VIEs' cash and cash equivalents as restricted cash and cash equivalents (VIE) because (i) the Company does not have any interest in or control over the VIEs' cash and cash equivalents and (ii) the Company's agreements with each VIE do not provide for the VIEs' cash and cash equivalents to be used for the development of the assets that the Company licensed from the applicable VIE. Assets recorded as a result of consolidating the Company's VIEs' financial condition into the Company's balance sheet 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 July 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 in the third quarter of 2016, the Company made an upfront payment to Moderna of \$20.0 million and a \$20.0 million cost-method investment in Moderna pursuant to a convertible promissory note that converted into preferred stock in August 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.

Under the terms of the Moderna Agreement, Moderna will lead discovery efforts and the Company will lead 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 advanced notice to Moderna, with the required length of notice dependent on whether any product developed under the Moderna Agreement has received marketing approval. The 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.

The Company evaluates the carrying value of its \$20.0 million cost-method investment in Moderna, which is not a publicly traded company, for impairment on a quarterly basis and has not recorded any adjustments to the carrying value of its investment to date.

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Janssen Pharmaceuticals, Inc.

In June 2014, the Company entered into an agreement (the “Janssen Agreement”) with Janssen Pharmaceuticals, Inc. (“Janssen Inc.”), which was amended in October 2014 to clarify certain roles and responsibilities of the parties.

Pursuant to the Janssen Agreement, Janssen Inc. has an exclusive worldwide license to develop and commercialize certain drug candidates for the treatment of influenza, including JNJ-3872 (formerly VX-787). The Company received non-refundable payments of \$35.0 million from Janssen Inc. in 2014, which were recorded as collaborative revenue. The Company has the potential to receive development, regulatory and commercial milestone payments as well as royalties on future product sales, if any. Janssen Inc. may terminate the Janssen Agreement, subject to certain exceptions, upon six months’ notice.

Janssen Inc. is responsible for costs related to the development and commercialization of the compounds. During the three and six months ended June 30, 2017 the Company recorded reimbursement for these development activities of \$0.3 million and \$1.8 million, respectively. During the three and six months ended June 30, 2016 the Company recorded reimbursement for these development activities of \$4.3 million and \$7.8 million, respectively. The reimbursements are recorded as a reduction to development expense in the Company’s condensed consolidated statements of operations primarily due to the fact that Janssen Inc. directs the activities and selects the suppliers associated with these activities.

Acquisition

Concert Pharmaceuticals

In July 2017, the Company acquired certain CF assets including CTP-656 from Concert Pharmaceuticals Inc. (“Concert”) pursuant to an asset purchase agreement that was entered into in March 2017 (the “Concert Agreement”). CTP-656 is an investigational CFTR potentiator that has the potential to be used as part of future once-daily combination regimens of CFTR modulators that treat the underlying cause of CF. As part of the Concert Agreement, Vertex paid Concert \$160 million in cash for all worldwide development and commercialization rights to CTP-656. If CTP-656 is approved as part of a combination regimen to treat CF, Concert could receive up to an additional \$90 million in milestones based on regulatory approval in the U.S. and reimbursement in the UK, Germany or France. There was no accounting impact relating to this agreement during the three and six months ended June 30, 2017. In the third quarter of 2017, the Company expects to record the \$160 million payment as a research and development expense.

D. Earnings Per Share

Basic net income (loss) per share attributable to Vertex common shareholders is based upon the weighted-average number of common shares outstanding during the period, excluding restricted stock and restricted stock units that have been issued but are not yet vested. Diluted net income (loss) 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 (loss) per share for the periods ended:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
	(in thousands, except per share amounts)			
Basic net income (loss) attributable to Vertex per common share calculation:				
Net income (loss) attributable to Vertex common shareholders	\$17,996	\$(64,525)	\$265,752	\$(106,156)
Less: Undistributed earnings allocated to participating securities	(23 )	—	(387 )	—
Net income (loss) attributable to Vertex common shareholders—basic	\$17,973	\$(64,525)	\$265,365	\$(106,156)
Basic weighted-average common shares outstanding	247,521	244,482	246,782	244,124
Basic net income (loss) attributable to Vertex per common share	\$0.07	\$(0.26 )	\$1.08	\$(0.43 )
Diluted net income (loss) attributable to Vertex per common share calculation:				
Net income (loss) attributable to Vertex common shareholders	\$17,996	\$(64,525)	\$265,752	\$(106,156)
Less: Undistributed earnings allocated to participating securities	(23 )	—	(382 )	—
Net income (loss) attributable to Vertex common shareholders—diluted	\$17,973	\$(64,525)	\$265,370	\$(106,156)
Weighted-average shares used to compute basic net income (loss) per common share	247,521	244,482	246,782	244,124
Effect of potentially dilutive securities:				
Stock options	2,787	—	2,407	—
Restricted stock and restricted stock units	1,264	—	958	—
Other	63	—	52	—
Weighted-average shares used to compute diluted net income (loss) per common share	251,635	244,482	250,199	244,124
Diluted net income (loss) attributable to Vertex per common share	\$0.07	\$(0.26 )	\$1.06	\$(0.43 )
The Company did not include the securities in the following table in the computation of the dilutive net income (loss) per share attributable to Vertex common shareholders calculations because the effect would have been anti-dilutive during each period:				

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
	(in thousands)			
Stock options	3,112	12,231	7,065	12,231
Unvested restricted stock and restricted stock units	6	3,506	32	3,506

## 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 June 30, 2017, the Company's investments were primarily in money market funds, corporate equity securities, corporate debt securities and commercial paper.

As of June 30, 2017, all of the Company's financial assets 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, corporate debt securities, commercial paper and corporate equity securities. The Company's financial assets valued based on Level 2 inputs consisted of corporate debt securities and commercial paper, which consisted of investments in highly-rated investment-grade corporations.



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

	Fair Value Measurements as of June 30, 2017			
	Total	Fair Value Hierarchy		
	(in thousands)	Level 1	Level 2	Level 3
Financial instruments carried at fair value (asset position):				
Cash equivalents:				
Money market funds	\$318,411	\$318,411	\$—	\$—
Commercial paper	5,996	—	5,996	—
Marketable securities:				
Corporate equity securities	51,049	51,049	—	—
Corporate debt securities	291,124	—	291,124	—
Commercial paper	103,347	—	103,347	—
Prepaid and other current assets:				
Foreign currency forward contracts	985	—	985	—
Total financial assets	\$770,912	\$369,460	\$401,452	\$—
Financial instruments carried at fair value (liability position):				
Other liabilities, current portion:				
Foreign currency forward contracts	\$(8,067 )	\$—	\$(8,067 )	\$—
Other liabilities, excluding current portion:				
Foreign currency forward contracts	(1,435 )	—	(1,435 )	—
Total financial liabilities	\$(9,502 )	\$—	\$(9,502 )	\$—
	Fair Value Measurements as of December 31, 2016			
	Total	Fair Value Hierarchy		
	(in thousands)	Level 1	Level 2	Level 3
Financial instruments carried at fair value (asset position):				
Cash equivalents:				
Money market funds	\$280,560	\$280,560	\$—	\$—
Marketable securities:				
Government-sponsored enterprise securities	15,508	15,508	—	—
Corporate equity securities	64,560	64,560	—	—
Commercial paper	59,404	—	59,404	—
Corporate debt securities	111,140	—	111,140	—
Prepaid and other current assets:				
Foreign currency forward contracts	14,407	—	14,407	—
Other assets:				
Foreign currency forward contracts	1,186	\$—	1,186	\$—
Total financial assets	\$546,765	\$360,628	\$186,137	\$—
Financial instruments carried at fair value (liability position):				
Other liabilities, current portion:				
Foreign currency forward contracts	\$(144 )	\$—	\$(144 )	\$—
Total financial liabilities	\$(144 )	\$—	\$(144 )	\$—

The Company's VIEs invested in cash equivalents consisting of money market funds of \$62.6 million as of June 30, 2017, 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 VIEs includes the fair value of the contingent payments, which consist of milestone, royalty

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and option payments, which are valued based on Level 3 inputs. Please refer to Note C, "Collaborative Arrangements," for further information.

## F. Marketable Securities

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

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
As of June 30, 2017				
Cash and cash equivalents:				
Cash and money market funds	\$1,217,134	\$ —	\$ —	\$1,217,134
Commercial paper	5,996	—	—	5,996
Total cash and cash equivalents	\$1,223,130	\$ —	\$ —	\$1,223,130
Marketable securities:				
Corporate equity securities	43,213	7,836	—	51,049
Commercial paper (matures within 1 year)	103,386	1	(40)	103,347
Corporate debt securities (matures within 1 year)	218,216	4	(143)	218,077
Corporate debt securities (matures after 1 year)	73,115	2	(70)	73,047
Total marketable securities	\$437,930	\$ 7,843	\$ (253)	\$445,520
Total cash, cash equivalents and marketable securities	\$1,661,060	\$ 7,843	\$ (253)	\$1,668,650

## As of December 31, 2016

## Cash and cash equivalents:

Cash and money market funds	\$1,183,945	\$ —	\$ —	\$1,183,945
Total cash and cash equivalents	\$1,183,945	\$ —	\$ —	\$1,183,945

## Marketable securities:

Government-sponsored enterprise securities (matures within 1 year)	\$15,506	\$ 2	\$ —	\$15,508
Corporate equity securities	43,213	21,347	—	64,560
Commercial paper (matures within 1 year)	59,331	73	—	59,404
Corporate debt securities (matures within 1 year)	111,225	—	(85)	111,140
Total marketable securities	\$229,275	\$ 21,422	\$ (85)	\$250,612
Total cash, cash equivalents and marketable securities	\$1,413,220	\$ 21,422	\$ (85)	\$1,434,557

The Company has a limited number of marketable securities in insignificant loss positions as of June 30, 2017, which the Company does not intend to sell and has concluded it will not be required to sell before recovery of the amortized costs of the investment at maturity. There were no charges recorded for other-than-temporary declines in fair value of marketable securities nor gross realized gains or losses recognized in the three and six months ended June 30, 2017 and 2016.

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## Notes to Condensed Consolidated Financial Statements

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G. Accumulated Other Comprehensive  
Income (Loss)

A summary of the Company's changes in accumulated other comprehensive income (loss) by component is shown below:

	Foreign Currency Translation Adjustment	Unrealized Holding Gains (Losses) on Marketable Securities, Net of Tax	Unrealized Gains (Losses) on Foreign Currency Forward Contracts, Net of Tax	Total
	(in thousands)			
Balance at December 31, 2016	\$(7,862 )	\$ 17,521	\$ 11,514	\$ 21,173
Other comprehensive loss before reclassifications	(7,253 )	(13,747 )	(17,215 )	(38,215 )
Amounts reclassified from accumulated other comprehensive loss	—	—	(4,711 )	(4,711 )
Net current period other comprehensive (loss) income	\$(7,253 )	\$ (13,747 )	\$ (21,926 )	\$(42,926)
Balance at June 30, 2017	\$(15,115)	\$ 3,774	\$(10,412 )	\$(21,753)
	(in thousands)			
	Foreign Currency Translation Adjustment	Unrealized Holding Gains on Marketable Securities	Unrealized Gains (Losses) on Foreign Currency Forward Contracts, Net of Tax	Total
Balance at December 31, 2015	\$(2,080)	\$ 126	\$ 3,778	\$ 1,824
Other comprehensive (loss) income before reclassifications	(5,201 )	200	1,847	(3,154 )
Amounts reclassified from accumulated other comprehensive loss	—	—	(2,060 )	(2,060 )
Net current period other comprehensive (loss) income	\$(5,201)	\$ 200	\$ (213 )	\$(5,214)
Balance at June 30, 2016	\$(7,281)	\$ 326	\$ 3,565	\$(3,390)

## 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 foreign currency forward contracts that are designated as cash flow hedges under U.S. 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 June 30, 2017, 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:

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	As of June 30, 2017	As of December 31, 2016
Foreign Currency	(in thousands)	
Euro	\$209,800	\$164,368
British pound sterling	71,917	65,237
Australian dollar	28,680	23,776
Total foreign currency forward contracts	\$310,397	\$253,381

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 June 30, 2017

Assets		Liabilities	
Classification	Fair Value	Classification	Fair Value
(in thousands)			
Prepaid and other current assets	\$ 985	Other liabilities, current portion	\$(8,067)
Other assets	—	Other liabilities, excluding current portion	(1,435 )
Total assets	\$ 985	Total liabilities	\$(9,502)

As of December 31, 2016

Assets		Liabilities	
Classification	Fair Value	Classification	Fair Value
(in thousands)			
Prepaid and other current assets	\$14,407	Other liabilities, current portion	\$(144)
Other assets	1,186	Other liabilities, excluding current portion	—
Total assets	\$15,593	Total liabilities	\$(144)

The following table summarizes the potential effect of offsetting derivatives by type of financial instrument on the Company's condensed consolidated balance sheets:

As of June 30, 2017

	Gross Amounts Recognized	Gross Amounts Offset	Gross Amounts Presented	Gross Amounts Not Offset	Legal Offset
Foreign currency forward contracts (in thousands)					
Total assets	\$985	\$	—\$985	\$ (985 )	\$—
Total liabilities	\$(9,502)	\$	—\$(9,502 )	\$ 985	\$(8,517)

As of December 31, 2016

	Gross Amounts Recognized	Gross Amounts Offset	Gross Amounts Presented	Gross Amounts Not Offset	Legal Offset
Foreign currency forward contracts (in thousands)					
Total assets	\$15,593	\$	—\$15,593	\$ (144 )	\$15,449
Total liabilities	\$(144 )	\$	—\$(144 )	\$ 144	\$—



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## I. Inventories

Inventories consisted of the following:

	As of	As of
	June 30,	December
	2017	31, 2016
	(in thousands)	
Raw materials	\$ 13,876	\$ 6,348
Work-in-process	63,116	56,672
Finished goods	15,271	14,584
Total	\$ 92,263	\$ 77,604

Based on its evaluation of, among other factors, information regarding tezacaftor's safety and efficacy, the Company has capitalized \$4.9 million of inventory costs for tezacaftor manufactured in preparation for its potential product launch as of June 30, 2017. In periods prior, the Company expensed costs associated with tezacaftor's raw materials and work-in-process as a development expense. The Company submitted a New Drug Application to the United States Food and Drug Administration and a Marketing Authorization Application to the European Medicines Agency for tezacaftor in combination with ivacaftor. The Company plans to continue to monitor the status of the tezacaftor regulatory process and the other factors used to determine whether or not to capitalize the tezacaftor inventory and, if there are significant negative developments regarding tezacaftor, the Company could be required to impair previously capitalized costs.

## J. Intangible Assets and Goodwill

## Intangible Assets

As of June 30, 2017 and December 31, 2016, in-process research and development intangible assets of \$284.3 million were recorded on the Company's condensed consolidated balance sheet. In 2015, the Company recorded an in-process research development intangible asset of \$255.3 million related to Parion's pulmonary ENaC platform, including the intellectual property related to VX-371 and VX-551, that are licensed by Parion to the Company. In 2014, the Company recorded an in-process research development intangible asset of \$29.0 million related to VX-210 that is licensed by BioAxxon to the Company.

## Goodwill

As of June 30, 2017 and December 31, 2016, 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.





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Property and equipment, net, included \$482.4 million and \$489.0 million as of June 30, 2017 and December 31, 2016, respectively, related to construction costs for the Fan Pier Buildings. The carrying value of the Company's lease agreement liability for the Fan Pier Buildings was \$472.4 million and \$472.6 million as of June 30, 2017 and December 31, 2016, 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 to be built in San Diego, California. The lease will commence upon completion of the building, scheduled for the first half of 2018, and will extend for 16 years from the commencement date. Pursuant to the lease agreement, 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 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.

Property and equipment, net, included \$57.1 million and \$15.0 million as of June 30, 2017 and December 31, 2016, respectively, related to construction costs for the San Diego Building. The carrying value of the Company's lease agreement liability for the San Diego Building was \$50.2 million and \$12.6 million as of June 30, 2017 and December 31, 2016, 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 proceeds of the borrowing under the Credit Agreement were used primarily to repay the Company's then outstanding indebtedness under the Macquarie Loan (as defined below). 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

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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.

**Term Loan**

In July 2014, the Company entered into a credit agreement with the lenders party thereto, and Macquarie US Trading LLC ("Macquarie"), as administrative agent. The credit agreement provided for a \$300.0 million senior secured term loan (the "Macquarie Loan"). On October 13, 2016, the Company terminated and repaid all outstanding obligations under the Macquarie Loan.

The Macquarie Loan initially bore interest at a rate of 7.2% per annum, which was reduced to 6.2% per annum based on the FDA's approval of ORKAMBI. The Term Loan bore interest at a rate of LIBOR plus 5.0% per annum during the third year of the term.

The Company incurred \$5.3 million in fees paid to Macquarie that were recorded as a discount on the Macquarie Loan and were recorded as interest expense using the effective interest method over the term of the loan in the Company's condensed consolidated statements of operations.

**L. Stock-based Compensation Expense**

During the three and six months ended June 30, 2017 and 2016, the Company recognized the following stock-based compensation expense:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
	(in thousands)			
Stock-based compensation expense by type of award:				
Stock options	\$27,915	\$31,826	\$54,896	\$58,086
Restricted stock and restricted stock units	43,906	29,608	84,651	57,141
ESPP share issuances	2,246	1,436	4,310	3,960
Less stock-based compensation expense capitalized to inventories	(1,485 )	(928 )	(2,293 )	(1,773 )
Total stock-based compensation included in costs and expenses	\$72,582	\$61,942	\$141,564	\$117,414

**Stock-based compensation expense by line item:**

Research and development expenses	\$43,832	\$40,640	\$88,669	\$75,088
Sales, general and administrative expenses	28,750	21,302	52,895	42,326
Total stock-based compensation included in costs and expenses	\$72,582	\$61,942	\$141,564	\$117,414

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

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As of June 30, 2017  
 Unrecognized Weighted-average  
 Expense Recognition Period  
 (in thousands) (in years)

## Type of award:

Stock options	\$165,047	2.51
Restricted stock and restricted stock units	\$249,474	2.41
ESPP share issuances	\$5,064	0.62

The following table summarizes information about stock options outstanding and exercisable at June 30, 2017:

Range of Exercise Prices	Options Outstanding		Weighted-average Exercise Price (per share)	Options Exercisable	
	Number	Weighted-average		Number	Weighted-average
	Outstanding	Remaining Contractual Life		Exercisable	Exercise Price
	(in thousands)	(in years)		(in thousands)	(per share)
\$18.93–\$20.00	129	0.61	\$ 18.93	129	\$ 18.93
\$20.01–\$40.00	943	2.57	\$ 34.74	943	\$ 34.74
\$40.01–\$60.00	1,233	5.15	\$ 48.58	1,233	\$ 48.58
\$60.01–\$80.00	1,042	6.69	\$ 75.83	784	\$ 75.63
\$80.01–\$100.00	5,336	8.45	\$ 89.52	1,733	\$ 89.89
\$100.01–\$120.00	1,437	7.56	\$ 109.35	786	\$ 109.27
\$120.01–\$131.89	1,416	8.03	\$ 130.34	763	\$ 130.00
Total	11,536	7.21	\$ 86.12	6,371	\$ 77.74

## M. Other Arrangements

## Sale of HIV Protease Inhibitor Royalty Stream

In 2008, the Company sold to a third party its rights to receive royalty payments from GlaxoSmithKline plc, net of royalty amounts to be earned by and due to a third party, for a one-time cash payment of \$160.0 million. These royalty payments relate to net sales of HIV protease inhibitors, which had been developed pursuant to a collaboration agreement between the Company and GlaxoSmithKline plc. As of June 30, 2017, the Company had \$9.6 million in deferred revenues related to the one-time cash payment, which it is recognizing over the life of the collaboration agreement with GlaxoSmithKline plc based on the units-of-revenue method. In addition, the Company continues to recognize royalty revenues equal to the amount of the third-party subroyalty and an offsetting royalty expense for the third-party subroyalty payment.

## N. Income Taxes

The Company is subject to United States federal, state, and foreign income taxes. For the three and six months ended June 30, 2017, the Company recorded a provision for income taxes of \$4.3 million and \$8.3 million, respectively, which included a provision of \$8.1 million and \$8.5 million, respectively, related to the Company's VIEs' income tax provision. The Company has no liability for taxes payable by the Company's VIEs and the income tax provision and related liability have been allocated to noncontrolling interest (VIE). For the three and six months ended June 30, 2016, the Company recorded a provision for income taxes of \$18.1 million and \$23.6 million, respectively, which included a provision of \$17.5 million and \$20.6 million, respectively, related to the Company's VIEs' income tax provision.

As of June 30, 2017 and December 31, 2016, the Company did not have unrecognized tax benefits. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of June 30, 2017, no interest and penalties have been accrued. The Company does not expect that its unrecognized tax benefits will

materially

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increase within the next twelve months. The Company did not recognize any material interest or penalties related to uncertain tax positions as of June 30, 2017 and December 31, 2016.

The Company continues to maintain a valuation allowance against certain deferred tax assets where it is more likely than not that the deferred tax asset will not be realized because of its extended history of annual losses.

As described in Footnote A, the Company adopted Accounting Standards Update (ASU) 2016-09, during the six month period ended June 30, 2017. The ASU eliminates additional paid in capital ("APIC") pools and requires excess tax benefits and tax deficiencies to be recorded in the condensed consolidated statement of operations when the awards vest or are settled. Amendments related to accounting for excess tax benefits have been adopted prospectively resulting in a tax benefit of \$30.4 million and \$30.8 million for the three and six months ended June 30, 2017, respectively. In connection with the adoption of this new standard, the Company recorded a cumulative-effect adjustment of \$410.8 million as of January 1, 2017 to accumulated deficit and deferred tax assets, with an equal offsetting adjustment to the Company's valuation allowance. In addition, the Company has recorded \$9.4 million related to the impact from adoption of the provisions related to forfeiture rates to accumulated deficit. This change also increased the Company's deferred tax assets by \$3.4 million that is offset by an increase to the valuation allowance in the same amount.

The Company files United States 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 United States or any other major taxing jurisdiction for years before 2011, except where the Company has net operating losses or tax credit carryforwards that originate before 2011. The Company currently is under examination by Canada Revenue Agency for the years ending December 31, 2011 through December 31, 2013. No adjustments have been reported.

At June 30, 2017, foreign earnings, which were not significant, have been retained indefinitely by foreign subsidiary companies for reinvestment; therefore, no provision has been made for income taxes that would be payable upon the distribution of such earnings, and it would not be practicable to determine the amount of the related unrecognized deferred income tax liability. Upon repatriation of those earnings, in the form of dividends or otherwise, the Company would be subject to United States federal income taxes (subject to an adjustment for foreign tax credits) and withholding taxes payable to the various foreign countries.

**O. Restructuring Liabilities**

**Research and Development Restructuring**

In February 2017, the Company decided to consolidate its research activities into its Boston, Milton Park and San Diego locations and to close the research site in Canada. As a result, the Company is in the process of closing one of its research sites. In connection with this decision, approximately 70 positions were affected. The Company estimates that it will incur aggregate restructuring charges of approximately \$12.4 million, including \$6.9 million for employee salary, severance and benefit costs, \$2.2 million in assets associated with the restructuring that have become impaired and \$3.3 million for other costs primarily related to the Company's exit from the facility.

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The restructuring charge and other activities recorded during the the three and six months ended June 30, 2017 and the related liability balance as of June 30, 2017 were as follows:

	Three Months Ended June 30, 2017 (in thousands)	Six Months Ended June 30, 2017
Liability, beginning of the period	\$3,727	\$—
Restructuring expense	3,222	12,440
Cash payments	(3,861 )	(7,119 )
Asset impairments and other non-cash items	419	(1,814 )
Liability, end of the period	\$3,507	\$3,507

## 2003 Kendall Restructuring

In 2003, the Company adopted a plan to restructure its operations to coincide with its increasing internal emphasis on advancing drug candidates through clinical development to commercialization. The restructuring liability relates to specialized laboratory and office space that is leased to the Company pursuant to a 15-year lease that terminates in 2018. The Company has not used more than 50% of this space since it adopted the plan to restructure its operations in 2003. This unused laboratory and office space currently is subleased to third parties.

The activities related to the restructuring liability for the three and six months ended June 30, 2017 and 2016 were as follows:

	Three Months Ended June 30, 2017    2016		Six Months Ended June 30, 2017    2016	
	(in thousands)			
Liability, beginning of the period	\$2,525	\$7,224	\$4,328	\$7,944
Restructuring expense	342	(11 )	827	192
Cash payments	(3,695 )	(3,833 )	(8,959 )	(7,764 )
Cash received from subleases	2,818	3,008	5,794	6,016
Liability, end of the period	\$1,990	\$6,388	\$1,990	\$6,388

## Fan Pier Move Restructuring

In connection with the relocation of its Massachusetts operations to Fan Pier in Boston, Massachusetts, which commenced in 2013, the Company is incurring restructuring charges related to its remaining lease obligations at its facilities in Cambridge, Massachusetts. The majority of these restructuring charges were recorded in the third quarter of 2014 upon decommissioning three facilities in Cambridge. During 2015, the Company terminated two of these lease agreements resulting in a credit to restructuring expense equal to the difference between the Company's estimated future cash flows related to its lease obligations for these facilities and the termination payment paid to the Company's landlord on the effective date of the termination. The third major facility included in this restructuring activity is 120,000 square feet of the Kendall Square Facility that the Company continued to use for its operations following its 2003 Kendall Restructuring. The rentable square footage in this portion of the Kendall Square Facility was subleased to a third party in February 2015. The Company will continue to incur charges through April 2018 related to the difference between the Company's estimated future cash flows related to this portion of the Kendall Square Facility, which include an estimate for sublease income to be received from the Company's sublessee and its actual cash flows. The Company discounted the estimated cash flows related to this restructuring activity at a discount rate of 9%. The activities related to the restructuring liability for the three and six months ended June 30, 2017 and 2016 were as follows:





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	Three Months		Six Months	
	Ended June 30,		Ended June 30,	
	2017	2016	2017	2016
	(in thousands)			
Liability, beginning of the period	\$1,995	\$5,449	\$3,626	\$5,964
Restructuring expense	(41 )	149	255	382
Cash payments	(2,911 )	(3,096 )	(7,316 )	(6,252 )
Cash received from subleases	2,478	2,361	4,956	4,769
Liability, end of the period	\$1,521	\$4,863	\$1,521	\$4,863

## Other Restructuring Activities

The Company has engaged in several other restructuring activities that are unrelated to its Research and Development Restructuring, 2003 Kendall Restructuring and Fan Pier Move Restructuring. The most significant activity commenced in October 2013 when the Company adopted a restructuring plan that included (i) a workforce reduction primarily related to the commercial support of INCIVEK following the continued and rapid decline in the number of patients being treated with INCIVEK as new medicines for the treatment of HCV infection neared approval and (ii) the write-off of certain assets. This action resulted from the Company's decision to focus its investment on future opportunities in CF and other research and development programs.

The remaining restructuring activities were completed in 2016. As such, there was no outstanding liability as of June 30, 2017. The activities related to the Company's other restructuring liabilities for the three and six months ended June 30, 2016 were as follows:

	Three	Six
	Months	Months
	Ended	Ended
	June 30,	June 30,
	2016	2016
	(in thousands)	
Liability, beginning of the period	\$1,262	\$1,450
Restructuring expense	205	456
Cash payments	(234 )	(673 )
Liability, end of the period	\$1,233	\$1,233

## P. 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.

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VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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 June 30, 2017 or December 31, 2016.

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

#### OVERVIEW

We are in the business of discovering, developing, manufacturing and commercializing medicines for serious diseases. We use precision medicine approaches with the goal of creating transformative medicines for patients in specialty markets. 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 indications. Our two marketed products are ORKAMBI (lumacaftor in combination with ivacaftor) and KALYDECO (ivacaftor) and we are currently seeking approval for tezacaftor in combination with ivacaftor, which is a two-drug combination regimen for patients with CF. In addition, we are evaluating multiple triple combination regimens in patients with CF that include one or more next-generation CFTR corrector compounds in Phase 1 and Phase 2 clinical trials.

#### Cystic Fibrosis

ORKAMBI and KALYDECO are approved to treat approximately 40% of the 75,000 CF patients in North America, Europe and Australia. ORKAMBI is approved as a treatment for approximately 25,000 patients who have two copies of the F508del mutation, or F508del homozygous, in their cystic fibrosis transmembrane conductance regulator, or CFTR, gene. KALYDECO is approved for the treatment of approximately 5,000 CF patients who have the G551D mutation or other specified mutations in their CFTR gene. Our goal is to develop treatment regimens that will provide benefits to as many patients with CF as possible and will enhance the benefits that currently are being provided to patients taking our medicines.

If tezacaftor in combination with ivacaftor is approved, we expect that it would provide an additional treatment option primarily to CF patients who are currently eligible for either ORKAMBI or KALYDECO. If we are able to successfully develop a triple combination regimen that includes a next-generation CFTR corrector compound, including VX-440, VX-152, VX-659 or VX-445, we believe such regimen could potentially provide benefit 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 patients who have one copy of the F508del mutation in their CFTR gene and a second mutation in their CFTR gene that results in minimal CFTR function, or F508del/Min patients, and (ii) an additional treatment option to CF patients who are eligible for either ORKAMBI, KALYDECO or, if approved, tezacaftor in combination with ivacaftor.

#### Tezacaftor in combination with ivacaftor

In the first quarter of 2017, we obtained positive results from two Phase 3 clinical trials of tezacaftor, a corrector compound, in combination with ivacaftor. The clinical trials demonstrated that the tezacaftor/ivacaftor combination provided statistically significant improvements in lung function (percent predicted forced expiratory volume in one second, or ppFEV1) in patients with CF 12 years of age and older who have certain mutations in their CFTR gene. The 24-week EVOLVE clinical trial evaluated tezacaftor in combination with ivacaftor in F508del homozygous patients with CF. This clinical trial met its primary endpoint with a mean absolute improvement in ppFEV1 through 24 weeks of 4.0 percentage points from baseline compared to placebo ( $p < 0.0001$ ). The second clinical trial, EXPAND, was an 8-week crossover clinical trial that evaluated the combination treatment in patients with CF who have one mutation that results in residual CFTR function and one F508del mutation. This clinical trial met the primary endpoints of absolute change in ppFEV1 from baseline to the average of the Week 4 and Week 8 measurements, with the tezacaftor/ivacaftor combination treatment demonstrating a mean absolute improvement of 6.8 percentage points compared to placebo ( $p < 0.0001$ ) and the ivacaftor monotherapy group demonstrating a mean absolute improvement of 4.7 percentage points compared to placebo ( $p < 0.0001$ ). Across both clinical trials, the tezacaftor/ivacaftor combination treatment was generally well tolerated.

Based on these results, we submitted a New Drug Application, or NDA, to the U.S. Food and Drug Administration, or FDA, and a Marketing Authorization Application, or MAA, to the European Medicines Agency, or EMA, for tezacaftor in combination with ivacaftor in patients with CF 12 years of age and older who are F508del homozygous or who have one copy of the F508del mutation in their CFTR gene and a second mutation in their CFTR gene that results in residual CFTR function.

In addition, we have completed enrollment in a Phase 3 clinical trial evaluating tezacaftor in combination with ivacaftor in patients 12 years of age or older who have one copy of the F508del mutation in their CFTR gene and a

second mutation that results in a gating mutation in the CFTR gene that has been shown to be responsive to ivacaftor alone. We expect to receive data from this clinical trial in the second half of 2017. We also are conducting a Phase 3 clinical trial of the tezacaftor/ivacaftor combination in patients with CF six to eleven years of age in the U.S. The clinical trial is evaluating the safety and tolerability of the tezacaftor/ivacaftor combination in children who are homozygous for the F508del mutation and in children who have one copy of the F508del mutation and a gating or residual function mutation.

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### Next-generation CFTR corrector compounds

In July 2017, we obtained positive results from Phase 1 and Phase 2 clinical trials of three different triple combination regimens in 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. Initial data from the Phase 2 clinical trials showed mean absolute improvements in ppFEV1 of 9.7 and 12.0 percentage points for VX-152 (200mg q12h) and VX-440 (600mg q12h), respectively, in triple combination with tezacaftor and ivacaftor. Initial data from the Phase 1 clinical trial showed mean absolute improvement in ppFEV1 of 9.6 percentage points for VX-659 in triple combination with tezacaftor and ivacaftor. Additional data on VX-152 and VX-440, as well as data from ongoing or planned Phase 2 clinical trials of VX-445 and VX-659 are expected in late 2017 or early 2018. Pending additional data from these ongoing and planned clinical trials and discussions with regulatory agencies and a steering committee of global CF experts, we plan to initiate pivotal development of one or more triple combination regimens in the first half of 2018.

Further information on the data from the Phase 1 and Phase 2 clinical trials of our next-generation CFTR corrector compounds is set forth below in the section titled “Next-Generation Clinical Trial Data.”

### ENaC Inhibition

VX-371, an investigational epithelial sodium channel, or ENaC, inhibitor, is being evaluated in a Phase 2 development program. We exclusively licensed VX-371 from Parion Sciences, Inc., or Parion, in 2015.

### Research and Development

We are engaged in a number of other research and mid- and early-stage development programs, including in the areas of pain and neurology. We have also entered into third-party collaborations, pursuant to which we are engaged in the discovery and development of nucleic acid-based therapies for a variety of diseases, including CF. We plan to continue investing in our research programs and fostering scientific innovation in order to identify and develop transformative medicines. Our current research programs include programs targeting cystic fibrosis, adrenoleukodystrophy, alpha-1 antitrypsin deficiency, sickle cell disease and polycystic kidney disease. 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.

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.

### Collaboration Arrangements

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:



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CRISPR Therapeutics AG, or 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;

Parion, pursuant to which we are developing ENaC inhibitors for the treatment of pulmonary diseases;

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

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

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's and Moderna's activities unrelated to our collaborations have no effect on our consolidated financial statements. Parion and BioAxone are being accounted for as variable interest entities, or VIEs, and are 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. Each of our consolidated VIEs is engaging in activities unrelated to our collaboration, including in the case of Parion, seeking to develop novel treatments for pulmonary and ocular diseases. The revenues and expenses unrelated to the programs we in-license from our VIEs are immaterial to our consolidated financial statements. In each case, the activities unrelated to our collaboration represent approximately 1% of our total revenues and total expenses on an annual basis. Because we consolidate our 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).

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

Merck KGaA, which is advancing four oncology research and development programs; and

Janssen Pharmaceuticals, Inc., which is developing JNJ-3872 (formerly VX-787) for the treatment of influenza.

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.

## 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 statutes, 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. False claims laws prohibit anyone from 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 services not provided as claimed, or claims for medically unnecessary items or

services. 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

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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 are working 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. To date, we have reached a pricing and reimbursement agreement for ORKAMBI with several European countries, including Germany, Ireland and Italy, and remain in negotiations with several others. Consistent with our experience with KALYDECO when it was first approved, we expect reimbursement discussions in ex-U.S. markets may take a significant period of time.

### Recent Transaction

#### Concert Pharmaceuticals

In July 2017, we acquired certain CF assets, including CTP-656, from Concert Pharmaceuticals, Inc., or Concert, pursuant to an agreement that we entered into in March 2017. CTP-656 is an investigational CFTR potentiator that has the potential to be used as part of future once-daily combination regimens of CFTR modulators that treat the underlying cause of CF. Pursuant to the agreement, in the third quarter of 2017, we paid Concert \$160 million in cash for all worldwide development and commercialization rights to CTP-656. If CTP-656 is approved as part of a combination regimen to treat CF, Concert could receive up to an additional \$90 million in milestones based on regulatory approval in the U.S. and reimbursement in the UK, Germany or France. There was no accounting impact relating to this agreement during the six months ended June 30, 2017. In the third quarter of 2017, we expect to record the \$160 million payment as a research and development expense.

### Next-Generation Clinical Trial Data

#### VX-440

We are evaluating VX-440 (200mg and 600mg q12h) in combination with tezacaftor and ivacaftor as part of a randomized, double-blind Phase 2 clinical trial in F508del/Min patients and in F508del homozygous patients, in each case who are 18 years of age and older. The primary objectives for the clinical trial are safety, tolerability and efficacy as assessed by mean absolute change in ppFEV1 from baseline. Secondary endpoints include change in sweat chloride and Cystic Fibrosis Questionnaire-Revised, or CFQ-R. In July 2017, we obtained initial data from this clinical trial, which is set forth below.

**Overall Safety Data:** In the clinical trial, the triple combination regimen was generally well tolerated. The majority of adverse events were mild or moderate. The most common adverse events (>10%), regardless of treatment group, were infective pulmonary exacerbation, cough, sputum increased and diarrhea. There was one discontinuation due to an adverse event in the triple combination treatment groups (elevated liver enzymes >5x upper limit of normal in the VX-440 600mg group) and one in the control groups (respiration abnormal and sputum increased). One additional patient treated with the triple combination had elevated liver enzymes (>8x upper limit of normal in the VX-440 600mg group), which were observed on the final day of dosing. In both patients, the elevated liver enzymes returned to normal after treatment discontinuation or completion.

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4-Week Efficacy Data in F508del/Min Patients: The first part of the clinical trial evaluated the triple combination for four weeks in 47 F508del/Min patients (11 in placebo, 18 in VX-440 200mg and 18 in VX-440 600mg). A summary of the within-group lung function and sweat chloride data is provided below:

## VX-440 in F508del/Min Patients

Mean Absolute Within-Group Change From Baseline Through Day 29*	Mean Absolute Within-Group Change in ppFEV <sub>1</sub> (percentage points)	Mean Absolute Within-Group Change in Sweat Chloride (mmol/L)
Triple placebo	+1.4 (p=0.4908)	+1.6 (p=0.6800)
VX-440 (200mg q12h) + tezacaftor (50mg q12h or 100mg QD) + ivacaftor (150mg q12h)	+10.0 (p<0.0001)	-20.7 (p<0.0001)
VX-440 (600mg q12h) + tezacaftor (50mg q12h) + ivacaftor (300mg q12h)	+12.0 (p<0.0001)	-33.1 (p<0.0001)

\* all p values are within group p-values based on mixed effect models; values expressed as 'Through Day 29' are the average of Day 15 and Day 29 measures

A secondary endpoint in the clinical trial measured mean absolute change in the Respiratory Domain of CFQ-R, a validated patient-reported outcome tool at Day 29. In this clinical trial, the mean absolute improvement for patients with a minimal function mutation who received the triple combination were 18.3 points (VX-440 200mg) and 20.7 points (VX-440 600mg). The improvement for those who received placebo was 2.2 points.

4-Week Efficacy Data in F508del Homozygous Patients: The second part of the clinical trial is ongoing and aims to evaluate the addition of VX-440 for four weeks in 26 F508del homozygous patients who were already receiving the combination of tezacaftor and ivacaftor (6 in placebo and 20 in VX-440 600mg). In this part of the clinical trial, all participants received four weeks of treatment with tezacaftor and ivacaftor and were then randomized to the addition of VX-440 or placebo for four additional weeks. A summary of the within-group lung function and sweat chloride data for the triple combination treatment period, from baseline (end of the 4-week tezacaftor/ivacaftor run-in period), is provided below:

## VX-440 in F508del/F508del Patients

Mean Absolute Within-Group Change From Baseline Through Day 29*	Mean Absolute Within-Group Change in ppFEV <sub>1</sub> (percentage points)	Mean Absolute Within-Group Change in Sweat Chloride (mmol/L)
Placebo + tezacaftor (100mg QD) + ivacaftor (150mg q12h)	-2.5 (p=0.2755)	+2.1 (p=0.7385)
VX-440 (600mg q12h) + tezacaftor (50mg q12h) + ivacaftor (300mg q12h)	+9.5 (p<0.0001)	-31.3 (p<0.0001)

\* all p values are within group p-values based on mixed effect models; values expressed as 'Through Day 29' are the average of Day 15 and Day 29 measures

The safety follow-up portion of the clinical trial in F508del homozygous patients is ongoing.

## VX-152

We are evaluating VX-152 (100mg, 200mg and 300mg q12h) in combination with tezacaftor and ivacaftor as part of a randomized, double-blind Phase 2 clinical trial in F508del/Min patients and in F508del homozygous patients who are 18 years of age and older. The primary objective for the clinical trial is safety and tolerability. Secondary endpoints include mean absolute change in ppFEV<sub>1</sub> and change in sweat chloride. In July 2017, we obtained data from the 100mg and 200mg arms of the clinical trial in F508del/Min patients and from the 200mg arm in F508del homozygous patients.

Safety Data: In the clinical trial, the triple combination regimen was generally well tolerated. The majority of adverse events were mild or moderate. The most common adverse events (>10%), regardless of treatment group, were cough, sputum increased, infective pulmonary exacerbation, productive cough, diarrhea and fatigue. There was one discontinuation due to an adverse event in the triple combination treatment groups (pneumonia in the VX-152 200mg group) and none in the control groups.



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2-Week Initial Efficacy Data in F508del/Min Patients: In the first part of the clinical trial, the triple combination was evaluated for two weeks in 21 F508del/Min patients 18 years of age and older (5 in combined placebo, 6 in VX-152 100mg and 10 in VX-152 200mg). A summary of the initial within-group lung function and sweat chloride data (secondary endpoints) from the VX-152 100mg and 200mg dose groups is provided below.

## VX-152 in F508del/Min Patients

Observed Mean Absolute Within-Group Change from Baseline at Day 15*	Observed Mean Absolute Within-Group Change in ppFEV <sub>1</sub> (percentage points)	Observed Mean Absolute Within-Group Change in Sweat Chloride (mmol/L)
Triple placebo	-0.9 (p=0.6245)	+1.0 (p=0.5659)
VX-152 (100mg q12h) + tezacaftor (100mg QD) + ivacaftor (150mg q12h)	+5.6 (p=0.0135)	-19.6 (p=0.0004)
VX-152 (200mg q12h) + tezacaftor (100mg QD) + ivacaftor (150mg q12h)	+9.7 (p=0.0017)	-14.1 (p=0.0219)

\* p-values presented are within-group p-values based on 1 sample t-test; an efficacy analysis using mixed effect models will be conducted following completion of an additional cohort of patients currently being treated in the clinical trial

The first part of the clinical trial is ongoing to evaluate the triple combination of VX-152 (300mg q12h), tezacaftor and ivacaftor in F508del/Min patients. We expect this data to be available later in 2017.

2-Week Initial Efficacy Data in F508del Homozygous Patients: The second part of the clinical trial is ongoing to evaluate the addition of VX-152 for two weeks in 14 F508del homozygous patients 18 years of age and older who were already receiving the combination of tezacaftor and ivacaftor (4 in placebo and 10 in VX-152 200mg). A summary of the initial within-group lung function and sweat chloride data (secondary endpoints) for the triple combination treatment period, from baseline (end of the 4-week tezacaftor/ivacaftor run-in period), is provided below:

## VX-152 in F508del/F508del Patients

Observed Mean Absolute Within-Group Change from Baseline at Day 15*	Observed Mean Absolute Within-Group Change in ppFEV <sub>1</sub> (percentage points)	Observed Mean Absolute Within-Group Change in Sweat Chloride (mmol/L)
Placebo + tezacaftor (100mg QD) + ivacaftor (150mg q12h)	-1.4 (p=0.2773)	+3.4 (p=0.1212)
VX-152 (200mg q12h) + tezacaftor (100mg QD) + ivacaftor (150mg q12h)	+7.3 (p=0.0354)	-20.9 (p=0.0010)

\* p-values presented are within-group p-values based on 1 sample t-test; an efficacy analysis using mixed effect models will be conducted following completion of an additional cohort of patients currently being treated in the clinical trial

The second part of the clinical trial is ongoing to evaluate the addition of VX-152 (300mg q12h) for four weeks in F508del homozygous patients who are already receiving the combination of tezacaftor and ivacaftor. We expect this data to be available in early 2018.

## VX-659

We completed a randomized, double-blind, placebo-controlled Phase 1 clinical trial of single and multiple ascending doses of VX-659 alone and in triple combination with tezacaftor and ivacaftor in healthy volunteers. The clinical trial also evaluated the safety and tolerability of VX-659 as part of a triple combination for two weeks in 12 F508del/Min patients 18 years of age and older (3 in placebo and 9 in VX-659 120mg). In this part of the clinical trial, sweat chloride was evaluated as an additional endpoint, and the absolute change in ppFEV<sub>1</sub> was evaluated as part of the safety analysis.

In patients with CF, VX-659 was generally well tolerated in triple combination with tezacaftor and ivacaftor. The majority of adverse events were mild or moderate. The most common adverse events (>10%), regardless of treatment group,

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were cough, infective pulmonary exacerbation and productive cough. There were no discontinuations due to adverse events in either group.

At Day 15, there was a mean absolute improvement in ppFEV1 of +9.6 percentage points from baseline in those receiving the triple combination regimen of VX-659 (120mg q12h), tezacaftor and ivacaftor and a mean decrease in sweat chloride of -41.6 mmol/L. For those receiving placebo, there was a mean absolute decrease in ppFEV1 of -0.4 percentage points and a mean decrease in sweat chloride of 11.0 mmol/L.

**Risks associated with Next-Generation CFTR Corrector Compounds**

The continued development of our next-generation CFTR corrector compounds remains subject to a number of risks and uncertainties including, among other things, (i) that we could experience unforeseen delays in conducting our development programs relating to triple combination treatments and in submitting related regulatory filings, (ii) that the initial results set forth above may differ from the final results from these ongoing clinical trials and may not be predictive of results in future clinical trials, (iii) that regulatory authorities may not approve, or approve on a timely basis, triple combination treatments due to safety, efficacy or other reasons, and (iv) and other risks listed under Risk Factors in Part II, Item 1A of this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K, filed with the SEC on February 23, 2017.



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## RESULTS OF OPERATIONS

	Three Months Ended June 30,		Increase/(Decrease)		Six Months Ended June 30,		Increase/(Decrease)	
	2017	2016	\$	%	2017	2016	\$	%
	(in thousands)				(in thousands)			
Revenues	\$544,135	\$431,608	\$ 112,527	26 %	\$1,258,853	\$829,688	\$ 429,165	52 %
Operating costs and expenses	491,428	428,255	63,173	15 %	935,304	840,665	94,639	11 %
Other items, net	(34,711 )	(67,878 )	33,167	49 %	(57,797 )	(95,179 )	37,382	39 %
Net income (loss) attributable to Vertex	\$17,996	\$(64,525 )	\$ 82,521	n/a	\$265,752	\$(106,156)	\$ 371,908	n/a

## Net Income (Loss) Attributable to Vertex

Net income attributable to Vertex was \$18.0 million in the second quarter of 2017 as compared to a net loss attributable to Vertex of \$(64.5) million in the second quarter of 2016. Our revenues increased in the second quarter of 2017 as compared to the second quarter of 2016 primarily due to increased ORKAMBI net product revenues. Our operating costs and expenses increased in the second quarter of 2017 as compared to the second quarter of 2016 primarily due to increases in cost of product revenues, research and development expenses and sales, general and administrative expenses.

Net income attributable to Vertex was \$265.8 million in the first half of 2017 as compared to a net loss attributable to Vertex of \$(106.2) million in the first half of 2016. Our revenues increased significantly in the first half of 2017 as compared to the first half of 2016 due to \$230.0 million in one-time collaborative revenues related to the strategic collaboration and license agreement we established with Merck KGaA in the first quarter of 2017 and increased ORKAMBI and KALYDECO net product revenues. Our operating costs and expenses increased in the first half of 2017 as compared to the first half of 2016 primarily due to increases in cost of product revenues, research and development expenses, sales, general and administrative expenses and restructuring expenses.

## Diluted Net Income (Loss) Per Share Attributable to Vertex Common Shareholders

Diluted net income per share attributable to Vertex common shareholders was \$0.07 in the second quarter of 2017 as compared to a diluted net loss per share attributable to Vertex common shareholders of \$(0.26) in the second quarter of 2016. Diluted net income per share attributable to Vertex common shareholders was \$1.06 in the first half of 2017 as compared to a diluted net loss per share attributable to Vertex common shareholders of \$(0.43) in the first half of 2016.

## Revenues

	Three Months Ended June 30,		Increase/(Decrease)		Six Months Ended June 30,		Increase/(Decrease)	
	2017	2016	\$	%	2017	2016	\$	%
	(in thousands)				(in thousands)			
Product revenues, net	\$513,988	\$425,651	\$ 88,337	21 %	\$994,610	\$820,061	\$ 174,549	21 %
Royalty revenues	2,861	5,282	(2,421 )	(46 )%	4,412	8,878	(4,466 )	(50 )%
Collaborative revenues	27,286	675	26,611	n/a	259,831	749	259,082	n/a
Total revenues	\$544,135	\$431,608	\$ 112,527	26 %	\$1,258,853	\$829,688	\$ 429,165	52 %

## Product Revenues, Net

	Three Months Ended June 30,		Increase/(Decrease)		Six Months Ended June 30,		Increase/(Decrease)	
	2017	2016	\$	%	2017	2016	\$	%
	(in thousands)				(in thousands)			
ORKAMBI	\$324,407	\$245,496	\$ 78,911	32 %	\$619,268	\$468,624	\$ 150,644	32 %
KALYDECO	189,633	180,235	9,398	5 %	\$375,348	\$350,744	\$ 24,604	7 %
INCIVEK	(52 )	(80 )	28	35 %	(6 )	693	(699 )	n/a
Total product revenues, net	\$513,988	\$425,651	\$ 88,337	21 %	\$994,610	\$820,061	\$ 174,549	21 %

Our total net product revenues increased in the second quarter and first half of 2017 as compared to the second quarter and first half of 2016 primarily due to increased net product revenues from ORKAMBI. In the second quarter and first half of 2017, we recognized approximately \$36.3 million and \$67.7 million, respectively, in ex-U.S. ORKAMBI net product

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revenues, as compared to \$15.9 million and \$24.7 million in the second quarter and first half of 2016, respectively. Our condensed consolidated balance sheets include \$147.7 million collected as of June 30, 2017, in France related to ORKAMBI, which has not resulted in any revenues. We believe that the level of our ORKAMBI revenues during 2017 will be dependent upon whether, when and on what terms we are able to obtain reimbursement in additional ex-U.S. markets, the number and rate at which additional patients begin treatment with ORKAMBI, the proportion of initiated patients who remain on treatment and the compliance rates for patients who remain on treatment.

KALYDECO net product revenues increased in the second quarter of 2017 as compared to the second quarter of 2016 primarily due to additional patients being treated with KALYDECO as a result of label expansions. The increase in KALYDECO net product revenues in the first half of 2017 as compared to the first half of 2016 included approximately \$9 million in one-time revenue credits in the first quarter of 2017 primarily related to the finalization of reimbursement agreements in certain European countries. In the second quarter and first half of 2017, we recognized approximately \$78.0 million and \$162.2 million, respectively, in ex-U.S. KALYDECO net product revenues, as compared to \$76.9 million and \$152.5 million in the second quarter and first half of 2016, respectively.

We have withdrawn INCIVEK, which we previously marketed as a treatment for hepatitis C virus infection, from the market in the United States.

**Royalty Revenues**

Our royalty revenues were \$2.9 million and \$4.4 million in the second quarter and first half of 2017, respectively, as compared to \$5.3 million and \$8.9 million in the second quarter and first half of 2016, respectively. Our royalty revenues primarily consist of revenues related to a cash payment we received in 2008 when we sold our rights to certain HIV royalties.

**Collaborative Revenues**

Our collaborative revenues were \$27.3 million and \$259.8 million in the second quarter and first half of 2017, respectively, as compared to \$0.7 million and \$0.7 million in the second quarter and first half of 2016, respectively. The increase in our collaborative revenues during the second quarter of 2017 as compared to the second quarter of 2016 was primarily related to a \$20.0 million upfront payment received by Parion, one of our VIEs, in the second quarter of 2017 as part of a license agreement it entered into with a third party. We are not a party to such license agreement and have no economic interest in either the license or the \$20.0 million upfront payment. The increase in our collaborative revenues during the first half of 2017 as compared to the first half 2016 was primarily due to revenue recognized related to the one-time upfront payment Merck KGaA paid in the first quarter of 2017. 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 June 30,		Increase/(Decrease)		Six Months Ended June 30,		Increase/(Decrease)	
	2017	2016	\$	%	2017	2016	\$	%
	(in thousands)				(in thousands)			
Cost of product revenues	\$70,535	\$44,154	\$26,381	60 %	\$116,777	\$93,943	\$22,834	24 %
Royalty expenses	670	1,098	(428)	(39) %	1,416	1,958	(542)	(28) %
Research and development expenses	289,451	271,008	18,443	7 %	563,014	526,868	36,146	7 %
Sales, general and administrative expenses	127,249	111,652	15,597	14 %	240,575	216,866	23,709	11 %
Restructuring expenses, net	3,523	343	3,180	n/a	13,522	1,030	12,492	n/a
Total costs and expenses	\$491,428	\$428,255	\$63,173	15 %	\$935,304	\$840,665	\$94,639	11 %
Cost of Product Revenues								

Our cost of product revenues includes 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. Pursuant to our agreement with Cystic Fibrosis Foundation Therapeutics Incorporated, or CFFT, our tiered third-party royalties on sales of KALYDECO and ORKAMBI, calculated as a percentage of net sales, range from the single digits to the sub-teens. As

a result of the tiered royalty rate, our cost of product revenues as a percentage of CF product revenues is lower at the beginning of each calendar year.

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In the second quarter of 2017, our cost of product revenues increased as compared to the second quarter of 2016 primarily due to the increased CF net product revenues and the increased third party royalty rate. In the second half of 2017, we expect our cost of product revenues as a percentage of total CF product revenues to be similar to the cost of product revenues as a percentage of total CF product revenues in the second quarter of 2017.

In the first half of 2016, our cost of product revenues included a \$13.9 million commercial milestone that was earned by CFFT related to sales of ORKAMBI. There are no further commercial milestones payable to CFFT.

**Royalty Expenses**

Royalty expenses primarily consist of expenses related to a subroyalty payable to a third party on net sales of an HIV protease inhibitor sold by GlaxoSmithKline. Royalty expenses do not include royalties we pay to CFFT on sales of KALYDECO and ORKAMBI, which instead are included in cost of product revenues.

**Research and Development Expenses**

	Three Months Ended June 30,		Increase/(Decrease)		Six Months Ended June 30,		Increase/(Decrease)	
	2017	2016	\$	%	2017	2016	\$	%
	(in thousands)				(in thousands)			
Research expenses	\$77,222	\$79,886	\$ (2,664 )	(3 )%	\$150,278	\$142,896	\$ 7,382	5 %
Development expenses	212,229	191,122	21,107	11 %	412,736	383,972	28,764	7 %
Total research and development expenses	\$289,451	\$271,008	\$ 18,443	7 %	\$563,014	\$526,868	\$ 36,146	7 %

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 1, 2014, we have incurred \$3.5 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 2016 and the first half of 2017, 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 recently submitted an NDA and an MAA for tezacaftor in combination with ivacaftor. We cannot make a meaningful estimate when, if ever, our other clinical development programs will generate revenues and cash flows.

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## Research Expenses

	Three Months Ended June 30,		Increase/(Decrease)			Six Months Ended June 30,		Increase/(Decrease)		
	2017	2016	\$	%		2017	2016	\$	%	
	(in thousands)					(in thousands)				
Research Expenses:										
Salary and benefits	\$19,508	\$19,268	\$240	1	%	\$41,041	\$39,978	\$1,063	3	%
Stock-based compensation expense	15,034	13,409	1,625	12	%	28,725	24,065	4,660	19	%
Laboratory supplies and other direct expenses	11,824	11,810	14	—	%	23,189	21,684	1,505	7	%
Outsourced services	12,077	6,534	5,543	85	%	19,414	10,695	8,719	82	%
Collaboration and asset acquisition payments	—	11,000	(11,000)	(100)	%	—	11,000	(11,000)	(100)	%
Infrastructure costs	18,779	17,865	914	5	%	37,909	35,474	2,435	7	%
Total research expenses	\$77,222	\$79,886	\$(2,664)	(3)	%	\$150,278	\$142,896	\$7,382	5	%

We maintain a substantial investment in research activities. Our research expenses decreased by 3% in the second quarter of 2017 as compared to the second quarter of 2016 and increased by 5% in the first half of 2017 as compared to the first half 2016. Collaboration and asset acquisition payments in the second quarter and first half of 2016 included \$11.0 million in expenses related to the acquisition of early-stage research assets for which there were no comparable expenses in the second quarter and first half of 2017. We expect to continue to invest in our research programs with a focus on identifying drug candidates with the goal of creating transformative medicines.

## Development Expenses

	Three Months Ended June 30,		Increase/(Decrease)			Six Months Ended June 30,		Increase/(Decrease)		
	2017	2016	\$	%		2017	2016	\$	%	
	(in thousands)					(in thousands)				
Development Expenses:										
Salary and benefits	\$50,680	\$45,062	\$5,618	12	%	\$102,634	\$89,413	\$13,221	15	%
Stock-based compensation expense	28,798	27,231	1,567	6	%	59,944	51,023	8,921	17	%
Laboratory supplies and other direct expenses	12,313	13,005	(692)	(5)	%	23,343	21,255	2,088	10	%
Outsourced services	88,855	71,555	17,300	24	%	162,290	156,043	6,247	4	%
Drug supply costs	1,043	4,204	(3,161)	(75)	%	2,992	6,857	(3,865)	(56)	%
Infrastructure costs	30,540	30,065	475	2	%	61,533	59,381	2,152	4	%
Total development expenses	\$212,229	\$191,122	\$21,107	11	%	\$412,736	\$383,972	\$28,764	7	%

Our development expenses increased by 11% in the second quarter of 2017 as compared to the second quarter of 2016 and increased by 7% in the first half 2017 as compared to the first half of 2016, primarily due to increased outsourced services 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. In the second half of 2017, we expect our development expenses to increase as compared to the first half of 2017 due to expenses related to the advancement of our triple-combination regimens and the \$160 million upfront payment to Concert that we expect to be reflected as a development expense in our condensed consolidated statement of operations in the third quarter of 2017.

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## Sales, General and Administrative Expenses

	Three Months Ended June 30,		Increase/(Decrease)		Six Months Ended June 30,		Increase/(Decrease)	
	2017	2016	\$	%	2017	2016	\$	%
	(in thousands)				(in thousands)			

Sales, general and administrative expenses	\$127,249	\$111,652	\$15,597	14 %	\$240,575	\$216,866	\$23,709	11 %
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Sales, general and administrative expenses increased by 14% in the second quarter of 2017 as compared to the second quarter of 2016 and increased by 11% in the first half 2017 as compared to the first half of 2016, primarily due to increased investment in commercial support for ORKAMBI in ex-U.S. markets.

## Restructuring Expense, Net

We recorded restructuring expenses of \$3.5 million and \$13.5 million in the second quarter and the first half of 2017, respectively, as compared to restructuring expenses of \$0.3 million and \$1.0 million in the second quarter and first half of 2016, respectively. The increases in our restructuring expenses in the second quarter and first half of 2017 primarily relate 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

## Interest Expense, Net

Interest expense, net was \$14.7 million and \$31.4 million in the second quarter and first half of 2017, respectively, as compared to \$20.2 million and \$40.9 million in the second quarter and first half of 2016, respectively. The decrease in interest expense, net in the second quarter and first half of 2017 as compared to the second quarter and first half of 2016 was primarily due to the repayment of the \$300.0 million of the revolving credit facility in February 2017.

During the remainder of 2017, we expect to incur approximately \$30 million of interest expense associated with the leases for our corporate headquarters and our interest expense related to our revolving credit facility will be dependent on whether, and to what extent, we reborrow amounts under the existing facility.

## Other (Expense) Income, Net

Other (expense) income, net was an expense of \$2.5 million and \$3.1 million in the second quarter and first half of 2017 as compared to expense of \$1.2 million in the second quarter of 2016 and income of \$3.2 million in the first half of 2016. Other (expense) income, net in each of the second quarter and first half of 2017 and the second quarter and first half of 2016 was primarily due to foreign exchange gains and losses.

## Income Taxes

We recorded a provision for income taxes of \$4.3 million and \$8.3 million in the second quarter and first half of 2017 as compared to \$18.1 million and \$23.6 million in the second quarter and first half of 2016. The provision for income taxes in the second quarter and first half of 2017 included approximately \$7.4 million in income tax to Parion, one of our VIEs, associated with Parion's entry into a license agreement with a third party in the second quarter of 2017. The provision for income taxes in the second quarter and first half of 2016 was primarily due to income tax on our VIEs.

## Noncontrolling Interest (VIEs)

The net (income) loss attributable to noncontrolling interest (VIEs) recorded on our condensed consolidated statements of operations reflects Parion and 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 three and six months ended June 30, 2017 and 2016 is as follows:

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	Three Months Ended June 30, 2017		Six Months Ended June 30, 2017	
	2016		2016	
	(in thousands)			
(Income) loss attributable to noncontrolling interest before provision for income taxes and changes in fair value of contingent payments	\$(18,045)	\$2,835	\$(16,498)	\$3,674
Provision for income taxes	8,132	17,511	8,523	20,573
Increase in fair value of contingent payments	(3,260 )	(48,720 )	(6,990 )	(58,150 )
Net income attributable to noncontrolling interest	\$(13,173)	\$(28,374)	\$(14,965)	\$(33,903)

**LIQUIDITY AND CAPITAL RESOURCES**

As of June 30, 2017, we had cash, cash equivalents and marketable securities of \$1.67 billion, which represented an increase of \$234 million from \$1.43 billion as of December 31, 2016. In the first half of 2017, our cash, cash equivalents and marketable securities balance increased due to cash received from our collaboration with Merck KGaA in the first quarter of 2017 and cash receipts from product sales, partially offset by the \$300.0 million repayment of our revolving credit facility in the first quarter of 2017. We expect that our future cash flows will be substantially dependent on CF product sales.

**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 and KALYDECO from the United States and ex-U.S. markets. Future net product revenues for ORKAMBI from ex-U.S. markets will be dependent on, among other things, the timing of and ability to complete reimbursement discussions in European countries.

In February 2017, we repaid the \$300.0 million we had borrowed under our \$500.0 million revolving credit facility. 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 half of 2017, 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. 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. Under the terms of our credit agreement entered into in October 2016, we are required to repay any outstanding principal amounts in 2021. We also 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. 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 target and we may enter into additional business development transactions that require additional capital. For example, we paid Concert \$160.0 million in the third quarter of 2017 to acquire certain CF assets including CTP-656.

We expect that cash flows from ORKAMBI and KALYDECO, 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 and KALYDECO 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

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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, 2016, which was filed with the Securities and Exchange Commission, or SEC, on February 23, 2017. There have been no material changes from the contractual commitments and obligations previously disclosed in that Annual Report on Form 10-K, except that:

In February 2017, we repaid the outstanding \$300 million balance of our revolving credit facility.

In July 2017, we acquired certain CF assets including CTP-656 from Concert pursuant to an asset purchase agreement. At closing, we paid Concert \$160 million in cash for all worldwide development and commercialization rights to CTP-656 and may be required to pay up to an additional \$90 million in milestones based on regulatory approval in the U.S. and reimbursement in the UK, Germany or France.

### 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 six months ended June 30, 2017, 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, 2016, which was filed with the SEC on February 23, 2017.

### 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.

##### 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.

##### Foreign Exchange Market Risk

As a result of our foreign operations, we face exposure to movements in foreign currency exchange rates, primarily the Euro, British Pound, Australian Dollar and Canadian Dollar against the U.S. dollar. The current exposures arise primarily from cash, accounts receivable, intercompany receivables, payables 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 hedges for Euro, British Pound and Australian Dollar. These cash flow hedges qualify for hedge accounting. As of June 30, 2017, we held foreign exchange forward contracts with notional amounts totaling \$310.4 million. As of June 30, 2017, our outstanding foreign exchange forward contracts had a net fair value of \$(8.5) million.

Based on our foreign currency exchange rate exposures at June 30, 2017, 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 \$31.0 million at June 30, 2017. 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 June 30, 2017 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 June 30, 2017 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

#### PART II. Other Information

##### Item 1. Legal Proceedings

There have been no material changes from the legal proceedings previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2016, which was filed with the Securities and Exchange Commission, or SEC, on February 23, 2017.

##### 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, 2016, which was filed with the SEC on February 23, 2017. There have been no material changes from the risk factors previously disclosed in the Annual Report on Form 10-K, except that the first four risk factors set forth below shall replace the first three risk factors set forth in the Annual Report on Form 10-K and the fifth risk factor set forth below shall be added as a new risk factor.

All of our product revenues and the vast majority of our total revenues are derived from sales of medicines for the treatment of cystic fibrosis. If we are unable to continue to increase revenues from sales of our cystic fibrosis medicines or if we do not meet the expectations of investors or public equity market analysts, our business would be materially harmed and the market price of our common stock would likely decline.

Substantially all of our product revenues and the vast majority of our total revenues are derived from the sale of CF medicines. As a result, our future success is dependent on our ability to continue to increase revenues from sales of our CF medicines. In the near term, this will require us to maintain KALYDECO net product revenues and increase ORKAMBI net product revenues. In the medium term, this will require us to obtain approval for, and successfully commercialize, tezacaftor in combination with ivacaftor. In the longer term, this will require us to successfully develop, obtain approval for and commercialize at least one triple-combination therapy that will allow us to treat patients who have one copy of the F508del mutation in their CFTR gene and a second mutation in their CFTR gene that results in minimal CFTR function and to improve the treatment options available to patients with CF who are eligible for our current medicines. If we are unable to increase our CF product revenues or if we experience adverse developments with respect to development or commercialization of our CF medicines, our results of operations will be

adversely affected and our business will be materially harmed.

We are investing significant resources in the development of our next-generation CFTR corrector compounds in triple combinations and if we are unable to show the safety and efficacy of these compounds, experience delays in doing so or are unable to successfully commercialize at least one of these medicines, our business would be materially harmed.

We are investing significant resources in the development of our next-generation CFTR corrector compounds, including VX-152, VX-440, VX-659 and VX-445, which we are evaluating as part of triple combination treatment regimens for the treatment of patients with CF. We believe that a significant portion of the long-term value attributed to our company by investors is based on the commercial potential of these triple-combination therapies. In July 2017, we obtained initial positive results from Phase 2 clinical trials of VX-152 and VX-440 and a Phase 1 clinical trial of VX-659. In each case, these clinical trials enrolled a limited number of patients with CF and we expect to receive additional information regarding these compounds in the second half of 2017 and early 2018. Based on these results, we expect to initiate pivotal programs to evaluate one or more of these triple combination regimens in the first half of 2018.

In order to ultimately obtain approval for a triple-combination regimen, we will need to demonstrate that the compounds are safe and effective in a significantly larger number of patients than were involved in the clinical trials conducted to date. Initial results from ongoing clinical trials may differ materially from final results from such clinical trials. The results from preclinical and early clinical studies do not always accurately predict results in later, large-scale clinical trials. If the data from our ongoing or planned clinical trials or non-clinical studies of triple combination regimens including our next-generation CFTR compounds are not favorable, the FDA and comparable foreign regulatory authorities may not approve these treatment regimens and/or we may be forced to delay or terminate the development of these treatment regimens, which would have an adverse effect on our business. Even successfully completed large-scale clinical trials may not result in marketable medicines. If a triple combination that includes a next-generation CFTR corrector compounds fails to achieve its primary endpoint in clinical trials, if safety issues arise or if the results from our clinical trials are otherwise

inadequate to support regulatory approval of our triple combination therapies, commercialization of that combination regimen could be delayed or halted.

Even if we gain marketing approval for one or more combination therapies containing a next-generation CFTR corrector compound in a timely manner, we cannot be sure that such combination therapy will be commercially successful. In addition, since we expect that a significant portion of the patients for whom a triple combination treatment regimen would be indicated would also be eligible for our then existing medicines, a portion of the revenues from our triple combination regimens will likely displace revenues from our then marketed products reducing the overall effect of the commercialization of our triple combination regimens on our total revenues.

If the anticipated or actual timing of marketing approvals for these compounds, or the market acceptance of these compounds, if approved, including treatment reimbursement levels agreed to by third-party payors, do not meet the expectations of investors or public market analysts, the market price of our common stock would likely decline.

Our business currently depends heavily on ORKAMBI and KALYDECO net product revenues and we expect to continue to depend on these revenues at least until we obtain approval for tezacaftor in combination with ivacaftor.

Our two marketed medicines are ORKAMBI and KALYDECO, which are approved to treat patients with CF who have specific mutations in their CFTR gene. ORKAMBI and KALYDECO net product revenues represented approximately 49% and 30% of our total revenues in the first half of 2017, respectively, and we expect ORKAMBI and KALYDECO net product revenues to represent substantially all of our total revenues for the remainder of 2017. A majority of our net product revenues are from sales of ORKAMBI and most of our ORKAMBI net product revenues have come from the United States. We have recognized limited ex-U.S. net product revenues due to the ongoing reimbursement discussions in many ex-U.S. countries and have experienced challenges in the commercialization of ORKAMBI both in the United States and in ex-U.S. markets. Our ORKAMBI U.S. revenues have been affected by uptake, discontinuations and compliance rates. Our ORKAMBI ex-U.S. revenues have been affected by the same factors as our U.S. ORKAMBI revenues and challenges with respect to obtaining reimbursement for ORKAMBI in ex-U.S. markets. Factors that affect our ORKAMBI net product revenues include:

- the rate at which patients initiate treatment of ORKAMBI, the proportion of initiated patients who remain on treatment and the compliance rate for patients who remain on treatment;
- the safety and efficacy profile of ORKAMBI;
- our ability to obtain reimbursement for ORKAMBI and any changes in reimbursement policies of payors and other third parties; and
- legal, administrative, regulatory or legislative developments, including pricing limitations.

Since the regulations that govern pricing, coverage and reimbursement for drugs vary widely from country to country, there is no assurance that coverage and reimbursement will be available outside of the United States and, even if it is available, the timing or the level of reimbursement may not be satisfactory. Adverse pricing limitations or a delay in obtaining coverage and reimbursement would decrease our future net product revenues and harm our business.

If we continue to experience challenges with the commercialization of ORKAMBI or are unable to sustain KALYDECO net product revenues or if either medicine were to become subject to problems such as safety or efficacy issues, the introduction or greater acceptance of competing products, changes in reimbursement policies of payors and other third parties, or adverse legal, administrative, regulatory or legislative developments, our ability commercialization of our products would be impaired and our stock price would likely decline.

Our business depends on the success of tezacaftor in combination with ivacaftor, which has not been approved by the FDA or the European Commission. If we are unable to obtain marketing approval or experience material delays in obtaining marketing approval for, or reimbursement arrangements relating to, tezacaftor in combination with ivacaftor, our business could be materially harmed.

In the first quarter of 2017, we obtained positive results from two Phase 3 clinical trials of tezacaftor in combination with ivacaftor that showed statistically significant improvements in lung function in patients with CF 12 years of age

and older who have certain mutations in their CFTR gene. Based on these results, we submitted an NDA in the United States and

an MAA in Europe for this potential combination regimen. Obtaining approval of an NDA or an MAA is a lengthy, expensive and uncertain process, and we may not be successful. Obtaining approval depends on many factors including:

- whether or not the FDA and European regulatory authorities determine that the evidence gathered in well-controlled clinical trials, other clinical trials and nonclinical studies demonstrates that the combination regimen is safe; and
- whether or not the FDA and European regulatory authorities are satisfied that the manufacturing facilities, processes and controls for the combination are adequate, that the labeling is satisfactory and that plans for post-marketing studies, safety monitoring and risk evaluation and mitigation are sufficient.

Obtaining marketing approval for the combination of tezacaftor and ivacaftor in one country or region does not ensure that we will be able to obtain marketing approval in any other country or region.

Even if a tezacaftor in combination with ivacaftor is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming clinical trials or reporting as conditions of approval. If we experience material delays in obtaining marketing approval for the combination of tezacaftor and ivacaftor in either the United States or Europe, our future net product revenues and cash flows will be adversely effected. If we do not obtain approval to market the combination of tezacaftor and ivacaftor in the United States or Europe, our business will be materially harmed. Additionally, even if the combination of tezacaftor and ivacaftor receives marketing approval, coverage and reimbursement may not be available and, even if it is available, the level of reimbursement may not be satisfactory.

We may not realize the anticipated benefits of our acquisition of CTP-656 from Concert Pharmaceuticals, Inc.

In July 2017, we acquired certain CF assets from Concert Pharmaceuticals, Inc., or Concert, including CTP-656, an investigational CFTR potentiator that has the potential to be used as part of future once-daily combination regimens of CFTR modulators that treat the underlying cause of CF. Acquisitions are inherently risky and we may not realize the anticipated benefits of such transaction, which involves numerous risks including:

- that we fail to successfully develop and/or integrate CTP-656 into our pipeline in order to achieve our strategic objectives;
- that we receive inadequate or unfavorable data from clinical trials evaluating the CTP-656 in combination with other CFTR modulators; and
- the potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of CTP-656 or any of the other assets acquired from Concert, including but not limited to, problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, safety, and other known and unknown liabilities.

#### 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 net product revenues from KALYDECO and ORKAMBI;
- 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 our products and drug candidates, including the NDA and MAA submission for tezacaftor in combination with ivacaftor and the ongoing and planned clinical trials to evaluate our next-generation CFTR correctors;
- our ability to successfully market KALYDECO and ORKAMBI or any of our other drug candidates for which we obtain regulatory approval;





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, 2016, which was filed with the SEC on February 23, 2017. 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 June 30, 2017:

Period	Total Number of Shares Purchased	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	Maximum Number of Shares that May Yet be Purchased Under the Plans or Programs
April 1, 2017 to April 30, 2017	22,818	\$0.01	—	—
May 1, 2017 to May 31, 2017	28,883	\$0.01	—	—
June 1, 2017 to June 30, 2017	5,972	\$0.01	—	—

30,  
 2017  
 Total 57,673 \$0.01 — —

The repurchases were made under the terms of our Amended and Restated 2006 Stock and Option Plan and our Amended and Restated 2013 Stock and Option Plan. Under these plans, we award shares of restricted stock to our employees that typically are subject to a lapsing right of repurchase by us. We may exercise this right of repurchase if a restricted stock recipient's service to us is terminated. If we exercise this right, we are required to repay the purchase price paid by or on behalf of the recipient for the repurchased restricted shares, which typically is the par value per share of \$0.01. Repurchased

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shares are returned and are available for future awards under the terms of our Amended and Restated 2013 Stock and Option Plan.

Item 6. Exhibits

Exhibit Number	Exhibit Description
3.1	Restated Articles of Organization of Vertex Pharmaceuticals Incorporated, as amended.
3.2	Amended and Restated By-Laws of Vertex Pharmaceuticals Incorporated, as subsequently amended on April 26, 2016 and June 8, 2017.
10.1	Amended and Restated 2013 Stock and Option Plan. (1) *
31.1	Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.
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

(1) Incorporated by reference to Appendix C to the Registrant's definitive proxy statement on Schedule 14A, filed with the Securities and Exchange Commission on April 28, 2017.

\* Management contract, compensatory plan or agreement.

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

July 28, 2017      By:    /s/ Ian F. Smith  
                              Ian F. Smith  
                              Executive Vice President, Chief Operating Officer and Chief Financial  
                              Officer  
                              (principal financial officer and  
                              duly authorized officer)