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VERTEX PHARMACEUTICALS INCORPORATED  
 FORM 10-Q  
 FOR THE QUARTER ENDED SEPTEMBER 30, 2015

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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 September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Revenues:				
Product revenues, net	\$302,511	\$137,099	\$593,774	\$362,879
Royalty revenues	5,759	8,386	17,628	32,134
Collaborative revenues	1,546	33,502	2,999	40,846
Total revenues	309,816	178,987	614,401	435,859
Costs and expenses:				
Cost of product revenues	30,269	10,208	55,059	28,435
Royalty expenses	1,691	3,976	6,068	18,525
Research and development expenses	246,284	190,939	685,741	654,043
Sales, general and administrative expenses	99,772	75,224	280,026	226,882
Restructuring expenses, net	1,826	40,843	682	46,761
Total costs and expenses	379,842	321,190	1,027,576	974,646
Loss from operations	(70,026 )	(142,203 )	(413,175 )	(538,787 )
Interest expense, net	(21,134 )	(20,384 )	(63,552 )	(51,686 )
Other (expenses) income, net	(1,326 )	(3,990 )	(5,025 )	34,192
Loss from continuing operations before provision for income taxes	(92,486 )	(166,577 )	(481,752 )	(556,281 )
Provision for income taxes	1,330	3,419	31,760	4,915
Loss from continuing operations	(93,816 )	(169,996 )	(513,512 )	(561,196 )
Loss from discontinued operations, net of tax benefit of \$0	—	(64 )	—	(703 )
Net loss	(93,816 )	(170,060 )	(513,512 )	(561,899 )
(Income) loss attributable to noncontrolling interest	(1,333 )	—	30,909	—
Net loss attributable to Vertex	\$(95,149 )	\$(170,060 )	\$(482,603 )	\$(561,899 )
Amounts attributable to Vertex:				
Loss from continuing operations	(95,149 )	(169,996 )	(482,603 )	(561,196 )
Loss from discontinued operations	—	(64 )	—	(703 )
Net loss attributable to Vertex	(95,149 )	(170,060 )	(482,603 )	(561,899 )
Amounts per share attributable to Vertex common shareholders:				
Net loss from continuing operations:				
Basic	\$(0.39 )	\$(0.72 )	\$(2.00 )	\$(2.40 )
Diluted	\$(0.39 )	\$(0.72 )	\$(2.00 )	\$(2.40 )
Net loss from discontinued operations:				
Basic	\$—	\$—	\$—	\$—
Diluted	\$—	\$—	\$—	\$—
Net loss:				
Basic	\$(0.39 )	\$(0.72 )	\$(2.00 )	\$(2.40 )
Diluted	\$(0.39 )	\$(0.72 )	\$(2.00 )	\$(2.40 )
Shares used in per share calculations:				

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Basic	241,969	236,137	240,749	234,207
Diluted	241,969	236,137	240,749	234,207

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 Loss  
(unaudited)  
(in thousands)

	Three Months Ended		Nine Months Ended	
	September 30,		September 30,	
	2015	2014	2015	2014
Net loss	\$(93,816 )	\$(170,060 )	\$(513,512 )	\$(561,899 )
Changes in other comprehensive loss:				
Unrealized holding gains (losses) on marketable securities	56	(30 )	186	25
Unrealized gains on foreign currency forward contracts	4,546	1,838	572	1,713
Foreign currency translation adjustment	(1,384 )	(624 )	(164 )	(271 )
Total changes in other comprehensive loss	3,218	1,184	594	1,467
Comprehensive loss	(90,598 )	(168,876 )	(512,918 )	(560,432 )
Comprehensive (income) loss attributable to noncontrolling interest	(1,333 )	—	30,909	—
Comprehensive loss attributable to Vertex	\$(91,931 )	\$(168,876 )	\$(482,009 )	\$(560,432 )

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)

	September 30, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	\$756,250	\$625,259
Marketable securities, available for sale	249,580	761,847
Restricted cash and cash equivalents (VIE)	75,765	8,418
Accounts receivable, net	165,272	75,964
Inventories	49,197	30,848
Prepaid expenses and other current assets	63,388	44,175
Total current assets	1,359,452	1,546,511
Property and equipment, net	706,670	715,812
Intangible assets	284,340	29,000
Goodwill	50,384	39,915
Restricted cash	22,086	176
Other assets	11,061	3,265
Total assets	\$2,433,993	\$2,334,679
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$78,899	\$71,194
Accrued expenses	249,698	209,676
Deferred revenues, current portion	14,000	17,468
Accrued restructuring expenses, current portion	8,682	33,107
Capital lease obligations, current portion	14,153	17,806
Senior secured term loan, current portion	—	14,206
Other liabilities, current portion	9,527	4,797
Total current liabilities	374,959	368,254
Deferred revenues, excluding current portion	17,501	27,808
Accrued restructuring expenses, excluding current portion	9,122	12,748
Capital lease obligations, excluding current portion	39,273	39,293
Deferred tax liability	113,860	15,044
Fan Pier lease obligation, excluding current portion	472,724	473,073
Senior secured term loan, excluding current portion	294,832	280,569
Other liabilities, excluding current portion	34,990	21,707
Total liabilities	1,357,261	1,238,496
Commitments and contingencies		
Shareholders' equity:		
Preferred stock, \$0.01 par value; 1,000,000 shares authorized; none issued and outstanding at September 30, 2015 and December 31, 2014	—	—
Common stock, \$0.01 par value; 500,000,000 and 300,000,000 shares authorized at September 30, 2015 and December 31, 2014, respectively; 245,646,269 and 241,764,398 shares issued and outstanding at September 30, 2015 and December 31, 2014, respectively	2,419	2,385
Additional paid-in capital	6,106,270	5,777,154
Accumulated other comprehensive income	1,511	917

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Accumulated deficit	(5,188,053 )	(4,705,450 )
Total Vertex shareholders' equity	922,147	1,075,006
Noncontrolling interest	154,585	21,177
Total shareholders' equity	1,076,732	1,096,183
Total liabilities and shareholders' equity	\$2,433,993	\$2,334,679

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



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## VERTEX PHARMACEUTICALS INCORPORATED

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

(unaudited)

(in thousands)

	Common Stock		Additional Paid-in Capital	Accumulated		Total Vertex Shareholders' Equity	Noncontrolling Interest	Total Shareholders' Equity
	Shares	Amount		Other Comprehensive Income (Loss)	Accumulated Deficit			
Balance at December 31, 2013	233,789	\$2,320	\$5,321,286	\$ (306 )	\$(3,966,895)	\$ 1,356,405	\$ —	\$ 1,356,405
Other comprehensive income, net of tax	—	—	—	1,467	—	1,467	—	1,467
Net loss	—	—	—	—	(561,899 )	(561,899 )	—	(561,899 )
Issuance of common stock under benefit plans	6,449	55	223,812	—	—	223,867	—	223,867
Stock-based compensation	—	—	136,065	—	—	136,065	—	136,065
Balance at September 30, 2014	240,238	\$2,375	\$5,681,163	\$ 1,161	\$(4,528,794)	\$ 1,155,905	\$ —	\$ 1,155,905
Balance at December 31, 2014	241,764	\$2,385	\$5,777,154	\$ 917	\$(4,705,450)	\$ 1,075,006	\$ 21,177	\$ 1,096,183
Other comprehensive loss, net of tax	—	—	—	594	—	594	—	594
Net loss	—	—	—	—	(482,603 )	(482,603 )	(30,909 )	(513,512 )
Issuance of common stock under benefit plans	3,882	34	139,419	—	—	139,453	—	139,453
Stock-based compensation	—	—	189,697	—	—	189,697	—	189,697
Noncontrolling interest upon consolidation	—	—	—	—	—	—	164,317	164,317
Balance at September 30, 2015	245,646	\$2,419	\$6,106,270	\$ 1,511	\$(5,188,053)	\$ 922,147	\$ 154,585	\$ 1,076,732

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)

	Nine Months Ended September 30,	
	2015	2014
Cash flows from operating activities:		
Net loss	\$(513,512	) \$(561,899 )
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization expense	46,596	46,921
Stock-based compensation expense	186,379	135,160
Deferred income taxes	7,793	—
Impairment of property and equipment	23	978
Other non-cash items, net	(2,899	) 7
Changes in operating assets and liabilities:		
Accounts receivable, net	(88,735	) (7,315 )
Inventories	(16,127	) (4,901 )
Prepaid expenses and other assets	(23,737	) (19,110 )
Accounts payable	6,283	(5,544 )
Accrued expenses and other liabilities	90,320	12,210
Accrued restructuring expense	(28,051	) 27,249
Deferred revenues	(13,751	) (15,085 )
Net cash used in operating activities	(349,418	) (391,329 )
Cash flows from investing activities:		
Purchases of marketable securities	(292,135	) (1,066,772 )
Maturities of marketable securities	804,588	1,203,400
Payment for acquisition of variable interest entity	(80,000	) —
Expenditures for property and equipment	(32,775	) (36,525 )
(Increase) decrease in restricted cash and cash equivalents	(21,980	) 9
Decrease in restricted cash and cash equivalents (VIE)	14,830	—
Decrease (increase) in other assets	(982	) (92 )
Net cash provided by investing activities	391,546	100,020
Cash flows from financing activities:		
Issuances of common stock under benefit plans	139,689	201,274
Payments on capital lease obligations	(16,515	) (17,215 )
Proceeds from capital lease financing	13,386	—
Payments on Fan Pier lease obligation	(45,438	) (45,438 )
Proceeds from senior secured term loan	—	294,383
Payments returned related to Fan Pier lease obligation	—	8,050
Net cash provided by financing activities	91,122	441,054
Effect of changes in exchange rates on cash	(2,259	) (481 )
Net increase in cash and cash equivalents	130,991	149,264
Cash and cash equivalents—beginning of period	625,259	569,299
Cash and cash equivalents—end of period	\$756,250	\$718,563
Supplemental disclosure of cash flow information:		
Cash paid for interest	\$19,074	\$2,817
Cash paid for income taxes	\$1,261	\$798

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Capitalization of costs related to Fan Pier lease obligation	\$—	\$25,564
Assets acquired under capital lease	\$—	\$8,985
Issuances of common stock exercises from employee benefit plans receivable	\$(236	) \$23,035

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

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VERTEX PHARMACEUTICALS INCORPORATED  
Notes to Condensed Consolidated Financial Statements  
(unaudited)

A. Basis of Presentation and Accounting Policies

Basis of Presentation

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

The condensed consolidated financial statements reflect the operations of (i) the Company, (ii) its wholly-owned subsidiaries and (iii) consolidated variable interest entities (VIEs). All material intercompany balances and transactions have been eliminated. The Company operates in one segment, pharmaceuticals.

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 September 30, 2015 and 2014.

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, 2014, which are contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2014 that was filed with the Securities and Exchange Commission (the "SEC") on February 13, 2015 (the "2014 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, 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 2014 Annual Report on Form 10-K.

Recent Accounting Pronouncements

For a discussion of recent accounting pronouncements please refer to Note A, "Nature of Business and Accounting Policies—Recent Accounting Pronouncements," in the 2014 Annual Report on Form 10-K. The Company did not adopt any new accounting pronouncements during the nine months ended September 30, 2015 that had a material effect on its condensed consolidated financial statements.

B. Product Revenues, Net

The Company sells its products principally to a limited number of specialty pharmacy providers and selected regional wholesalers 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.



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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 Customer's 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 cost 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.

The following table summarizes activity in each of the product revenue allowance and reserve categories for the nine months ended September 30, 2015:

	Trade Allowances	Rebates, Chargebacks and Discounts	Product Returns	Other Incentives	Total
	(in thousands)				
Balance at December 31, 2014	\$1,463	\$29,102	\$4,713	\$745	\$36,023
Provision related to current period sales	6,120	39,533	429	2,402	48,484
Adjustments related to prior period sales	(143 )	(12,342 )	(993 )	(235 )	(13,713 )
Credits/payments made	(5,115 )	(22,465 )	(3,254 )	(1,652 )	(32,486 )
Balance at September 30, 2015	\$2,325	\$33,828	\$895	\$1,260	\$38,308

### C. Collaborative Arrangements

#### Cystic Fibrosis Foundation Therapeutics Incorporated

In April 2011, the Company entered into an amendment (the "April 2011 Amendment") to its existing collaboration agreement with Cystic Fibrosis Foundation Therapeutics Incorporated ("CFFT") pursuant to which CFFT agreed to provide financial support for (i) development activities for VX-661, a compound that targets the processing and trafficking defect of the F508del CFTR proteins discovered under the collaboration, and (ii) additional research and development activities directed at discovering new compounds targeting the processing and trafficking defect of the F508del protein.

Under the April 2011 Amendment, CFFT agreed to provide the Company with up to \$75.0 million in funding over approximately five years for research and development activities. The Company retains the right to develop and commercialize KALYDECO (ivacaftor), ORKAMBI (lumacaftor in combination with ivacaftor), lumacaftor, VX-661 and any other compounds discovered during the course of the research collaboration with CFFT. The Company recognized no collaborative revenues from this collaboration during the three and nine months ended September 30, 2015 and \$2.0 million and \$6.5 million of collaborative revenues from this collaboration during the three and nine months ended September 30, 2014, respectively.

In the original agreement, as amended prior to the April 2011 Amendment, the Company agreed to pay CFFT tiered royalties calculated as a percentage, ranging from single digits to sub-teens, of annual net sales of any approved drugs discovered during the research term that ended in 2008, including KALYDECO, ORKAMBI, lumacaftor and VX-661. The April 2011 Amendment provides for a tiered royalty in the same range on net sales of compounds targeting the processing and trafficking defect of F508del CFTR proteins discovered during the research term that began in 2011 and ended in February 2014. In each of the third quarter of 2012 and the first quarter of 2013, CFFT earned a commercial milestone payment of \$9.3 million from the Company upon achievement of certain sales levels for KALYDECO. These milestones were reflected in the Company's cost of product revenues. There are no additional

commercial milestone payments payable by the Company to CFFT related to sales levels for KALYDECO. The Company also is obligated to make up to two one-time commercial milestone payments to CFFT upon achievement of certain sales levels for ORKAMBI.



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The Company began marketing KALYDECO in the United States and certain countries in the European Union in 2012 and began marketing ORKAMBI in the United States in July 2015. The Company has royalty obligations to CFRT for each compound commercialized pursuant to this collaboration until the expiration of patents covering that compound. 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 life 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 patent life extensions. CFRT may terminate its funding obligations under the collaboration, as amended, in certain circumstances, in which case there will be a proportional adjustment to the royalty rates and commercial milestone payments for certain compounds. The collaboration also may be terminated by either party for a material breach by the other, subject to notice and cure provisions.

**Janssen Pharmaceutica NV**

The Company has a collaboration agreement (the "Janssen HCV Agreement") with Janssen Pharmaceutica NV ("Janssen NV") for the development, manufacture and commercialization of telaprevir, which Janssen NV began marketing under the brand name INCIVO in certain of its territories in September 2011. Pursuant to the Janssen HCV Agreement, as amended, Janssen NV has a fully-paid license to manufacture and commercialize INCIVO in its territories including Europe, South America, the Middle East, Africa and Australia, subject to the payment of third-party royalties on net sales of INCIVO. In addition to the collaborative revenues, the Company recorded royalty revenues and corresponding royalty expenses related to third-party royalties that Janssen NV remains responsible for based on INCIVO net sales.

During the three and nine months ended September 30, 2015 and 2014, the Company recognized the following revenues attributable to the Janssen NV collaboration:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
	(in thousands)			
Royalty revenues (INCIVO)	\$ 158	\$ 2,284	\$ 1,742	\$ 12,917
Collaborative revenues (telaprevir)	441	1,390	1,620	4,262
Total revenues attributable to the Janssen NV collaboration	\$ 599	\$ 3,674	\$ 3,362	\$ 17,179

**CRISPR Therapeutics AG**

On October 26, 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. In connection with the CRISPR Agreement, the Company made an upfront payment to CRISPR of \$75.0 million and will make a \$30.0 million investment in CRISPR pursuant to a convertible loan agreement.

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 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 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 such point. 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.



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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 milestone and royalty 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 time to develop the drug candidates, 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 milestone and royalty payments. The following collaborations are, or were previously, reflected in the Company's financial statements for being consolidated as VIEs:

Parion Sciences, Inc.

License and Collaboration Agreement

On June 4, 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 P-1055, for the potential treatment of cystic fibrosis, or CF, and other pulmonary diseases. The Company is leading development activities for VX-371 and P-1055 in CF and other pulmonary diseases 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 P-1055, 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 P-1055 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 such point. 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 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 Parion is a VIE based on, among other factors, the significance to Parion of the ENaC inhibitors licensed to the Company pursuant to the Parion Agreement and on the Company's power to direct the

activities that most significantly impact the economic performance of 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. In particular, the Company did not acquire any equity interest in Parion, any interest in Parion's cash and cash equivalents or any control over Parion's activities that do not relate to the Parion Agreement.

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Consideration for the Parion Agreement

The Company determined that the fair value of the consideration from the Company to Parion was \$255.3 million as of June 4, 2015, which consisted of (i) an \$80.0 million up-front payment, (ii) the estimated fair value of the contingent research and development milestones potentially payable by the Company to Parion and (iii) the estimated fair value of potential royalty payments payable by the Company to Parion. The Company valued the contingent milestone and royalty payments using (a) discount rates ranging from 4.1% to 5.9% for the development milestones and (b) a discount rate of 6.6% for royalties. The consideration paid and the preliminary fair value of the contingent milestone and royalty payments payable by the Company pursuant to the agreement are set forth in the table below:

	June 4, 2015 (in thousands)
Up-front payment	\$80,000
Fair value of contingent milestone and royalty payments	175,340
Total	\$255,340

Preliminary Allocation of Assets and Liabilities

For the purposes of the condensed consolidated balance sheets at June 4, 2015 and September 30, 2015, the Company preliminarily allocated the total consideration, which is comprised of the up-front payment and the fair value of the contingent milestone and royalty payments, intangible assets, goodwill, deferred tax liability, net and net other assets and liabilities.

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 P-1055, that are licensed by Parion to the Company. The difference between the preliminary fair value of the consideration and the fair value of Parion's assets (including the fair value of intangible assets) and liabilities was allocated to goodwill.

The following table summarizes the preliminary fair values of the assets and liabilities recorded on the effective date of the agreement:

	June 4, 2015 (in thousands)
Intangible assets	\$255,340
Goodwill	10,468
Deferred tax liability	(91,023 )
Net other assets (liabilities)	(10,468 )
Net assets attributable to noncontrolling interests BioAxone Biosciences, Inc.	\$164,317

In October 2014, the Company entered into a license and collaboration agreement (the "BioAxone Agreement") with BioAxone Biosciences, Inc. ("BioAxone"), a privately-held biotechnology company, 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 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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Alios BioPharma, Inc.

In 2011, the Company entered into a license and collaboration agreement (the “Alios Agreement”) with Alios BioPharma, Inc. (“Alios”), which was a privately-held biotechnology company, which resulted in the consolidation of Alios as a VIE through December 31, 2013. Pursuant to the Alios Agreement, the Company and Alios collaborated on the research, development and commercialization of HCV nucleotide analogues discovered by Alios through April 2014. In December 2014, the Alios Agreement terminated in accordance with its terms pursuant to a termination notice delivered by the Company in October 2014. As of September 30, 2014, the Company concluded that it no longer had significant continuing involvement with Alios due to its intent and ability to terminate the Alios Agreement, among other factors; therefore, the operations of Alios are presented as discontinued operations in these condensed consolidated financial statements.

Aggregate VIE Financial Information

The Company did not have any consolidated VIEs for the three and nine months ended September 30, 2014. An aggregate summary of net loss attributable to noncontrolling interest related to the Company's VIEs for the three and nine months ended September 30, 2015 is as follows:

	Three Months Ended September 30, 2015 (in thousands)	Nine Months Ended September 30, 2015	
Loss attributable to noncontrolling interest before provision for income taxes	\$(1,743	)	\$(3,322
Provision for income taxes	(777	)	(30,367
Increase in fair value of contingent milestone and royalty payments	3,853		2,780
Net (income) loss attributable to noncontrolling interest	\$1,333		\$ (30,909

During the three and nine months ended September 30, 2015, the fair value of the contingent milestone and royalty payments related to the BioAxeone collaboration decreased by \$0.6 million and \$0.1 million, respectively. During the three and nine months ended September 30, 2015, the fair value of the contingent milestone and royalty payments related to the Parion collaboration increased by \$4.5 million and \$2.9 million, respectively. The changes in the fair value of the contingent milestone and royalty payments were primarily due to the changes in market interest rates and the time value of money. As of September 30, 2015, the fair value of the contingent milestone and royalty payments related to the BioAxeone collaboration and the Parion collaboration was \$27.0 million and \$178.2 million, respectively.

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:

	September 30, 2015 (in thousands)	December 31, 2014
Restricted cash and cash equivalents (VIE)	\$75,765	\$8,418
Prepaid expenses and other current assets	3,057	268
Intangible assets	284,340	29,000
Goodwill	19,391	8,923
Other assets	461	42
Accounts payable	1,115	189
Accrued expenses and other current liabilities	32,519	3,891
Deferred tax liability, net	110,360	11,544
Other liabilities	300	300
Noncontrolling interest	154,585	21,177

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

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that the Company licensed from the applicable VIE. Assets recorded as a result of consolidating our 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.

**Outlicense Arrangements**

In the ordinary course of the Company's business, the Company has entered into various agreements pursuant to which it has outlicensed rights to certain drug candidates to third-party collaborators. Although the Company does not consider any of these outlicense arrangements to be material, the most notable of these outlicense arrangements is described below. Pursuant to these outlicense arrangements, our collaborators are responsible for all costs related to the continued development of such drug candidates. Depending on the terms of the arrangements, the Company's collaborators may be required to make upfront payments, milestone payments upon the achievement of certain product research and development objectives and/or pay royalties on future sales, if any, of commercial products resulting from the collaboration.

**Janssen Pharmaceuticals, Inc.**

In June 2014, the Company entered into an agreement (the "Janssen Influenza 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 Influenza Agreement, Janssen Inc. has an exclusive worldwide license to develop and commercialize certain drug candidates for the treatment of influenza, including 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. is responsible for costs related to the development and commercialization of the compounds. During the three and nine months ended September 30, 2015, the Company recorded reimbursement for these development activities of \$4.0 million and \$18.7 million, respectively. During the three and nine months ended September 30, 2014, the Company recorded reimbursement for these development activities of \$4.3 million. 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. Janssen Inc. may terminate the Janssen Influenza Agreement, subject to certain exceptions, upon six months' notice.

**D. Earnings Per Share**

Basic net 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 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.

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

	Three Months Ended September 30, 2015		Nine Months Ended September 30, 2015	
	2014	2014	2014	2014
	(in thousands)			
Stock options	12,025	13,097	12,025	13,097
Unvested restricted stock and restricted stock units	3,367	2,672	3,367	2,672

**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:

- Level 1: Quoted prices in active markets for identical assets or liabilities. An active market for an asset or liability is a market in which transactions for the asset or liability occur with sufficient frequency and volume to provide pricing information on an ongoing basis.
- Level 2: Observable inputs other than Level 1 inputs. Examples of Level 2 inputs include quoted prices in active markets for similar assets or liabilities and quoted prices for identical assets or liabilities in markets that are not active.
- Level 3: Unobservable inputs based on the Company's assessment of the assumptions that market participants would 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 September 30, 2015, the Company's investments were in money market funds, corporate debt securities and commercial paper.

As of September 30, 2015, 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. The Company's financial assets valued based on Level 2 inputs consisted of corporate debt securities and commercial paper, which consist of investments in highly-rated investment-grade corporations.

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The following table sets forth the Company's financial assets and liabilities subject to fair value measurements:

	Fair Value Measurements as of September 30, 2015			
	Total	Fair Value Hierarchy		
		Level 1	Level 2	Level 3
	(in thousands)			
Financial assets carried at fair value:				
Cash equivalents:				
Money market funds	\$340,573	\$340,573	\$—	\$—
Corporate debt securities	25,109	—	25,109	—
Commercial paper	24,000	—	24,000	—
Marketable securities:				
Corporate debt securities	109,335	—	109,335	—
Commercial paper	140,245	—	140,245	—
Prepaid and other current assets:				
Foreign currency forward contracts	3,508	—	3,508	—
Other assets:				
Foreign currency forward contracts	651	—	651	—
Total financial assets	\$643,421	\$340,573	\$302,848	\$—
Financial liabilities carried at fair value:				
Other liabilities, current portion:				
Foreign currency forward contracts	\$(1,552 )	\$—	\$(1,552 )	\$—
Other liabilities, excluding current portion:				
Foreign currency forward contracts	(24 )	—	(24 )	—
Total financial liabilities	\$(1,576 )	\$—	\$(1,576 )	\$—

The Company's VIEs invested in cash equivalents consisting of money market funds of \$75.4 million as of September 30, 2015, 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 milestone and royalty payments, which are valued based on Level 3 inputs. Please refer to Note C, "Collaborative Arrangements," for further information.

As of September 30, 2015, the fair value and carrying value of the Company's Term Loan was \$294.8 million. The fair value of the Company's Term Loan was estimated based on Level 3 inputs computed using the effective interest rate of the Term Loan. The effective interest rate considers the timing and amount of estimated future interest payments as well as current markets rates. Please refer to Note K, "Long-term Obligations" for further information regarding the Company's Term Loan.

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## 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 September 30, 2015				
Cash and cash equivalents:				
Cash and money market funds	\$707,141	\$—	\$—	\$707,141
Commercial paper	\$25,109	\$—	\$—	\$25,109
Corporate debt securities	\$24,000	\$—	\$—	\$24,000
Total cash and cash equivalents	\$756,250	\$—	\$—	\$756,250
Marketable securities:				
Commercial paper (due within 1 year)	\$140,131	\$114	\$—	\$140,245
Corporate debt securities (due within 1 year)	109,386	28	(79	) 109,335
Total marketable securities	\$249,517	\$142	\$(79	) \$249,580
Total cash, cash equivalents and marketable securities	\$1,005,767	\$142	\$(79	) \$1,005,830
As of December 31, 2014				
Cash and cash equivalents:				
Cash and money market funds	\$625,259	\$—	\$—	\$625,259
Total cash and cash equivalents	\$625,259	\$—	\$—	\$625,259
Marketable securities:				
Government-sponsored enterprise securities (due within 1 year)	\$463,788	\$14	\$(52	) \$463,750
Commercial paper (due within 1 year)	51,674	72	—	51,746
Corporate debt securities (due within 1 year)	196,065	2	(66	) 196,001
Corporate debt securities (due after 1 year through 5 years)	50,443	—	(93	) 50,350
Total marketable securities	\$761,970	\$88	\$(211	) \$761,847
Total cash, cash equivalents and marketable securities	\$1,387,229	\$88	\$(211	) \$1,387,106

The Company has a limited number of marketable securities in insignificant loss positions as of September 30, 2015, which the Company does not intend to sell and has concluded it will not be required to sell before recovery of the amortized costs for the 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 nine months ended September 30, 2015 and 2014.

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G. Accumulated Other Comprehensive (Loss)  
Income

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

	Foreign Currency Translation Adjustment	Unrealized Holding Gains (Losses) on Marketable Securities	Unrealized Gains (Losses) on Foreign Currency Forward Contracts	Total
	(in thousands)			
Balance at December 31, 2014	\$(971)	) \$(123	) \$2,011	\$917
Other comprehensive (loss) income before reclassifications	(164	) 186	4,072	4,094
Amounts reclassified from accumulated other comprehensive loss	—	—	(3,500	) (3,500
Net current period other comprehensive (loss) income	\$(164	) \$186	\$572	\$594
Balance at September 30, 2015	\$(1,135	) \$63	\$2,583	\$1,511
	(in thousands)			
	Foreign Currency Translation Adjustment	Unrealized Holding Gains on Marketable Securities	Unrealized (Losses) Gains on Foreign Currency Forward Contracts	Total
Balance at December 31, 2013	\$(325	) \$42	\$(23	) \$(306
Other comprehensive (loss) income before reclassifications	(271	) 25	2,140	1,894
Amounts reclassified from accumulated other comprehensive loss	—	—	(427	) (427
Net current period other comprehensive (loss) income	\$(271	) \$25	\$1,713	\$1,467
Balance at September 30, 2014	\$(596	) \$67	\$1,690	\$1,161

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 GAAP having contractual durations from one to eighteen months. To date, the existence of operational sites in countries outside the United States has limited the degree to which the Company has sought to hedge its revenues in certain foreign currencies. 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 September 30, 2015, all hedges were determined to be highly effective and the Company has 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:

	As of September 30, 2015 (in thousands)	As of December 31, 2014
Foreign Currency		
Euro	\$106,620	\$20,209
British pound sterling	78,861	13,515
Australian dollar	25,414	—
Total foreign currency forward contracts	\$210,895	\$33,724

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 September 30, 2015

Assets		Liabilities	
Classification	Fair Value	Classification	Fair Value
(in thousands)			
Prepaid and other current assets	\$3,508	Other liabilities, current portion	\$(1,552 )
Other assets	651	Other liabilities, excluding current portion	(24 )
Total assets	\$4,159	Total liabilities	\$(1,576 )

As of December 31, 2014

Assets		Liabilities	
Classification	Fair Value	Classification	Fair Value
(in thousands)			
Prepaid and other current assets	\$2,011	Other liabilities, current portion	\$—
Total assets	\$2,011	Total liabilities	\$—

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

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	As of September 30, 2015				
	Gross Amounts Recognized (in thousands)	Gross Amounts Offset	Gross Amount Presented	Gross Amount Not Offset	Legal Offset
Foreign currency forward contracts					
Total assets	\$4,159	\$—	\$4,159	\$(1,576)	\$2,583
Total liabilities	\$(1,576)	\$—	\$(1,576)	\$1,576	\$—
	As of December 31, 2014				
	Gross Amounts Recognized (in thousands)	Gross Amounts Offset	Gross Amount Presented	Gross Amount Not Offset	Legal Offset
Foreign currency forward contracts					
Total assets	\$2,011	\$—	\$2,011	\$—	\$2,011

## I. Inventories

Inventories consisted of the following:

	As of September 30, 2015 (in thousands)	As of December 31, 2014
Raw materials	\$8,669	\$8,506
Work-in-process	34,115	20,508
Finished goods	6,413	1,834
Total	\$49,197	\$30,848

## J. Intangible Assets and Goodwill

## Intangible Assets

As of September 30, 2015, in-process research and development intangible assets of \$284.3 million were recorded on the Company's condensed consolidated balance sheet. The increase of \$255.3 million as compared to the \$29.0 million recorded as of December 31, 2014 is due to the Company's collaboration with Parion.

In June 2015, in connection with entering into the Parion Agreement, the Company recorded an in-process research and development intangible asset of \$255.3 million based on the Company's estimate of the fair value of Parion's lead investigational ENaC inhibitors, including VX-371 and P-1055, that were licensed by the Company from Parion. The Company aggregated the fair value of the ENaC inhibitors into a single intangible asset because the phase, nature and risks of development as well as the amount and timing of benefits associated with the assets were similar. In October 2014, the Company recorded an in-process research and development intangible asset of \$29.0 million based on the Company's estimate of the fair value of VX-210, a drug candidate for patients with spinal cord injuries that was licensed by the Company from BioAxone. The Company used discount rates of 7.1% and 7.5% in the present-value models to estimate the fair values of the ENaC inhibitors and VX-210 intangible assets, respectively.

The Company also conducted an evaluation of Parion and BioAxone's other programs at the effective date of the Parion Agreement and BioAxone Agreement, respectively, and determined that market participants would not have ascribed value to those programs because of the stage of development of the assets in each program and uncertainties related to the potential clinical development and commercialization of the programs.

## Goodwill

As of September 30, 2015, goodwill of \$50.4 million was recorded on the Company's condensed consolidated balance sheet. The Company allocated \$10.5 million to goodwill related to the Parion Agreement during the nine months ended September 30, 2015. This goodwill relates to the potential synergies between licensed drug candidates and the Company's CF drugs and drug candidates. None of the goodwill related to the Parion Agreement is expected to be deductible for income tax purposes. As of December 31, 2014, \$39.9 million of goodwill was recorded on the Company's 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 “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 and determined that the Fan Pier Leases did not meet the criteria for “sale-leaseback” treatment upon completion of the Buildings, the Company recorded project construction costs



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incurred by the landlord as an asset and a related financing obligation during the construction period and 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 Buildings were constructed. The portion of the lease obligations allocated to the land is treated as an operating lease that commenced in 2011.

Property and equipment, net, included \$505.6 million and \$515.0 million as of September 30, 2015 and December 31, 2014, respectively, related to construction costs for the Buildings. The carrying value of the Company's lease agreement liability for the Buildings was \$473.1 million and \$473.4 million as of September 30, 2015 and December 31, 2014, respectively.

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 provides for a \$300.0 million senior secured term loan ("Term Loan"). The credit agreement also provides that, subject to satisfaction of certain conditions, the Company may request that the lenders establish an incremental senior secured term loan facility in an aggregate amount not to exceed \$200.0 million. The Term Loan initially bore interest at a rate of 7.2% per annum, which will be reduced to 6.2% per annum based on the FDA's approval of ORKAMBI. The Term Loan will bear interest at a rate of LIBOR plus 5.0% per annum during the third year of the term.

The maturity date of all loans under the facilities is July 9, 2017. Interest is payable quarterly and on the maturity date. In October 2015, the Company amended the terms of the credit agreement to provide for, among other things, a modification to the repayment schedule of the loan. As amended, the Company is required to repay principal on the Term Loan in quarterly installments of \$75 million from October 1, 2016 through the maturity date. The Company may prepay the Term Loan, in whole or in part, at any time; provided that prepayments prior to the July 9, 2016 are subject to a make-whole premium to ensure Macquarie receives approximately the present value of two years of interest payments over the life of the loan. The Company expects that amending the credit agreement will be accounted for as a debt modification, as opposed to an extinguishment of debt, based on an insignificant change to the present value of the future cash flows relating to the credit agreement.

The Company's obligations under the facilities are unconditionally guaranteed by certain of its domestic subsidiaries. All obligations under the facilities, and the guarantees of those obligations, are secured, subject to certain exceptions, by substantially all of the Company's assets and the assets of all guarantors, including the pledge of all or a portion of the equity interests of certain of its subsidiaries.

The credit agreement requires that the Company maintain, on a quarterly basis, a minimum level of KALYDECO net revenues. Further, the credit agreement includes negative covenants, subject to exceptions, restricting or limiting the Company's ability and the ability of its subsidiaries to, among other things, incur additional indebtedness, grant liens, engage in certain investment, acquisition and disposition transactions, pay dividends, repurchase capital stock and enter into transactions with affiliates. The credit agreement also contains customary representations and warranties, affirmative covenants and events of default, including payment defaults, breach of representations and warranties, covenant defaults and cross defaults. If an event of default occurs, the administrative agent would be entitled to take various actions, including the acceleration of amounts due under outstanding loans. There have been no events of default as of or during the period ended September 30, 2015.

Based on the Company's evaluation of the Term Loan, the Company determined that the Term Loan contains several embedded derivatives. These embedded derivatives are clearly and closely related to the host instrument because they relate to the Company's credit risk; therefore, they do not require bifurcation from the host instrument, the Term Loan. The Company incurred \$5.3 million in fees paid to Macquarie that were recorded as a discount on the Term Loan and are being recorded as interest expense using the effective interest method over the term of the loan in the Company's condensed consolidated statements of operations. As of September 30, 2015, the unamortized discount associated with the Term Loan that was embedded in the senior secured term loan caption on the Company's condensed consolidated

balance sheet was \$5.2 million.

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L. Stock-based Compensation Expense

The Company issues stock options, restricted stock and restricted stock units with service conditions, which are generally the vesting periods of the awards. The Company also has issued, to certain members of senior management, restricted stock and restricted stock units that vest upon the earlier of the satisfaction of (i) a performance condition or (ii) a service condition and stock options that vest upon the earlier of the satisfaction of (a) performance conditions or (b) a service condition. In addition, the Company issued pursuant to a retention program restricted stock awards to certain members of senior management that will vest upon the satisfaction of both (i) a performance condition and (ii) a service condition. The Company also issues shares pursuant to an employee stock purchase plan ("ESPP").

In the second quarter of 2015, the Company's shareholders approved an amendment and restatement of the 2013 Stock and Option Plan that, among other things, increased the number of shares of common stock available for issuance under the plan by 7,800,000 shares, plus the number of shares that remained available for issuance under our 2006 Stock and Option Plan, which rolled-over into the 2013 Stock and Option Plan.

During the three and nine months ended September 30, 2015 and 2014, the Company recognized the following stock-based compensation expense included in loss from continuing operations:

	Three Months Ended September 30, 2015		Nine Months Ended September 30, 2015	
	2014	2014	2014	2014
	(in thousands)			
Stock-based compensation expense by type of award:				
Stock options	\$39,074	\$26,291	\$105,720	\$78,403
Restricted stock and restricted stock units	26,203	18,418	78,274	51,431
ESPP share issuances	1,738	1,883	5,703	6,231
Less stock-based compensation expense capitalized to inventories	(1,281)	(456)	(3,318)	(905)
Total stock-based compensation included in costs and expenses	\$65,734	\$46,136	\$186,379	\$135,160
Stock-based compensation expense by line item:				
Research and development expenses	\$44,701	\$31,131	\$124,550	\$91,284
Sales, general and administrative expenses	21,033	15,005	61,829	43,876
Total stock-based compensation included in costs and expenses	\$65,734	\$46,136	\$186,379	\$135,160

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

	As of September 30, 2015	
	Unrecognized Expense, Net of Estimated Forfeitures (in thousands)	Weighted-average Recognition Period (in years)
Type of award:		
Stock options	\$197,031	2.30
Restricted stock and restricted stock units	\$190,786	2.73
ESPP share issuances	\$1,885	0.44



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The following table summarizes information about stock options outstanding and exercisable at September 30, 2015:

Range of Exercise Prices	Options Outstanding			Options Exercisable	
	Number Outstanding (in thousands)	Weighted-average Remaining Contractual Life (in years)	Weighted-average Exercise Price (per share)	Number Exercisable (in thousands)	Weighted-average Exercise Price (per share)
\$18.93–\$20.00	138	2.35	\$18.93	138	\$18.93
\$20.01–\$40.00	2,499	3.81	\$34.56	2,284	\$34.36
\$40.01–\$60.00	2,593	6.80	\$48.27	1,458	\$49.52
\$60.01–\$80.00	1,567	8.31	\$75.94	551	\$74.95
\$80.01–\$100.00	1,932	8.28	\$90.27	638	\$87.16
\$100.01–\$120.00	1,791	9.32	\$109.24	201	\$109.22
\$120.01–\$134.69	1,505	9.78	\$131.21	168	\$127.52
Total	12,025	7.31	\$74.90	5,438	\$53.98

#### 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 September 30, 2015, the Company had \$31.2 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.

##### Other income (expense), net

In April 2014, the Company received a one-time cash payment of \$36.7 million from its landlord pursuant to the Fan Pier Leases. This payment related to bonds issued pursuant to an Infrastructure Development Assistance Agreement between The Commonwealth of Massachusetts and the Company's landlord. The bonds were issued in connection with the landlord's contribution to infrastructure improvements and also were dependent upon employment levels at the Company through the bond issuance date. The Company accounted for the cash payment as a government grant as it was provided in part related to the Company's employment level in Massachusetts. Such grants are recognized as income in the period in which the conditions of the grant are met and there is reasonable assurance that the grant will be received, provided it is not subject to refund. In the second quarter of 2014, the Company recorded \$36.7 million as a credit to other income (expense), net in its consolidated statements of operations because the Company's employment obligations related to these funds were satisfied as of the date of issuance of the bonds and the payment received was not subject to refund.

#### N. Income Taxes

The Company is subject to U.S. federal, state, and foreign income taxes. For the three and nine months ended September 30, 2015, the Company recorded a provision for income taxes of \$1.3 million and \$31.8 million, respectively. The provision for income taxes recorded in the three and nine months ended September 30, 2015 included \$0.8 million and \$30.4 million, respectively, related to the Company's VIE's income tax provision. The VIE's income tax provision for the nine months ended September 30, 2015 primarily related to the income tax effect on the \$80.0 million up-front payment received by Parion from the Company in June 2015. The Company has no liability for taxes payable by Parion and the income tax provision and related liability have been allocated to noncontrolling interest (VIE). For the three and nine months ended September 30,



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2014, the Company recorded a provision for income taxes of \$3.4 million and \$4.9 million, respectively, related to state income taxes and income earned in various foreign jurisdictions.

As of September 30, 2015 and December 31, 2014, the Company had unrecognized tax benefits of \$0.9 million. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of September 30, 2015, no interest and penalties have been accrued. The Company does not expect that its unrecognized tax benefits will materially increase within the next twelve months. The Company did not recognize any material interest or penalties related to uncertain tax positions as of September 30, 2015 and December 31, 2014. In 2015, it is reasonably possible that the Company will reduce the balance of its unrecognized tax benefits by approximately \$0.5 million due to the application of statute of limitations and settlements with taxing authorities, all of which would reduce the Company's effective tax rate.

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.

The Company files U.S. federal income tax returns and income tax returns in various state, local and foreign jurisdictions. The Company is no longer subject to any tax assessment from an income tax examination in the United States before 2011 or any other major taxing jurisdiction for years before 2009, except where the Company has net operating losses or tax credit carryforwards that originated before 2009. The Company currently is under examination by the Internal Revenue Service for the year ended December 31, 2011 and in Canada, Pennsylvania, Delaware, New York and Texas for varying periods including the years ended December 31, 2011 through 2013. No adjustments have been reported. The Company is not under examination by any other jurisdictions for any tax year. The Company concluded audits with Massachusetts and Revenue Quebec during 2015 and the Canada Revenue Agency and Revenue Quebec during 2014 with no material adjustments.

The Company currently intends to reinvest the total amount of its unremitted earnings. At September 30, 2015, 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 U.S. federal income taxes (subject to an adjustment for foreign tax credits) and withholding taxes payable to the various foreign countries.

#### O. Restructuring Liabilities

##### 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 nine months ended September 30, 2015 and 2014 were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
	(in thousands)			
Liability, beginning of the period	\$9,924	\$14,936	\$11,596	\$19,115
Cash payments	(3,975 )	(4,249 )	(10,544 )	(12,071 )
Cash received from subleases	2,919	2,689	8,194	8,067
Restructuring expense (income)	146	(464 )	(232 )	(2,199 )
Liability, end of the period	\$9,014	\$12,912	\$9,014	\$12,912





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**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 the first quarter of 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 nine months ended September 30, 2015 and 2014 were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
	(in thousands)			
Liability, beginning of the period	\$9,017	\$3,256	\$33,390	\$797
Cash payments	(3,070 )	(2,266 )	(25,421 )	(6,643 )
Cash received from subleases	1,820	—	1,820	—
Restructuring expense (income)	51	39,752	(1,971 )	46,588
Liability, end of the period	\$7,818	\$40,742	\$7,818	\$40,742

**Other Restructuring Activities**

The Company has incurred several other restructuring activities that are unrelated to its 2003 Kendall Restructuring and the Fan Pier Move Restructuring. In October 2013, 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 cystic fibrosis and other research and development programs.

The activities related to the Company's other restructuring liabilities for the three and nine months ended September 30, 2015 and 2014 were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
	(in thousands)			
Liability, beginning of the period	\$902	\$792	\$869	\$8,441
Cash payments	(1,559 )	(399 )	(2,782 )	(8,865 )
Restructuring expense	1,629	1,555	2,885	2,372
Liability, end of the period	\$972	\$1,948	\$972	\$1,948



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P. Commitments and Contingencies

Financing Arrangements

As of September 30, 2015, the Company had irrevocable stand-by letters of credit outstanding that were issued in connection with property leases and other similar agreements totaling \$21.9 million that are cash collateralized. The cash used to support these letters of credit is included in restricted cash, as of September 30, 2015, on the Company's condensed consolidated balance sheet.

Litigation

On May 28, 2014, a purported shareholder class action Local No. 8 IBEW Retirement Plan & Trust v. Vertex Pharmaceuticals Incorporated, et al. was filed in the United States District Court for the District of Massachusetts, naming the Company and certain of the Company's current and former officers and directors as defendants. The lawsuit alleged that the Company made material misrepresentations and/or omissions of material fact in the Company's disclosures during the period from May 7, 2012 through May 29, 2012, all in violation of Section 10(b) of the Securities Exchange Act of 1934, as amended, and Rule 10b-5 promulgated thereunder. The purported class consists of all persons (excluding defendants) who purchased the Company's common stock between May 7, 2012 and May 29, 2012. The plaintiffs seek unspecified monetary damages, costs and attorneys' fees as well as disgorgement of the proceeds from certain individual defendants' sales of the Company's stock. On October 8, 2014, the Court approved Local No. 8 IBEW Retirement Fund as lead plaintiff, and Scott and Scott LLP as lead counsel for the plaintiff and the putative class. On February 23, 2015, the Company filed a reply to the plaintiffs' opposition to its motion to dismiss. The court heard oral argument on the motion to dismiss on March 6, 2015 and took the motion under advisement. On September 30, 2015, the court granted the Company's motion to dismiss. On October 15, 2015, the plaintiff filed a notice of appeal. The Company believes the claims to be without merit and intend to vigorously defend the litigation. As of September 30, 2015, the Company has not recorded any reserves for this purported class action.

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

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to recover all or a portion of any future amounts paid. The Company has never paid any material amounts to defend lawsuits or settle claims related to these indemnification provisions. Accordingly, the Company believes the estimated fair value of these indemnification arrangements is minimal.

**Other Contingencies**

The Company has certain contingent liabilities that arise in the ordinary course of its business activities. The Company accrues a reserve for contingent liabilities when it is probable that future expenditures will be made and such expenditures can be reasonably estimated. There were no material contingent liabilities accrued as of September 30, 2015 or December 31, 2014.

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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 early-stage development programs in other indications, while maintaining our financial strength.

We market KALYDECO (ivacaftor) for the treatment of certain patients with CF who have specific genetic mutations in their cystic fibrosis transmembrane conductance regulator, or CFTR, gene. In July 2015, we began marketing ORKAMBI (lumacaftor in combination with ivacaftor) in the United States for the treatment of patients with CF twelve years of age and older who have two copies (homozygous) of the F508del mutation in their CFTR gene, which is the most prevalent form of CF. We also are seeking approval to market lumacaftor in combination with ivacaftor for this patient population in Europe, Canada and Australia.

Cystic Fibrosis

Ivacaftor

KALYDECO was approved in 2012 in the United States and European Union as a treatment for patients with CF six years of age and older who have the G551D mutation in their CFTR gene. Our KALYDECO net product revenues have been increasing over the last several years due to the increased number of patients who are being treated with KALYDECO in the United States and ex-U.S. markets as we have expanded the label for KALYDECO and obtained reimbursement for additional patients eligible for treatment with KALYDECO in ex-U.S. markets.

In September 2015, the European Union Committee for Medicinal Products for Human Use, or CHMP, issued positive opinions recommending approval of KALYDECO for patients with CF two to five years of age who have one of nine gating mutations in their CFTR gene and in patients with CF 18 years of age and older who have the R117H mutation in their CFTR gene. The CHMP's opinions will be reviewed by the European Commission, which has the authority to approve medicines for the European Union. In Europe, we believe there are approximately 125 patients with CF two to five years of age with one of the nine gating mutations included in the opinion and approximately 250 patients with CF 18 years of age and old with the R117H mutation in their CFTR gene.

We have submitted a supplemental New Drug Application, or sNDA, for KALYDECO for patients with CF two years of age and older who have one of 23 residual function mutations to the United States Food and Drug Administration, or FDA. A target review date for the sNDA of February 6, 2016 was set under the Prescription Drug User Fee Act for the FDA's decision on the sNDA. The sNDA was based on preclinical data for ivacaftor in the 23 residual function mutations, the established clinical profile of KALYDECO and on previously reported data from an exploratory Phase 2a clinical trial in 24 patients with residual function mutations. Nineteen of the patients in this clinical trial had one of the 23 residual function mutations included in the sNDA. We believe more than 1,500 patients with CF two years of age and older in the United States have the mutations represented in the sNDA.

In the first quarter of 2016, we plan to commence a clinical trial for ivacaftor in patients with CF less than two years of age to evaluate the effect of ivacaftor on markers of CF disease in young children. The clinical trial will utilize a weight-based dose of ivacaftor granules that can be mixed in soft foods or liquids.

Lumacaftor in Combination with Ivacaftor

In July 2015, the FDA approved ORKAMBI for the treatment of patients with CF twelve years of age and older who are homozygous for the F508del mutation in their CFTR gene. In the third quarter of 2015, we began marketing ORKAMBI in the United States and recognized our first net product revenues from ORKAMBI. Our future ORKAMBI net product revenues in the United States will reflect the number of patients for whom ORKAMBI is prescribed, the level of rebates, chargebacks, discounts and other adjustments to our ORKAMBI gross product revenues and patient adherence to the recommended treatment regimen. We believe that there currently are approximately 8,500 patients in the United States who are eligible for treatment with ORKAMBI and that as of September 30, 2015 more than 3,000 patients in the United States have started treatment with ORKAMBI.

We submitted a Marketing Authorization Application, or MAA, for ORKAMBI for the treatment of patients with CF twelve years of age and older who are homozygous for the F508del mutation in their CFTR gene to the European

Medicines Agency, or EMA, in November 2014. In September 2015, the CHMP issued a positive opinion recommending approval of the MAA. The CHMP's opinion will be reviewed by the European Commission, which has the authority to approve medicines for

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the European Union. We believe that there are approximately 12,000 patients with CF twelve years of age and older who are homozygous for the F508del mutation in Europe.

We are conducting two Phase 3 clinical trials to evaluate lumacaftor in combination with ivacaftor for the treatment of patients with CF six to eleven years of age who are homozygous for the F508del mutation in their CFTR gene. The first clinical trial is fully enrolled and if successful, will be used to support an sNDA to the FDA in the first half of 2016. Enrollment is ongoing in the second clinical trial, which is required to support approval of lumacaftor in combination with ivacaftor in this patient population in the European Union.

### VX-661 in Combination with Ivacaftor

In the first quarter of 2015, we initiated a Phase 3 development program for VX-661 in combination with ivacaftor in multiple CF patient populations who have at least one copy of the F508del mutation. These clinical trials are enrolling patients with CF with the following mutations:

• Two copies of the F508del in their CFTR gene;

• One copy of the F508del mutation in their CFTR gene and a second mutation in their CFTR gene that results in a gating defect in the CFTR protein;

• One copy of the F508del mutation in their CFTR gene and a second mutation in their CFTR gene that results in residual CFTR function; and

• One copy of the F508del mutation in their CFTR gene and a second mutation that results in minimal CFTR function.

We expect to enroll more than 1,000 patients with CF in this Phase 3 development program. In mid-2016, we expect to complete enrollment in the first three of these clinical trials and the first part of the fourth clinical trial.

### ENaC Inhibition

In June 2015, we entered into a collaboration with Parion Sciences, Inc., or Parion, to develop investigational epithelial sodium channel, or ENaC, inhibitors, including VX-371 (formerly P-1037), for the potential treatment of CF and other pulmonary diseases. VX-371 is currently being evaluated in an exploratory Phase 2a clinical trial in approximately 120 patients with CF with any mutation in their CFTR gene, including those who have mutations not expected to respond to ivacaftor alone. We expect data from this clinical trial in mid-2016. We expect to initiate a Phase 2a clinical trial of VX-371 in early 2016, that evaluates the addition of VX-371 to treatment with ORKAMBI for patients with CF who are homozygous for the F508del mutation in their CFTR gene.

### Next-generation CFTR Corrector Compounds

We are developing two next-generation CFTR corrector compounds, VX-152 and VX-440, that we plan to evaluate as part of combination treatment regimens. We initiated the first Phase 1 clinical trial in healthy volunteers of a next-generation corrector compound in October 2015 and plan to initiate the first Phase 1 clinical trial in healthy volunteers of the second next-generation corrector compound in November 2015. If these clinical trials are successful, we plan to initiate Phase 2 clinical trials of VX-152 or VX-440 in combination with VX-661 and/or ivacaftor in the second half of 2016. In human bronchial epithelial, or HBE, cells with two copies of the F508del mutation in the CFTR gene, as well as in HBE cells with one copy of the F508del mutation in the CFTR gene and a second mutation that results in minimal CFTR function, the triple combinations (VX-152/VX-661/ivacaftor and VX-440/VX-661/ivacaftor) resulted in chloride transport (measured as a percent of normal) that was approximately three-fold greater than the use of a lumacaftor/ivacaftor combination in these cells.

### Research and Early-Stage Development

We are engaged in a number of other research and early-stage development programs, including in the areas of oncology and pain.

#### Oncology

We are conducting two Phase 1 clinical trials of VX-970, a protein kinase inhibitor of ATR, dosed intravenously in combination with commonly used DNA-damaging chemotherapies across a range of solid tumor types, including triple negative breast cancer and non-small cell lung cancer. Data from a Phase 1 safety and pharmacokinetics clinical trial demonstrated that treatment with VX-970 was generally well-tolerated, both alone and in combination with the chemotherapy drug carboplatin, and there was preliminary evidence of anti-tumor activity. We also have entered into a cooperative research and development agreement with the National Cancer Institute to evaluate VX-970 across other types of cancers. The first clinical trial conducted under the agreement is ongoing, and six additional clinical trials are



planned. We are also in Phase 1

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development of VX-803, a second ATR inhibitor, alone and in combination with chemotherapy. We expect to initiate Phase 1 clinical development of VX-984, a third oncology drug candidate, alone and in combination with chemotherapy, in early 2016.

**Pain**  
We are developing VX-150 and VX-241, two drug candidates for the treatment of pain. By the end of 2015, we expect to initiate a Phase 2 clinical trial to evaluate VX-150 in approximately 100 patients with symptomatic osteoarthritis of the knee. We expect to begin clinical development of VX-241 in the first half of 2016.

We plan to continue investing in our research programs and fostering scientific innovation in order to identify and develop transformative medicines. 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.

### **CRISPR Collaboration**

In October 2015, we entered into an agreement with CRISPR Therapeutics AG, or 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. We and CRISPR will focus the initial discovery efforts on genes known to cause and contribute to cystic fibrosis and sickle cell disease. We have the exclusive rights to license up to six CRISPR-Cas9-based targets. In connection with this collaboration agreement, we made an upfront payment to CRISPR of \$75.0 million and will provide a \$30.0 million investment in CRISPR, which is a private company. We will fund all of the discovery activities, which will be conducted primarily by CRISPR. For potential hemoglobinopathy treatments, including treatments for sickle cell disease, we will share equally all research and development costs and worldwide revenues with CRISPR, and CRISPR would lead the commercialization efforts in the United States. For other targets that we elect to license, we would lead all development and global commercialization activities, and CRISPR would be entitled to development, regulatory and sales milestones and royalties on net sales.

### **HCV Infection**

Prior to 2014, we recognized significant net product revenues based on sales of INCIVEK (telaprevir), a product for the treatment of genotype 1 HCV infection that we marketed in North America. In October 2013, in response to declining sales of INCIVEK and increased competition, we reduced our focus on marketing INCIVEK and eliminated the U.S. field-based sales force that had been promoting INCIVEK. We have withdrawn INCIVEK from the market in the United States, and we expect to wind-down any remaining activities relating to the field of HCV infection in 2015. In the fourth quarter of 2014, we terminated our collaboration with Alios BioPharma, Inc., or Alios, related to the development of HCV nucleotide analogues. Our financial statements reflect the activities related to Alios as discontinued operations.

### **Drug Discovery and Development**

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

If we believe that data from a completed registration program support approval of a drug candidate, we submit an NDA to the FDA requesting approval to market the drug candidate in the United States and seek analogous approvals from comparable regulatory authorities in foreign jurisdictions. To obtain approval, we must, among other things,

demonstrate with evidence gathered in nonclinical studies and well-controlled clinical trials that the drug candidate is safe and effective for the disease it is intended to treat and that the manufacturing facilities, processes and controls for the manufacture of the drug candidate are adequate. The FDA and foreign regulatory authorities have substantial discretion in deciding whether or not a drug candidate should be granted approval based on the benefits and risks of the drug candidate in the treatment of a particular disease, and

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could delay, limit or deny regulatory approval. If regulatory delays are significant or regulatory approval is limited or denied altogether, our financial results and the commercial prospects for the drug candidate involved will be harmed.

Regulatory Compliance

Our marketing of pharmaceutical products is subject to extensive and complex laws and regulations. We have a corporate compliance program designed to actively identify, prevent and mitigate risk through the implementation of compliance policies and systems, and through the promotion of a culture of compliance. Among other laws, regulations and standards, we are subject to various U.S. federal and state laws, and comparable foreign laws pertaining to health care fraud and abuse, including anti-kickback and false claims 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 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. Following the FDA's July 2015 approval of ORKAMBI in the United States, we are engaging 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. If ORKAMBI is approved in Europe and other foreign countries, we will need to focus on obtaining and maintaining 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. 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 following obtaining any marketing approvals for ORKAMBI in ex-U.S. markets.

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## RESULTS OF OPERATIONS

	Three Months Ended		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)	
	September 30, 2015	2014	\$	%	September 30, 2015	2014	\$	%
	(in thousands)				(in thousands)			
Revenues	\$309,816	\$178,987	\$130,829	73 %	\$614,401	\$435,859	\$178,542	41 %
Operating costs and expenses	379,842	321,190	58,652	18 %	1,027,576	974,646	52,930	5 %
Other items, net	(25,123 )	(27,857 )	2,734	10 %	(69,428 )	(23,112 )	\$(46,316 )	(200 )%
Net loss attributable to Vertex	\$(95,149 )	\$(170,060 )	\$(74,911 )	(44 )%	\$(482,603 )	\$(561,899 )	\$(79,296 )	(14 )%

## Net Loss Attributable to Vertex

Net loss attributable to Vertex was \$95.1 million in the third quarter of 2015 compared to a net loss attributable to Vertex of \$170.1 million in the third quarter of 2014. Our revenues increased in the third quarter of 2015 as compared to the third quarter of 2014 due to net product revenues from ORKAMBI, which was approved by the FDA in July 2015, and increased KALYDECO net product revenues, partially offset by decreased collaborative revenues. Our operating costs and expenses increased in the third quarter of 2015 as compared to the third quarter of 2014 primarily due to increases in research and development expenses, sales, general and administrative expenses and cost of product revenues, partially offset by decreased restructuring expenses. In the near term, we expect net loss (income) attributable to Vertex will be dependent on expected increases in ORKAMBI net product revenues.

Net loss attributable to Vertex was \$482.6 million in the nine months ended September 30, 2015 compared to a net loss attributable to Vertex of \$561.9 million in the nine months ended September 30, 2014. Our revenues increased in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014 due to net product revenues from ORKAMBI and increased KALYDECO net product revenues, partially offset by decreased royalty revenues and collaborative revenues. Our operating costs and expenses increased in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014, primarily due to increases in research and development expenses, sales, general and administrative expenses and cost of product revenues, partially offset by decreased restructuring expenses and royalty expenses. The change in other items, net in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014 was principally due to a \$36.7 million credit to other income (expense) recorded in the nine months ended September 30, 2014 related to a one-time payment received in connection with our headquarters lease for which there was no corresponding credit in the nine months ended September 30, 2015.

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

Diluted net loss per share attributable to Vertex common shareholders was \$0.39 in the third quarter of 2015 as compared to a diluted net loss per share attributable to Vertex common shareholders of \$0.72 in the third quarter of 2014. Diluted net loss per share attributable to Vertex common shareholders was \$2.00 in the nine months ended September 30, 2015 as compared to a diluted net loss per share attributable to Vertex common shareholders of \$2.40 in the nine months ended September 30, 2014.

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## Revenues

	Three Months Ended		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)	
	September 30, 2015	2014	\$	%	September 30, 2015	2014	\$	%
	(in thousands)				(in thousands)			
Product revenues, net	\$302,511	\$137,099	\$165,412	121 %	\$593,774	\$362,879	\$230,895	64 %
Royalty revenues	5,759	8,386	(2,627 )	(31 )%	17,628	32,134	(14,506 )	(45 )%
Collaborative revenues	1,546	33,502	(31,956 )	(95 )%	2,999	40,846	(37,847 )	(93 )%
Total revenues	\$309,816	\$178,987	\$130,829	73 %	\$614,401	\$435,859	\$178,542	41 %

## Product Revenues, Net

	Three Months Ended		Increase/(Decrease)		Nine Months Ended		Increase/(Decrease)	
	September 30, 2015	2014	\$	%	September 30, 2015	2014	\$	%
	(in thousands)				(in thousands)			
KALYDECO	\$165,929	\$126,801	\$39,128	31 %	\$450,991	\$339,371	\$111,620	33 %
ORKAMBI	130,767	—	N/A	N/A	130,767	—	N/A	N/A
INCIVEK	5,815	10,298	(4,483 )	(44 )%	12,016	23,508	(11,492 )	(49 )%
Total product revenues, net	\$302,511	\$137,099	\$165,412	121 %	\$593,774	\$362,879	\$230,895	64 %

Our total net product revenues increased in the third quarter and the nine months ended September 30, 2015 as compared to the third quarter and the nine months ended September 30, 2014 due to net product revenues from ORKAMBI, which was approved by the FDA in July 2015, and increased KALYDECO net product revenues. KALYDECO net product revenues were \$165.9 million in the third quarter of 2015, including \$70.9 million of net product revenues from international markets. The increase in KALYDECO net product revenues in the third quarter and the nine months ended September 30, 2015, as compared to the third quarter and the nine months ended September 30, 2014, was primarily due to additional patients being treated with KALYDECO as we completed reimbursement discussions in various jurisdictions and increased the number of patients eligible to receive KALYDECO through multiple label expansions that were approved by regulatory authorities in the United States and Europe during 2014 and 2015.

Our ORKAMBI net product revenues in the United States increased on a monthly basis during the third quarter of 2015 as patients initiated treatment following approval in early July 2015. We expect ORKAMBI revenues to increase in the fourth quarter of 2015 as compared to the third quarter of 2015 as additional patients initiate treatment with ORKAMBI. If approved in the fourth quarter of 2015, we expect to recognize our first ex-U.S. ORKAMBI net product revenues in the first half of 2016. Initially, we expect these revenues will be primarily from Germany due to the time it will take to complete the reimbursement discussions in other European countries.

We have withdrawn INCIVEK from the market in the United States. We may continue to recognize small incremental INCIVEK revenues over the next several quarters as we adjust our INCIVEK reserves for rebates, chargebacks and discounts.

## Royalty Revenues

Our royalty revenues were \$5.8 million and \$17.6 million in the third quarter and the nine months ended September 30, 2015, respectively, as compared to \$8.4 million and \$32.1 million in the third quarter and the nine months ended September 30, 2014, respectively. Since the beginning of 2014, our royalty revenues have consisted of (i) revenues related to a cash payment we received in 2008 when we sold our rights to certain HIV royalties and (ii) revenues related to certain third-party royalties payable by our collaborators on sales of HIV and HCV drugs that also result in corresponding royalty expenses. The decreased royalty revenues in the third quarter and the nine months ended September 30, 2015 as compared to the third quarter and the nine months ended September 30, 2014 were primarily due to the continued decline in net sales of INCIVO (telaprevir) by our collaborator Janssen NV.

## Collaborative Revenues

Our collaborative revenues were \$1.5 million and \$3.0 million in the third quarter and the nine months ended September 30, 2015, respectively, as compared to \$33.5 million and \$40.8 million in the third quarter and the nine months ended September 30, 2014, respectively. The decrease during the third quarter and the nine months ended September 30, 2015 as

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compared to the third quarter and the nine months ended September 30, 2014 was primarily attributable to a one-time \$30.0 million payment we received in July 2014 from Janssen Pharmaceuticals, Inc.

## Operating Costs and Expenses

	Three Months Ended September 30,		Increase/(Decrease)		Nine Months Ended September 30,		Increase/(Decrease)	
	2015	2014	\$	%	2015	2014	\$	%
	(in thousands)				(in thousands)			
Cost of product revenues	\$30,269	\$10,208	\$20,061	197 %	\$55,059	\$28,435	\$26,624	94 %
Royalty expenses	1,691	3,976	(2,285)	(57)%	6,068	18,525	(12,457)	(67)%
Research and development expenses	246,284	190,939	55,345	29 %	685,741	654,043	31,698	5 %
Sales, general and administrative expenses	99,772	75,224	24,548	33 %	280,026	226,882	53,144	23 %
Restructuring expenses, net	1,826	40,843	(39,017)	(96)%	682	46,761	(46,079)	(99)%
Total costs and expenses	\$379,842	\$321,190	\$58,652	18 %	\$1,027,576	\$974,646	\$52,930	5 %

## 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. Our cost of product revenues increased in the third quarter and the nine months ended September 30, 2015 as compared to the third quarter and the nine months ended September 30, 2014 due to increased net product revenues. We expect our cost of product revenues to continue to increase due to increased net product revenues, together with an expected increase in the third-party royalty rate payable to CFFT as we pay royalties at the top end of the royalty range, and the expected payment of an aggregate of \$27.8 million of commercial milestones to CFFT by early 2016 based on sales of ORKAMBI.

## Royalty Expenses

Royalty expenses include third-party royalties payable upon net sales of telaprevir by our collaborators in their territories and 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. Royalty expenses in the third quarter of 2015 decreased by \$2.3 million, or 57%, as compared to the third quarter of 2014 and decreased by \$12.5 million, or 67%, in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014, primarily as a result of decreased INCIVO (telaprevir) sales by our collaborator Janssen NV.

## Research and Development Expenses

	Three Months Ended September 30,		Increase/(Decrease)		Nine Months Ended September 30,		Increase/(Decrease)	
	2015	2014	\$	%	2015	2014	\$	%
	(in thousands)				(in thousands)			
Research expenses	\$69,342	\$63,460	\$5,882	9 %	\$200,099	\$195,825	\$4,274	2 %
Development expenses	176,942	127,479	49,463	39 %	485,642	458,218	27,424	6 %
Total research and development expenses	\$246,284	\$190,939	\$55,345	29 %	\$685,741	\$654,043	\$31,698	5 %

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, 2012, we have incurred \$3.2 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

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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 2014 and the nine months ended September 30, 2015, 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. Obtaining regulatory approval can be a lengthy, time-consuming and uncertain process. We have submitted an MAA to the EMA for lumacaftor in combination with ivacaftor. If we are successful in obtaining marketing approval from the European Commission in the fourth quarter of 2015, we currently do not expect to recognize revenues from ORKAMBI in Europe until the first half of 2016. We cannot make a meaningful estimate when, if ever, our other clinical development programs will generate revenues and cash flows.

## Research Expenses

	Three Months Ended September 30,		Increase/(Decrease)		Nine Months Ended September 30,		Increase/(Decrease)	
	2015	2014	\$	%	2015	2014	\$	%
	(in thousands)				(in thousands)			
Research Expenses:								
Salary and benefits	\$21,542	\$21,575	\$(33)	— %	\$61,796	\$63,017	\$(1,221)	(2) %
Stock-based compensation expense	16,342	11,523	4,819	42 %	43,199	32,414	10,785	33 %
Laboratory supplies and other direct expenses	8,755	7,988	767	10 %	28,339	27,963	376	1 %
Outsourced services	4,788	2,895	1,893	65 %	14,293	12,229	2,064	17 %
Infrastructure costs	17,915	19,479	(1,564)	(8) %	52,472	60,202	(7,730)	(13) %
Total research expenses	\$69,342	\$63,460	\$5,882	9 %	\$200,099	\$195,825	\$4,274	2 %

We maintain a substantial investment in research activities. Our research expenses increased by 9% in the third quarter of 2015 as compared to the third quarter of 2014 and were consistent in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014. We expect to continue to invest in our research programs with a focus on identifying drug candidates for specialty markets.

## Development Expenses

	Three Months Ended September 30,		Increase/(Decrease)		Nine Months Ended September 30,		Increase/(Decrease)	
	2015	2014	\$	%	2015	2014	\$	%
	(in thousands)				(in thousands)			
Development Expenses:								
Salary and benefits	\$42,101	\$40,571	\$1,530	4 %	\$123,723	\$121,411	\$2,312	2 %
Stock-based compensation expense	28,359	19,608	8,751	45 %	81,351	58,870	22,481	38 %
	6,696	5,392	1,304	24 %	22,113	25,040	(2,927)	(12) %

Laboratory supplies  
and other direct  
expenses

Outsourced services	70,927	32,483	38,444	118	%	177,324	156,018	21,306	14	%
Drug supply costs	2,977	2,253	724	32	%	7,262	6,777	485	7	%
Infrastructure costs	25,882	27,172	(1,290)	(5)	)%	73,869	90,102	(16,233)	(18)	)%
Total development expenses	\$176,942	\$127,479	\$49,463	39	%	\$485,642	\$458,218	\$27,424	6	%

Our development expenses increased by \$49.5 million or 39% in the third quarter of 2015 as compared to the third quarter of 2014, primarily due to an increase in outsourced services related to ongoing clinical trials, including our Phase 3 development program for VX-661 in combination with ivacaftor and an increase in stock-based compensation expense. Our

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development expenses increased by \$27.4 million, or 6%, in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014, primarily due to an increase in outsourced services and an increase in stock-based compensation expense, partially offset by a reduction in infrastructure costs. The increase in outsourced services expenses in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014 was primarily due to the increase in clinical trial expenses. The decrease in infrastructure costs in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014 was primarily attributable to the relocation of our corporate headquarters in Massachusetts from Cambridge to Boston in the nine months ended September 30, 2014.

## Sales, General and Administrative Expenses

	Three Months Ended September 30,		Increase/(Decrease)		Nine Months Ended September 30,		Increase/(Decrease)	
	2015	2014	\$	%	2015	2014	\$	%
	(in thousands)				(in thousands)			
Sales, general and administrative expenses	\$99,772	\$75,224	\$24,548	33 %	\$280,026	\$226,882	\$53,144	23 %

Sales, general and administrative expenses increased by 33% in the third quarter of 2015 as compared to the third quarter of 2014 and increased by 23% in the nine months ended September 30, 2015 as compared to the nine months ended September 30, 2014, primarily due to increased investment in commercial support for KALYDECO and ORKAMBI and costs incurred to prepare for the potential launch of ORKAMBI in ex-U.S. markets.

## Restructuring Expense, Net

We recorded restructuring expenses of \$1.8 million in the third quarter of 2015 as compared to restructuring expenses of \$40.8 million in the third quarter of 2014 and recorded restructuring expenses of \$0.7 million in the nine months ended September 30, 2015 as compared to restructuring expenses of \$46.8 million in the nine months ended September 30, 2014. Our restructuring expenses in the third quarter and the nine months ended September 30, 2015 and 2014 primarily relate to adjustments to our restructuring liability resulting from the relocation of our corporate headquarters to Boston, Massachusetts and the early termination of our leases in Cambridge, Massachusetts.

## Other Items

## Interest Expense, Net

Interest expense, net was \$21.1 million and \$63.6 million in the third quarter and the nine months ended September 30, 2015, respectively, compared to \$20.4 million and \$51.7 million in the third quarter and the nine months ended September 30, 2014, respectively. During the fourth quarter of 2015, we expect to incur approximately \$15 million of interest expense associated with the leases for our corporate headquarters and approximately \$5 million of interest expense related to the credit agreement that we entered into in July 2014.

## Other (Expense) Income, Net

Other (expense) income, net was an expense of \$1.3 million in the third quarter and an expense of \$5.0 million in the nine months ended September 30, 2015, compared to expense of \$4.0 million and income of \$34.2 million in the third quarter and the nine months ended September 30, 2014, respectively. Other income (expense), net in the nine months ended September 30, 2014 was primarily due to a credit of \$36.7 million related to a one-time cash payment in the second quarter of 2014 from our landlord pursuant to leases for our corporate headquarters.

## Income Taxes

We recorded a provision for income taxes of \$1.3 million and \$31.8 million in the third quarter and the nine months ended September 30, 2015, respectively, compared to \$3.4 million and \$4.9 million in the third quarter and the nine months ended September 30, 2014, respectively. The provision for income taxes in the nine months ended September 30, 2015 was principally due to the consolidation of Parion as a VIE into our condensed consolidated financial statements in the second quarter of 2015. The provision for income taxes in the third quarter and nine months ended September 30, 2014 related to state income taxes and income earned in various foreign jurisdictions.

## Discontinued Operations, Net of Tax

Our loss from discontinued operations was \$0.1 million and \$0.7 million in the third quarter and the nine months ended September 30, 2014, respectively, related to Alios, a variable interest entity that we consolidated from June 2011 through

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December 2013. As of September 30, 2014, we concluded that we no longer had significant continuing involvement with Alios. As a result, the effect of the Alios collaboration is presented as discontinued operations in our condensed consolidated statements of operations.

### LIQUIDITY AND CAPITAL RESOURCES

As of September 30, 2015, we had cash, cash equivalents and marketable securities of \$1.01 billion, which represented a decrease of \$11 million from \$1.02 billion as of June 30, 2015 and a decrease of \$381 million from \$1.39 billion as of December 31, 2014.

In the third quarter of 2015, we maintained our cash, cash equivalents and marketable securities balance due to increased cash receipts in the third quarter of 2015 from product sales together with \$51.8 million in cash we received from issuances of common stock pursuant to our employee benefit plans balancing cash expenditures in the third quarter of 2015 related to, among other things, research and development expenses and sales, general and administrative expenses.

The decrease in our cash, cash equivalents and marketable securities during the nine months ended September 30, 2015 was primarily due to cash expenditures we made during the nine months ended September 30, 2015 related to, among other things, research and development expenses and sales, general and administrative expenses and an \$80 million payment made in connection with entering into a collaboration agreement with Parion, partially offset by cash receipts from product sales and \$139.7 million in cash we received from issuances of common stock pursuant to our employee benefit plans. We also incurred \$35.9 million in costs for capital expenditures including net cash flows from capital leases during the nine months ended September 30, 2015.

Our future cash flows will be substantially dependent on continued revenue from KALYDECO, increasing ORKAMBI sales in the United States and on obtaining approval and government reimbursement for ORKAMBI in ex-U.S. markets.

#### 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. In the near-term, we expect cash flows from product revenues to increase due to sales of ORKAMBI in the United States. If we are successful in obtaining marketing approval for ORKAMBI from the European Commission in the fourth quarter of 2015, we currently do not expect to recognize revenues from ORKAMBI in Europe until the first half of 2016. Initially, we expect these revenues will be primarily from Germany due to the time it will take to complete the reimbursement discussions in other European countries. We have borrowed \$300.0 million under a credit agreement that we entered into in July 2014 and, subject to certain conditions, we may request up to an additional \$200.0 million pursuant to that credit agreement. In recent periods, we also have 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 operate our organization. In October 2015, we entered into an amendment to our credit agreement to, among other things, modify the repayment schedule of the loan. Under the terms of the credit agreement, we were previously required to repay the principal amount on the \$300.0 million we borrowed in July 2014 in installments of \$15 million on each of November 1, 2015, January 1, 2016, April 1, 2016 and July 1, 2016 and in installments of \$60 million on each of October 1, 2016, January 1, 2017, April 1, 2017 and July 9, 2017. As amended, our first principal payment will be in the fourth quarter of 2016 with us repaying \$75 million on each of October 1, 2016, January 1, 2017, April 1, 2017 and July 9, 2017. We also have substantial facility and capital lease obligations, including leases for two buildings in Boston, Massachusetts that continue through 2028. In addition, we have entered into certain collaboration agreements with third parties that include the funding of certain research, development and commercialization efforts with the potential for future milestone and royalty payments by us upon the achievement of pre-established developmental and regulatory targets.



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

In July 2014, we borrowed \$300.0 million pursuant to a credit agreement. In addition, subject to certain conditions, we may request that the lenders loan us up to an additional \$200.0 million under the credit agreement. We may raise additional capital through public offerings or private placements of our securities or securing new collaborative agreements or other methods of financing. We will continue to manage our capital structure and will consider all financing opportunities, whenever they may occur, that could strengthen our long-term liquidity profile. There can be no assurance that any such financing opportunities will be available on acceptable terms, if at all.

### CONTRACTUAL COMMITMENTS AND OBLIGATIONS

Our commitments and obligations were reported in our Annual Report on Form 10-K for the year ended December 31, 2014, which was filed with the Securities and Exchange Commission, or SEC, on February 13, 2015. 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 the second quarter of 2015, we entered into a collaboration agreement with Parion pursuant to which Parion is eligible to receive milestone and royalty payments, including up to \$490 million in development and regulatory milestone payments for the development of VX-371 (formerly P-1037) and/or P-1055 in CF;

in the fourth quarter of 2015, we entered into a collaboration agreement with CRISPR pursuant to which CRISPR is eligible to receive milestone and royalty payments, including up to \$420 million in development, regulatory and commercial milestone payments for each of up to six targets pursuant to the collaboration; and

in the fourth quarter of 2015, we entered into an amendment to our credit agreement to, among other things, modify the repayment schedule of the loan. As amended, our first principal payment will be in the fourth quarter of 2016 with us repaying \$75 million on each of October 1, 2016, January 1, 2017, April 1, 2017 and July 9, 2017.

### 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 nine months ended September 30, 2015, 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, 2014, which was filed with the SEC on February 13, 2015.

### RECENT ACCOUNTING PRONOUNCEMENTS

For a discussion of recent accounting pronouncements please refer to Note A, “Nature of Business and Accounting Policies—Recent Accounting Pronouncements,” in the 2014 Annual Report on Form 10-K. There were no new accounting pronouncements adopted during the nine months ended September 30, 2015 that had a material effect on our financial statements.

### 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

As of September 30, 2015, we invest our cash in a variety of financial instruments, principally money market funds, investment-grade corporate bonds and commercial paper. 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.

#### Foreign Exchange Market Risk

As a result of our foreign operations, we face exposure to movements in foreign currency exchange rates, primarily the Euro, Swiss Franc, 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 maintain a foreign currency management program with the objective of reducing the impact of exchange rate fluctuations on our operating results and forecasted revenues and expenses denominated in foreign currencies.

#### 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 September 30, 2015 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 September 30, 2015 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

Local No. 8 IBEW Retirement Plan & Trust v. Vertex Pharmaceuticals Incorporated, et al.

On May 28, 2014, a purported shareholder class action Local No. 8 IBEW Retirement Plan & Trust v. Vertex Pharmaceuticals Incorporated, et al. was filed in the United States District Court for the District of Massachusetts, naming us and certain of our current and former officers and directors as defendants. The lawsuit alleged that we made material misrepresentations and/or omissions of material fact in our disclosures during the period from May 7, 2012 through May 29, 2012, all in violation of Section 10(b) of the Securities Exchange Act of 1934, as amended, and Rule 10b-5 promulgated thereunder. The purported class consists of all persons (excluding defendants) who purchased our common stock between May 7, 2012 and May 29, 2012. The plaintiffs seek unspecified monetary damages, costs and attorneys' fees as well as disgorgement of the proceeds from certain individual defendants' sales of our stock. On October 8, 2014, the Court approved Local No. 8 IBEW Retirement Fund as lead plaintiff, and Scott and Scott LLP as lead counsel for the plaintiff and the putative class. We filed a motion to dismiss the complaint on December 8, 2014 and the plaintiffs filed their opposition to our motion to dismiss on January 22, 2015. On February 23, 2015, we filed a reply to the plaintiffs' opposition to our motion to dismiss. The court heard oral argument on our motion to dismiss on March 6, 2015 and took the motion under advisement. On September 30, 2015, the court granted our motion to dismiss. On October 15, 2015, the plaintiff filed a notice of appeal. We believe the claims to be without merit and intend to vigorously defend the litigation.

##### DOJ Subpoena

In the third quarter of 2015, we received a subpoena from the United States Department of Justice related to our marketed medicines. This subpoena requests documents relating primarily to our Good Laboratory Practices in a bioanalytical laboratory. We are in the process of responding to the subpoena and intend to cooperate.

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, 2014, which was filed with the SEC on February 13, 2015. There have been no material changes from the risk factors previously disclosed in that Annual Report on Form 10-K.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

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

- our expectations regarding the amount of, timing of and trends with respect to our revenues, costs and expenses and other gains and losses, including those related to net product revenues from KALYDECO and ORKAMBI;
- our expectations regarding clinical trials, development timelines and regulatory authority filings and submissions for ivacaftor, lumacaftor, VX-661, VX-371 (formerly P-1037), VX-152, VX-440, VX-970, VX-803, VX-984, VX-150 and VX-241, as well as the sNDA for KALYDECO for patients with CF two years of age and older who have one of 23 residual function mutations;
- our expectations regarding planned clinical trials for next-generation correctors based upon pre-clinical data;
- expectations regarding potential marketing approvals for drug candidates in ex-U.S. markets, including drug candidates that have received a positive opinion from the CHMP;
- our ability to successfully market KALYDECO and ORKAMBI or any of our other drug candidates for which we obtain regulatory approval;
- our expectations regarding the timing and structure of clinical trials of our drugs and drug candidates, including ivacaftor, lumacaftor, VX-661, VX-371 (formerly P-1037), VX-152, VX-440, VX-970, VX-803, VX-984, VX-150 and VX-241, and the expected timing of our receipt of data from our ongoing and planned clinical trials;

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

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

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

the establishment, development and maintenance of collaborative relationships;

potential business development activities;

our 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, 2014, which was filed with the SEC on February 13, 2015. 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 September 30, 2015:

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
July 1, 2015 to July 31, 2015	33,518	\$0.01	—	—
August 1, 2015 to August 31, 2015	56,800	\$0.01	—	—
September 1, 2015 to September 30, 2015	32,027	\$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 shares are returned and are available for future awards under the terms of our Amended and Restated 2013 Stock and Option Plan.

## Item 5. Other Information

On October 26, 2015, we entered into a strategic collaboration, option and license agreement, or the CRISPR Agreement, with CRISPR Therapeutics AG and its affiliates 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. Detailed information on the CRISPR Agreement is set forth in Note C of Part 1-Item 1 of this Quarterly Report on Form 10-Q under the heading "CRISPR Therapeutics AG."

Item 6. Exhibits

Exhibit Number Exhibit Description

10.1	2015 Amendments to Credit Agreement, dated as of July 9, 2014, among Vertex Pharmaceuticals Incorporated, Macquarie US Trading LLC and the other lenders party thereto.
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

**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

October 30, 2015

By: /s/ Ian F. Smith

Ian F. Smith

Executive Vice President and Chief Financial Officer

(principal financial officer and

duly authorized officer)