

VERTEX PHARMACEUTICALS INC / MA

Form 10-Q

November 06, 2014

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UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549

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FORM 10-Q

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

FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2014

or

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

FOR THE TRANSITION PERIOD FROM TO

Commission file number 000-19319

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Vertex Pharmaceuticals Incorporated

(Exact name of registrant as specified in its charter)

Massachusetts

04-3039129

(State or other jurisdiction of  
incorporation or organization)

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

50 Northern Avenue, Boston, Massachusetts

02210

(Address of principal executive offices)

(Zip Code)

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

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

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

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

Large accelerated filer ☒ Accelerated filer ☐ Non-accelerated filer ☐ Smaller reporting company ☐  
(Do not check if a smaller reporting company)

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

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

Common Stock, par value \$0.01 per share

240,521,809

Class

Outstanding at October 31, 2014



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FOR THE QUARTER ENDED SEPTEMBER 30, 2014

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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,” “INCIVIC” and “KALYDECO™” are registered trademarks of Vertex. Other brands, names and trademarks contained in this Quarterly Report on Form 10-Q, including “INCIVO™” and “TELAVIC™,” 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,	
	2014	2013	2014	2013
Revenues:				
Product revenues, net	\$137,099	\$186,653	\$362,879	\$708,823
Royalty revenues	8,386	27,012	32,134	119,705
Collaborative revenues	33,502	8,035	40,846	32,290
Total revenues	178,987	221,700	435,859	860,818
Costs and expenses:				
Cost of product revenues	10,208	20,048	28,435	75,698
Royalty expenses	3,976	7,291	18,525	32,315
Research and development expenses	190,939	219,442	654,043	643,636
Sales, general and administrative expenses	75,224	86,427	226,882	283,133
Restructuring expenses	40,843	12,048	46,761	12,863
Intangible asset impairment charge	—	—	—	412,900
Total costs and expenses	321,190	345,256	974,646	1,460,545
Loss from operations	(142,203 )	(123,556 )	(538,787 )	(599,727 )
Interest expense, net	(20,384 )	(95 )	(51,686 )	(10,109 )
Other income (expense), net	(3,990 )	4,751	34,192	3,360
Loss from continuing operations before provision for (benefit from) income taxes	(166,577 )	(118,900 )	(556,281 )	(606,476 )
Provision for (benefit from) income taxes	3,419	2,555	4,915	(123,774 )
Loss from continuing operations	(169,996 )	(121,455 )	(561,196 )	(482,702 )
Loss from discontinued operations, net of tax benefit of \$0, \$3,306, \$0 and \$9,089, respectively	(64 )	(7,207 )	(703 )	(20,299 )
Loss from discontinued operations attributable to noncontrolling interest (Alios)	—	4,530	—	13,688
Net loss from discontinued operations attributable to Vertex	(64 )	(2,677 )	(703 )	(6,611 )
Net loss attributable to Vertex	\$(170,060 )	\$(124,132 )	\$(561,899 )	\$(489,313 )
Net loss per share from continuing operations:				
Basic	\$(0.72 )	\$(0.53 )	\$(2.40 )	\$(2.17 )
Diluted	\$(0.72 )	\$(0.53 )	\$(2.40 )	\$(2.17 )
Net loss from discontinued operations per share attributable to Vertex common shareholders:				
Basic	\$—	\$(0.01 )	\$—	\$(0.03 )
Diluted	\$—	\$(0.01 )	\$—	\$(0.03 )
Net loss per share attributable to Vertex common shareholders:				
Basic	\$(0.72 )	\$(0.54 )	\$(2.40 )	\$(2.20 )
Diluted	\$(0.72 )	\$(0.54 )	\$(2.40 )	\$(2.20 )
Shares used in per share calculations:				
Basic	236,137	230,505	234,207	222,764
Diluted	236,137	230,505	234,207	222,764

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 September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
Loss from continuing operations	\$(169,996 )	\$(121,455 )	\$(561,196 )	\$(482,702 )
Loss from discontinued operations	(64 )	(7,207 )	(703 )	(20,299 )
Net loss	(170,060 )	(128,662 )	(561,899 )	(503,001 )
Changes in other comprehensive loss:				
Unrealized holding gains (losses) on marketable securities	(30 )	166	25	7
Unrealized gains on foreign currency forward contracts	1,838	—	1,713	—
Foreign currency translation adjustment	(624 )	514	(271 )	(7 )
Total changes in other comprehensive loss	1,184	680	1,467	—
Comprehensive loss	(168,876 )	(127,982 )	(560,432 )	(503,001 )
Comprehensive loss attributable to noncontrolling interest (Alios)	—	4,530	—	13,688
Comprehensive loss attributable to Vertex	\$(168,876 )	\$(123,452 )	\$(560,432 )	\$(489,313 )

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, 2014	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	\$718,563	\$569,299
Marketable securities, available for sale	759,174	895,777
Accounts receivable, net	114,308	85,517
Inventories	16,753	14,147
Prepaid expenses and other current assets	41,298	23,836
Total current assets	1,650,096	1,588,576
Restricted cash	121	130
Property and equipment, net	720,878	696,911
Goodwill	30,992	30,992
Other assets	3,999	2,432
Total assets	\$2,406,086	\$2,319,041
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$38,472	\$49,327
Accrued expenses	244,279	271,077
Deferred revenues, current portion	21,210	21,510
Accrued restructuring expenses, current portion	38,384	14,286
Capital lease obligations, current portion	18,124	16,893
Other liabilities, current portion	11,709	24,736
Total current liabilities	372,178	397,829
Deferred revenues, excluding current portion	34,723	49,459
Accrued restructuring expenses, excluding current portion	17,218	14,067
Capital lease obligations, excluding current portion	42,200	48,754
Construction financing lease obligation, excluding current portion	473,172	440,937
Senior secured term loan	294,740	—
Other liabilities, excluding current portion	15,950	11,590
Total liabilities	1,250,181	962,636
Commitments and contingencies		
Shareholders' equity:		
Preferred stock, \$0.01 par value; 1,000,000 shares authorized; none issued and outstanding at September 30, 2014 and December 31, 2013	—	—
Common stock, \$0.01 par value; 300,000,000 shares authorized at September 30, 2014 and December 31, 2013; 240,238,090 and 233,788,852 shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively	2,375	2,320
Additional paid-in capital	5,681,163	5,321,286
Accumulated other comprehensive gain (loss)	1,161	(306)
Accumulated deficit	(4,528,794)	(3,966,895)
Total shareholders' equity	1,155,905	1,356,405
Total liabilities and shareholders' equity	\$2,406,086	\$2,319,041

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 Other Comprehensive Loss	Accumulated Deficit	Total Vertex Shareholders' Equity	Noncontrolling Interest in Discontinued Operations (Alios)		Total Shareholders' Equity	Redeemable Noncontrolling Interest (Alios)
	Shares	Amount								
Balance, December 31, 2012	217,287	\$2,149	\$4,519,448	\$(550 )	\$(3,521,867)	\$999,180	\$196,672		\$1,195,852	\$38,530
Unrealized holding gains on marketable securities				7		7			7	
Foreign currency translation adjustment				(7 )		(7 )			(7 )	
Net loss					(489,313 )	(489,313 )	(13,688 )		(503,001 )	
Issuance of common stock under benefit plans	8,029	79	248,207			248,286	(70 )		248,216	
Convertible senior subordinated notes (due 2015) conversion	8,276	83	402,182			402,265			402,265	
Stock-based compensation expense			104,470			104,470	348		104,818	
Change in liquidation value of noncontrolling interest							(1,094 )		(1,094 )	1,094
Balance, September 30, 2013	233,592	\$2,311	\$5,274,307	\$(550 )	\$(4,011,180)	\$1,264,888	\$182,168		\$1,447,056	\$39,624
Balance, December 31, 2013	233,789	\$2,320	\$5,321,286	\$(306 )	\$(3,966,895)	\$1,356,405	\$—		\$1,356,405	\$—
Unrealized holding gains on marketable				25		25			25	

securities										
Unrealized										
gains on										
foreign										
currency										
forward										
contracts										
Foreign										
currency										
translation										
adjustment										
Net loss										
attributable to										
Vertex										
Issuance of										
common stock										
under benefit	6,449	55	223,812				223,867		223,867	
plans										
Stock-based										
compensation										
expense										
Balance,										
September 30,	240,238	\$2,375	\$5,681,163	\$1,161	\$(4,528,794)	\$1,155,905	\$—	\$1,155,905	\$—	
2014										

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,	
	2014	2013
Cash flows from operating activities:		
Loss from continuing operations	\$(561,196)	) \$(482,702)
Loss from discontinued operations	(703)	) (20,299)
Net loss	(561,899)	) (503,001)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization expense	46,921	30,734
Stock-based compensation expense	135,160	103,933
Other non-cash based compensation expense	—	5,856
Intangible asset impairment charge	—	412,900
Deferred income taxes	—	(130,164)
Impairment of property and equipment	978	6,650
Write-down of inventories to net realizable value	—	10,358
Other non-cash items, net	7	5,307
Changes in operating assets and liabilities:		
Accounts receivable, net	(7,315)	) 20,737
Inventories	(4,901)	) 5,212
Prepaid expenses and other assets	(19,110)	) (16,477)
Accounts payable	(5,544)	) (46,005)
Accrued expenses and other liabilities	12,210	26,172
Accrued restructuring expense	27,249	2,810
Deferred revenues	(15,085)	) (15,447)
Net cash used in operating activities	(391,329)	) (80,425)
Cash flows from investing activities:		
Purchases of marketable securities	(1,066,772)	) (1,850,015)
Sales and maturities of marketable securities	1,203,400	1,842,361
Expenditures for property and equipment	(36,525)	) (36,922)
Decrease in restricted cash and cash equivalents	9	31,807
Decrease in restricted cash and cash equivalents (Alios)	—	18,924
Decrease (increase) in deposits	(92)	) 1,094
Net cash provided by investing activities	100,020	7,249
Cash flows from financing activities:		
Issuances of common stock from employee benefit plans	201,274	242,360
Payments to redeem secured notes (due 2015)	—	(158)
Payments on capital lease obligations	(17,215)	) (14,601)
Payments on construction financing lease obligation	(45,438)	) (63,242)
Proceeds from senior secured term loan	294,383	—
Payments returned related to construction financing lease obligation	8,050	—
Net cash provided by financing activities	441,054	164,359
Effect of changes in exchange rates on cash	(481)	) 2,591
Net increase in cash and cash equivalents	149,264	93,774
Cash and cash equivalents—beginning of period	569,299	489,407
Cash and cash equivalents—end of period	\$718,563	\$583,181
Supplemental disclosure of cash flow information:		

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Cash paid for interest	\$2,817	\$7,637
Cash paid for income taxes	\$798	\$—
Conversion of convertible senior subordinated notes (due 2015) for common stock	\$—	\$399,842
Unamortized deferred debt issuance costs exchanged	\$—	\$4,230
Capitalization of costs related to construction financing lease obligation	\$25,564	\$176,484
Assets acquired under capital lease	\$8,985	\$38,520
Issuances of common stock exercises from employee benefit plans receivable	\$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 and (ii) its wholly-owned subsidiaries. The condensed consolidated statements of operations in this Quarterly Report on Form 10-Q reflect the operations of Alios BioPharma, Inc. ("Alios"), as well as direct expenses Vertex incurred as a result of the Alios Agreement, as discontinued operations. 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, 2014 and 2013.

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, 2013, which are contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2013 that was filed with the Securities and Exchange Commission (the "SEC") on February 11, 2014 (the "2013 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, noncontrolling interest (Alios), the consolidation and deconsolidation of a VIE, leases, discontinued operations presentation 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 2013 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 2013 Annual Report on Form 10-K. The Company did not adopt any new accounting pronouncements during the nine months ended September 30, 2014 that had a material effect on the Company's condensed consolidated financial statements.

In the second quarter of 2014, the Financial Accounting Standards Board issued amended guidance applicable to revenue recognition that will be effective for the Company for the year ending December 31, 2017. The new guidance must be adopted using either a full retrospective approach for all periods presented or a modified retrospective approach. Early adoption is not permitted. The new guidance applies a more principles-based approach to recognizing revenue. The Company is evaluating the new guidance and the expected effect on the Company's condensed consolidated financial statements.



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## VERTEX PHARMACEUTICALS INCORPORATED

## Notes to Condensed Consolidated Financial Statements

(unaudited)

**B. Product Revenues, Net**

The Company sells its products principally to a limited number of major and selected regional wholesalers and specialty pharmacy providers in North America as well as government-owned and supported customers in Europe (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 as long as (i) there is persuasive evidence that an arrangement exists between the Company and the Customer, (ii) collectibility is reasonably assured and (iii) the price is fixed or determinable.

In order to conclude that the price is fixed or determinable, the Company must be able to (i) calculate its gross product revenues from sales to Customers and (ii) reasonably estimate its net product revenues upon delivery to its 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 costs of incentives offered to certain indirect customers, including patients. 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, 2014:

	Trade Allowances	Rebates, Chargebacks and Discounts	Product Returns	Other Incentives	Total
	(in thousands)				
Balance at December 31, 2013	\$ 1,535	\$68,244	\$ 15,799	\$ 1,555	\$87,133
Provision related to current period sales	6,772	30,720	1,691	1,210	40,393
Adjustments related to prior period sales	(8	) 5,052	(2,778	) (72	) 2,194
Credits/payments made	(7,255	) (67,557	) (6,052	) (1,808	) (82,672
Balance at September 30, 2014	\$ 1,044	\$36,459	\$8,660	\$885	\$47,048

**C. Collaborative Arrangements****Janssen Pharmaceutica NV**

In 2006, the Company entered into 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. Under the Janssen HCV Agreement, Janssen NV agreed to be responsible for 50% of the drug development costs incurred under the development program for the parties’ territories (North America for the Company, and the rest of the world, other than specified countries in Asia, for Janssen NV) and has exclusive rights to commercialize telaprevir in its territories including Europe, South America, the Middle East, Africa and Australia. In November 2013, the Company and Janssen NV amended the collaboration agreement (the “2013 Janssen HCV Amendment”).

Janssen NV made a \$165.0 million up-front license payment to the Company in 2006. The Company amortized the up-front license payment over the Company’s estimated period of performance under the Janssen HCV Agreement through November 2013. As of November 2013, the effective date of the 2013 Janssen HCV Amendment, there was \$32.1 million remaining in deferred revenues related to this up-front license payment.





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## VERTEX PHARMACEUTICALS INCORPORATED

## Notes to Condensed Consolidated Financial Statements

(unaudited)

Janssen NV paid the Company a tiered royalty averaging in the mid-20% range as a percentage of net sales of INCIVO in Janssen NV's territories through 2013. Janssen NV was, and continues to be, responsible for certain third-party royalties on net sales of INCIVO in its territories.

Pursuant to the 2013 Janssen HCV Amendment, (i) Janssen NV made a payment of \$152.0 million to the Company in the fourth quarter of 2013; (ii) Janssen NV's obligations to pay the Company royalties on net sales of INCIVO (telaprevir) terminated after the fourth quarter of 2013; and (iii) Janssen NV received a fully-paid license to commercialize INCIVO in its territories, subject to the continued payment of certain third-party royalties on its net sales of INCIVO.

The Company determined that the 2013 Janssen HCV Amendment was a material modification to the Janssen HCV Agreement because there was a material change to the consideration and deliverables under the agreement and determined that there is one undelivered element under the Janssen HCV Agreement, as amended, which is the continuation of certain telaprevir development activities. The Company recognized \$182.4 million of collaborative revenues pursuant to the Janssen HCV Agreement in the fourth quarter of 2013. This amount was primarily attributable to (i) the residual consideration received from Janssen NV, including the \$152.0 million fourth quarter 2013 payment and the remaining deferred revenues related to the 2006 up-front payment, less (ii) the best estimate of selling price for the remaining telaprevir development activities. As of September 30, 2014, the remaining deferred revenue balance related to the Janssen NV collaboration was \$3.3 million and will be recognized as collaborative revenues as telaprevir development program activities are completed. In addition to the collaborative revenues, the Company will continue to record royalty revenues and corresponding royalty expenses related to third-party royalties that Janssen NV remains responsible for based on INCIVO net sales.

The Janssen HCV Agreement will continue in effect until the expiration of Janssen NV's third-party royalty obligations, which expire on a country-by-country basis on the later of (a) the last-to-expire patent covering INCIVO or (b) the last required payment by Janssen NV to the Company pursuant to the agreement. In the European Union, the Company has a patent covering the composition-of-matter of INCIVO that expires in 2026.

During the three and nine months ended September 30, 2014 and 2013, the Company recognized the following revenues attributable to the Janssen HCV collaboration:

	Three Months Ended September 30, 2014		Nine Months Ended September 30, 2014	
	2014	2013	2014	2013
	(in thousands)			
Royalty revenues (INCIVO)	\$2,284	\$20,994	\$12,917	\$104,108
Collaborative revenues:				
Up-front and amendment payments revenues	\$—	\$3,107	\$—	\$9,321
Net reimbursement for telaprevir development costs	1,390	1,413	4,262	1,422
Reimbursement for manufacturing services	—	—	—	10,299
Total collaborative revenues attributable to the Janssen HCV collaboration	\$1,390	\$4,520	\$4,262	\$21,042
Total revenues attributable to the Janssen HCV collaboration	\$3,674	\$25,514	\$17,179	\$125,150

## Mitsubishi Tanabe Pharma Corporation

The Company has a collaboration agreement (the "MTPC Agreement") with Mitsubishi Tanabe Pharma Corporation ("Mitsubishi Tanabe") pursuant to which Mitsubishi Tanabe has a fully-paid license to manufacture and commercialize TELAVIC (the brand name under which Mitsubishi Tanabe is marketing telaprevir) in Japan and other specified countries in Asia. The Company recognized no collaborative revenues attributable to the Mitsubishi Tanabe collaboration in the three and nine months ended September 30, 2014 and 2013.

## 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

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## VERTEX PHARMACEUTICALS INCORPORATED

## Notes to Condensed Consolidated Financial Statements

(unaudited)

financial support for (i) development activities for VX-661, a corrector compound discovered under the collaboration, and (ii) additional research and development activities directed at discovering new corrector compounds. Under the April 2011 Amendment, CFFT agreed to provide the Company with up to \$75.0 million in funding over approximately five years for corrector-compound research and development activities. The Company retains the right to develop and commercialize KALYDECO (ivacaftor), lumacaftor (VX-809), VX-661 and any other compounds discovered during the course of the research collaboration with CFFT.

During the three and nine months ended September 30, 2014 and 2013, the Company recognized the following revenues attributable to the CFFT collaboration:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2013	2014	2013
	(in thousands)			
Collaborative revenues attributable to the CFFT collaboration	\$1,983	\$3,515	\$6,455	\$11,318

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, lumacaftor and VX-661. The April 2011 Amendment provides for a tiered royalty in the same range on net sales of corrector compounds 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 corrector compounds such as lumacaftor or VX-661. The Company began marketing KALYDECO in the United States and certain countries in the European Union in 2012. The Company has royalty obligations to CFFT 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. CFFT 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 corrector compounds. The collaboration also may be terminated by either party for a material breach by the other, subject to notice and cure provisions.

Alios BioPharma, Inc.

In June 2011, the Company entered into a license and collaboration agreement (the "Alios Agreement") with Alios, a privately-held biotechnology company. 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 April 2014, Vertex and Alios amended the Alios Agreement to eliminate the Company's obligations to conduct further development activities with respect to VX-135. In October 2014, the Company provided notice to Alios that the Alios Agreement would terminate in accordance with its terms in December 2014.

Under applicable accounting guidance, the Company consolidated Alios as a variable interest entity for the period from June 13, 2011 through December 31, 2013. The Company deconsolidated Alios as of December 31, 2013 and recorded a full impairment charge related to the Alios HCV nucleotide analogue program because the Company no longer had a variable interest in Alios as a whole and did not possess the power to direct the activities that most significantly affect the economic performance of Alios based on, among other factors, the decline in significance to Alios of the licensed HCV nucleotide analogue program.



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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.

**Noncontrolling Interest (Alios)**

Prior to the deconsolidation, the Company recorded net loss (income) attributable to noncontrolling interest (Alios) on its condensed consolidated statements of operations, reflecting Alios' net loss (income) for the reporting period, adjusted for changes in the fair value of contingent milestone payments and royalties payable by the Company to Alios, which was evaluated each reporting period. As noted above, as of September 30, 2014 the operations of Alios are presented as discontinued operations in these condensed consolidated financial statements. A summary of net loss from discontinued operations attributable to noncontrolling interest (Alios) for the three and nine months ended September 30, 2013 is as follows:

	Three Months Ended September 30, 2013 (in thousands)	Nine Months Ended September 30, 2013
Loss before benefit from income taxes	\$9,056	\$21,177
Decrease (increase) in fair value of contingent milestone and royalty payments	(1,220)	) 1,600
Benefit from income taxes	(3,306)	) (9,089)
Loss from discontinued operations attributable to noncontrolling interest (Alios)	\$4,530	\$13,688

The Company used present-value models to determine the estimated fair value of the contingent milestone and royalty payments until it deconsolidated Alios, based on assumptions regarding the probability of achieving the relevant milestones, estimates regarding the time to develop drug candidates, estimates of future product sales and the appropriate discount and tax rates. The Company based 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 represented a measure of credit risk associated with settling the liability. Significant judgment was used in determining the appropriateness of these assumptions at each reporting period.

The Company's net loss from discontinued operations attributable to noncontrolling interest (Alios) for the three and nine months ended September 30, 2014 was insignificant due to the deconsolidation of Alios effective December 31, 2013.

**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 become 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. The collaboration was subject to the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976. The waiting period expired in July 2014; therefore, there was no accounting impact relating to this agreement during the six months ended June 30, 2014.

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 a non-refundable up-front payment of \$30.0 million from Janssen Inc. in the third quarter of 2014 upon expiration of the waiting period under the Hart–Scott–Rodino Antitrust Improvements Act of 1976. Pursuant to the Janssen Influenza Agreement, the

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Company will receive an additional non-refundable payment of \$5.0 million in the fourth quarter of 2014 and 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. Janssen Inc. may terminate the Janssen Influenza Agreement, subject to certain exceptions, upon six months' notice.

The Company evaluated the deliverables, consisting of licenses to intellectual property and the obligation to complete certain fully-reimbursable research and development activities as directed by Janssen Inc., pursuant to the Janssen Influenza Agreement under multiple element arrangement guidance for collaborative arrangements. The Company concluded that the license has stand-alone value from the research and development activities and determined the relative selling price of these deliverables based on the Company's best estimate of selling price. The Company utilized a discounted cash flow model to determine its best estimate of selling price for the licenses to intellectual property and determined the best estimate of selling price for the research and development activities to be the estimated cost to complete the activities plus a commercially reasonable margin. The Company determined the license had stand-alone value based on the resources and know-how possessed by Janssen Inc. The Company concluded that the Janssen Influenza Agreement and the amendment to the Janssen Influenza Agreement should be accounted for as separate contracts due to the fact that the amendment did not impact the Company's obligations under the original agreement. Based on this analysis, the Company recognized \$30.0 million in collaborative revenues related to the up-front payment in the three and nine months ended September 30, 2014. The Company is recording the reimbursement for the research and development activities 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.

**D. Net Loss Per Share Attributable to Vertex Common Shareholders**

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 per share attributable to Vertex common shareholder calculations because the effect would have been anti-dilutive during each period:

	Three Months Ended September 30, 2014		Nine Months Ended September 30, 2014	
	2013		2013	
	(in thousands)			
Stock options	13,097	16,807	13,097	16,807
Unvested restricted stock and restricted stock units	2,672	2,838	2,672	2,838

**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, 2014, the Company's investments were in money market funds, government-sponsored enterprise securities, corporate debt securities and commercial paper.

As of September 30, 2014, 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 and government-sponsored enterprise securities. 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. During the three and nine months ended September 30, 2014 and 2013, the Company did not record an other-than-temporary impairment charge related to its financial assets.

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



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	Fair Value Measurements as of September 30, 2014			
	Fair Value Hierarchy			Level 3
	Total	Level 1	Level 2	
(in thousands)				
Financial assets carried at fair value:				
Cash equivalents:				
Money market funds	\$343,129	\$343,129	\$—	\$—
Marketable securities:				
Government-sponsored enterprise securities	467,868	467,868	—	—
Commercial paper	46,496	—	46,496	—
Corporate debt securities	244,810	—	244,810	—
Total	\$1,102,303	\$810,997	\$291,306	\$—

The fair value of the Company's foreign currency forward contracts, which were not material as of September 30, 2014, were based on Level 2 inputs and were determined using third party pricing services. Please refer to Note H, "Hedging," for further information regarding the Company's foreign currency forward contracts.

As of September 30, 2014, the carrying value of the Company's Term Loan was \$294.7 million, which was recorded on its condensed consolidated balance sheet. The fair value of the Term Loan was \$294.7 million and is 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 and the discount on the Term Loan. The Level 3 inputs related to the Term Loan are the amounts of the estimated future interest payments. Please refer to Note L, "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  (in thousands)	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
As of September 30, 2014				
Cash and cash equivalents:				
Cash and money market funds	\$718,562	\$1	\$—	\$718,563
Total cash and cash equivalents	\$718,562	\$1	\$—	\$718,563
Marketable securities:				
Government-sponsored enterprise securities (due within 1 year)	\$467,857	\$27	\$(16)	) \$467,868
Commercial paper (due within 1 year)	46,402	94	—	46,496
Corporate debt securities (due within 1 year)	213,352	2	—	213,354
Corporate debt securities (due after 1 year through 5 years)	31,497	—	(41)	) 31,456
Total marketable securities	\$759,108	\$123	\$(57)	) \$759,174
Total cash, cash equivalents and marketable securities	\$1,477,670	\$124	\$(57)	) \$1,477,737

As of December 31, 2013

Cash and cash equivalents:

Cash and money market funds	\$569,299	\$—	\$—	\$569,299
Total cash and cash equivalents	\$569,299	\$—	\$—	\$569,299

Marketable securities:

Government-sponsored enterprise securities (due within 1 year)	\$600,496	\$7	\$(53)	) \$600,450
Commercial paper (due within 1 year)	83,384	109	—	83,493
Corporate debt securities (due within 1 year)	189,674	14	(34)	) 189,654
Corporate debt securities (due after 1 year through 5 years)	22,181	6	(7)	) 22,180
Total marketable securities	\$895,735	\$136	\$(94)	) \$895,777
Total cash, cash equivalents and marketable securities	\$1,465,034	\$136	\$(94)	) \$1,465,076

The Company has a limited number of marketable securities in insignificant loss positions as of September 30, 2014, 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.

## G. Accumulated Other Comprehensive Loss

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

	Foreign Currency Translation Adjustment  (in thousands)	Unrealized Holding Gains on Marketable Securities	Unrealized Gains (Losses) on Foreign Currency Forward Contracts	Total
Balance at December 31, 2013	\$(325)	) \$42	\$(23)	) \$(306)
Other comprehensive income (loss) before reclassifications	(271)	) 25	2,140	1,894

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Amounts reclassified from accumulated other comprehensive loss	—	—	(427	) (427	)
Net current period other comprehensive income (loss)	\$(271	) \$25	\$1,713	\$1,467	
Balance at September 30, 2014	\$(596	) \$67	\$1,690	\$1,161	

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	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, 2012	\$(746)	) \$196	\$—	\$(550)
Other comprehensive loss before reclassifications	(7)	) 7	—	—
Amounts reclassified from accumulated other comprehensive loss	—	—	—	—
Net current period other comprehensive loss	\$(7)	) \$7	\$—	\$—
Balance at September 30, 2013	\$(753)	) \$203	\$—	\$(550)

**H. Hedging**

In 2013, the Company initiated 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 remaining contractual durations from one to twelve months.

The Company formally documents the relationship between foreign currency forward contracts (hedging instruments) and forecasted product revenues (hedged items), as well as the Company's risk management objective and strategy for undertaking various hedging activities, which includes matching all foreign currency forward contracts that are designated as cash flow hedges to forecasted transactions. The Company also formally assesses, both at the hedge's inception and on an ongoing basis, whether the foreign currency forward contracts are highly effective in offsetting changes in cash flows of hedged items on a prospective and retrospective basis. If the Company determines that (i) a foreign currency forward contract is not highly effective as a cash flow hedge, (ii) it has ceased to be a highly effective hedge or (iii) a 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, 2014, all hedges were determined to be highly effective. 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, 2014 (in thousands)	As of December 31, 2013
Foreign Currency		
Euro	\$24,009	\$17,468
British pound sterling	18,108	—
Total foreign currency forward contracts	\$42,117	\$17,468

Changes in fair value of these foreign currency forward contracts are included in accumulated other comprehensive loss as unrealized gains and losses until the forecasted underlying transaction occurs. Unrealized gains and losses on these foreign currency forward contracts are included in (i) prepaid expenses and other current assets and (ii) other liabilities, current portion, respectively, on the Company's condensed consolidated balance sheets. Realized gains and losses for the effective portion of such contracts are recognized in product revenues, net in the condensed consolidated statement of operations when the contract is settled with the counterparty. Cash flows from foreign currency forward contracts are classified within cash flows from operating activities in the same category as the cash flows from the hedged item.

The following table summarizes the fair value of the Company's outstanding foreign currency forward contracts included on the Company's condensed consolidated balance sheets:

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	As of September 30, 2014 (in thousands)	As of December 31, 2013
Fair value - assets	\$ 1,690	\$—
Fair value - liabilities	—	(23 )
Net carrying value	\$ 1,690	\$(23 )

## I. Inventories

The following table sets forth the Company's inventories by type:

	As of September 30, 2014 (in thousands)	As of December 31, 2013
Raw materials	\$—	\$489
Work-in-process	14,302	9,981
Finished goods	2,451	3,677
Total	\$16,753	\$14,147

As of September 30, 2014, the Company has capitalized \$1.8 million of inventory costs for lumacaftor manufactured in preparation for its planned product launch in mid-2015 based on its evaluation of, among other factors, information regarding lumacaftor's safety and efficacy. In periods prior to July 1, 2014, the Company expensed costs associated with lumacaftor's raw materials and work-in-process as a development expense. In November 2014, the Company submitted a New Drug Application to the United States Food and Drug Administration and a Marketing Authorization Application to the European Medicines Agency for lumacaftor in combination with ivacaftor. The Company plans to continue to monitor the status of the lumacaftor regulatory process and the other factors used to determine whether or not to capitalize the lumacaftor inventory and, if there are significant negative developments regarding lumacaftor, the Company could be required to impair previously capitalized costs.

## J. Intangible Assets and Goodwill

## Intangible Assets

As of September 30, 2014, the Company had no intangible assets recorded on its condensed consolidated balance sheet. The intangible assets that were previously reflected on the Company's condensed consolidated balance sheets related to drug candidates for the treatment of HCV infection. The field of HCV infection treatment is highly competitive and characterized by rapid technological advances and the development of drug candidates for the treatment of HCV infection is subject to numerous risks.

## ViroChem Acquisition

The Company determined that the fair value of the VX-222 intangible asset acquired from ViroChem was zero as of March 31, 2013. Accordingly, the Company recorded a \$412.9 million impairment charge in the three months ended March 31, 2013 and the nine months ended September 30, 2013. In connection with this impairment charge, the Company recorded a credit of \$127.6 million in its provision for income taxes. In the nine months ended September 30, 2013, the increase to the Company's net loss attributable to Vertex related to this impairment charge, net of the tax credit, was \$285.3 million, and the net increase to the Company's net loss per share attributable to Vertex common shareholders was \$1.28 per share.

## Alios Collaboration

In June 2011, the Company recorded \$250.6 million of intangible assets on its condensed consolidated balance sheet based on the Company's estimate of the fair value of Alios' HCV nucleotide analogue program, including the intellectual property related to ALS-2200 and ALS-2158. In the fourth quarter of 2013, the Company determined that the fair value of the HCV nucleotide analogue program was zero as of December 31, 2013. Accordingly, in the fourth quarter of 2013, the Company recorded a \$250.6 million impairment charge and a \$102.1 million benefit from income taxes.



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Goodwill

As of September 30, 2014 and December 31, 2013, goodwill of \$31.0 million was recorded on the Company's condensed consolidated balance sheets. There was no change to goodwill recorded during the three and nine months ended September 30, 2014 or 2013.

K. Convertible Senior Subordinated Notes

In 2010, the Company completed an offering of \$400.0 million in aggregate principal amount of 3.35% convertible senior subordinated notes due 2015 (the "2015 Notes"). This offering resulted in \$391.6 million of net proceeds to the Company. The underwriting discount and other expenses of \$8.4 million were recorded as debt issuance costs and were included in other assets on the Company's condensed consolidated balance sheets.

The 2015 Notes were convertible at any time, at the option of the holder, into common stock at a price equal to approximately \$48.83 per share, or 20.4794 shares of common stock per \$1,000 principal amount of the 2015 Notes. If the closing price of the Company's common stock exceeded 130% of the conversion price for at least 20 trading days within a period of 30 consecutive trading days, the Company had the right to redeem the 2015 Notes at its option at a redemption price equal to 100% of the principal amount of the 2015 Notes to be redeemed.

In the second quarter of 2013, the Company's common stock exceeded 130% of the conversion price of the 2015 Notes for at least 20 trading days within a period of 30 consecutive trading days, and the Company notified the holders of the 2015 Notes that it would redeem the 2015 Notes on June 17, 2013. In response to the Company's call of the 2015 Notes for redemption, in accordance with the provisions of the 2015 Notes, the holders of \$399.8 million in aggregate principal amount of 2015 Notes elected to convert their 2015 Notes into the Company's common stock at the conversion price of approximately \$48.83 per share. As a result of these conversions, the Company issued 8,188,448 shares of common stock. The remaining \$0.2 million in aggregate principal amount of 2015 Notes was redeemed on June 17, 2013.

Pursuant to the terms of the 2015 Notes, the Company made an additional payment of \$16.75 per \$1,000 principal amount, payable in shares of the Company's common stock, to the holders of the 2015 Notes that converted or redeemed their 2015 Notes after the Company called the 2015 Notes for redemption. These payments resulted in the issuance of an additional 87,109 shares of the Company's common stock. In the second quarter of 2013, the Company recognized an aggregate of \$6.7 million in interest expense related to the 2015 Notes. Unamortized debt issuance costs for the 2015 Notes of \$4.2 million were recorded as an offset to additional paid-in capital.

L. 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, including having responsibility to pay for a portion of the costs of finish work and structural elements of the Buildings, the Company was deemed for accounting purposes to be the owner of the Buildings during the construction period. Therefore, the Company recorded project construction costs incurred by the landlord as an asset and a related financing obligation during the construction period. The Company evaluated the Fan Pier Leases in the fourth quarter of 2013 and determined that the Fan Pier Leases did not meet the criteria for "sale-leaseback" treatment. This determination was based on, among other things, the Company's continuing involvement with the property in the form of non-recourse financing to the lessor. Accordingly, the Company began depreciating the asset and incurring interest expense related to the financing obligation during the fourth quarter of 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



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Property and equipment, net, included \$518.3 million and \$503.4 million as of September 30, 2014 and December 31, 2013, respectively, related to construction costs for the Buildings at Fan Pier in Boston, Massachusetts. The construction financing lease obligation related to the Buildings at Fan Pier was \$473.5 million and \$440.9 million as of September 30, 2014 and December 31, 2013, respectively.

**Term Loan**

On July 9, 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 bears interest at a rate of 7.2% per annum but shall be reduced to 6.2% per annum on the later to occur of (i) FDA approval in the United States of a product with a label claim for treating patients with cystic fibrosis 12 years of age and older who are homozygous with the F508del mutation ("FDA Approval"), and (ii) the one year anniversary of the closing, in each case, until the second anniversary of the closing. On and after the second anniversary of the closing, the Term Loan will bear interest at a rate per annum equal to LIBOR plus 5.0% to 7.5% depending on the receipt of FDA Approval.

The maturity date of all loans under the facilities is July 9, 2017. Interest is payable quarterly and on the maturity date. The Company is required to repay principal on the Term Loan in installments of \$15.0 million per quarter from October 1, 2015 through July 1, 2016 and in installments of \$60.0 million per quarter 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 second anniversary of the closing 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'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, 2014.

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 that will be recorded as interest expense using the effective interest method over the term of the loan in the Company's condensed consolidated statements of operations.

**Capital Leases**

The Company has outstanding capital leases for equipment, leasehold improvements and software licenses with terms through 2019. The following table sets forth the Company's future minimum payments due under capital leases as of September 30, 2014:



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Year	(in thousands)
2014	\$4,122
2015	20,792
2016	14,254
2017	13,129
2018	13,027
2019	3,047
Thereafter	—
Total payments	\$68,371
Less: amount representing interest	(8,047 )
Present value of payments	\$60,324

**Financing Arrangements**

The Company has outstanding \$33.5 million in irrevocable stand-by letters of credit issued in connection with property leases and other similar agreements that currently are supported by an unsecured credit facility that expires in April 2015. The credit facility provides the Company's creditor the ability to subjectively cash collateralize the letters of credit at the conclusion of any month, which is a contingency that the Company considers to have a remote possibility of occurring.

**M. 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 issues shares pursuant to an employee stock purchase plan ("ESPP"). Effective for equity awards granted on or after February 5, 2014, the Company provides to employees who have rendered significant service to the Company and meet certain age requirements, partial or full acceleration of vesting of these equity awards upon a termination of employment other than for cause. Less than 5% of the Company's employees were eligible for partial or full acceleration of any of their equity awards as of September 30, 2014. The Company typically recognizes stock-based compensation expense related to these awards over the service period from the date of grant until the qualified employees become eligible for partial or full acceleration of vesting.

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(unaudited)

During the three and nine months ended September 30, 2014 and 2013, the Company recognized the following stock-based compensation expense included in loss from continuing operations:

	Three Months Ended September 30, 2014		Nine Months Ended September 30, 2014	
	2013	2013	2013	2013
	(in thousands)			
Stock-based compensation expense by type of award:				
Stock options	\$26,291	\$18,899	\$78,403	\$68,285
Restricted stock and restricted stock units	18,418	10,998	51,431	30,108
ESPP share issuances	1,883	1,504	6,231	6,077
Less stock-based compensation expense capitalized to inventories	(456)	(204)	(905)	(885)
Total stock-based compensation expense included in costs and expenses	\$46,136	\$31,197	\$135,160	\$103,585

## Stock-based compensation expense by line item:

Research and development expenses	\$31,131	\$19,155	\$91,284	\$64,100
Sales, general and administrative expenses	15,005	12,042	43,876	39,485
Total stock-based compensation expense included in costs and expenses	\$46,136	\$31,197	\$135,160	\$103,585

In 2013, the Company also recognized stock-based compensation expense recorded to noncontrolling interest (Alios), which is reflected in the Company's condensed consolidated statements of shareholders equity and noncontrolling interest.

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, 2014	
Type of award:	Unrecognized Expense, Net of Estimated Forfeitures (in thousands)	Weighted-average Recognition Period (in years)
Stock options	\$180,746	2.28
Restricted stock and restricted stock units	\$116,900	2.27
ESPP share issuances	\$1,978	0.43

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## VERTEX PHARMACEUTICALS INCORPORATED

## Notes to Condensed Consolidated Financial Statements

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The following table summarizes information about stock options outstanding and exercisable at September 30, 2014:

Range of Exercise Prices	Options Outstanding		Options Exercisable		
	Number Outstanding	Weighted-average Remaining Contractual Life	Weighted-average Exercise Price	Number Exercisable	Weighted-average Exercise Price
	(in thousands)	(in years)	(per share)	(in thousands)	(per share)
\$10.41–\$20.00	297	1.97	\$16.16	297	\$16.16
\$20.01–\$30.00	633	4.64	\$29.32	482	\$29.13
\$30.01–\$40.00	3,458	4.75	\$36.24	2,627	\$35.71
\$40.01–\$50.00	2,934	8.11	\$46.32	661	\$46.67
\$50.01–\$60.00	1,093	7.07	\$54.27	655	\$54.80
\$60.01–\$70.00	126	8.92	\$65.78	28	\$64.76
\$70.01–\$80.00	1,998	9.36	\$76.71	360	\$74.59
\$80.01–\$90.00	1,231	8.81	\$83.09	369	\$82.53
\$90.01–\$96.87	1,327	9.80	\$96.73	—	\$—
Total	13,097	7.26	\$56.20	5,479	\$43.53

## N. 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, 2014, the Company had \$49.4 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.

## O. Income Taxes

The Company is subject to U.S. federal, state, and foreign income taxes. For the three and nine months ended September 30, 2014, the Company recorded a net 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. For the three and nine months ended September 30, 2013, the Company recorded a provision for income taxes of \$2.6 million and benefit from income taxes \$123.8 million, respectively. The benefit from income taxes in the nine months ended September 30, 2013 primarily related to a tax benefit associated with the Company's impairment of VX-222 in the first quarter of 2013. Please refer to "Note J, "Intangible Assets and Goodwill," for further information regarding the impairment charge.

As of September 30, 2014 and December 31, 2013, the Company had unrecognized tax benefits of \$5.7 million and \$2.0 million, respectively. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of September 30, 2014, 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, 2014 and December 31, 2013.

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 and any other major taxing jurisdiction for years before 2007, except where the Company has net operating losses or tax credit carryforwards that originated before 2005. During the second quarter of 2014, the

Company concluded an

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## VERTEX PHARMACEUTICALS INCORPORATED

## Notes to Condensed Consolidated Financial Statements

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audit by Revenue Quebec for the year ended December 31, 2011 with no material changes. The Company is currently under examination by Revenue Quebec for the year ended December 31, 2012 as well as the Massachusetts Department of Revenue and the Internal Revenue Service for the year ended December 31, 2011. No adjustments have been reported. The Company is not under examination by any other jurisdictions for any tax year.

The Company currently intends to reinvest the total amount of its unremitted earnings, which have not been significant to date, in the local international jurisdiction or to repatriate the earnings only when tax-effective. As a result, the Company has not provided for U.S. federal income taxes on the unremitted earnings of its international subsidiaries. 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. At September 30, 2014, 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.

**P. 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, 2014 and 2013 were as follows:

	Three Months Ended September 30, 2014		Nine Months Ended September 30, 2014	
	2013		2013	
	(in thousands)			
Liability, beginning of the period	\$14,936	\$22,052	\$19,115	\$23,328
Cash payments	(4,249)	(3,902)	(12,071)	(11,323)
Cash received from subleases	2,689	2,670	8,067	8,000
Restructuring (income) expense	(464)	524	(2,199)	1,339
Liability, end of the period	\$12,912	\$21,344	\$12,912	\$21,344

**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, which include lease obligations related to the 120,000 square feet of the Kendall Square facility that the Company continued to use for its operations following its 2003 Kendall Restructuring. The majority of these restructuring charges were recorded in the third quarter of 2014 upon decommissioning three facilities in Cambridge. To record the restructuring expense and related liability on the cease use date for these facilities, the Company estimated (i) its remaining lease obligations including operating costs for these facilities, (ii) the lead-time necessary to sublease the facilities, (iii) projected sublease rental rates and (iv) the anticipated duration of the subleases. The Company discounted the estimated cash flows related to the facilities using a credit-adjusted risk-free rate of 9%. The Company will continue to incur charges through April 2018 related to the difference between the Company's estimated future cash flows related to its lease obligations, which include an estimate for sublease income to be received if applicable, and its actual cash flows.

The activities related to the restructuring liability for the three and nine months ended September 30, 2014 were as follows:





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	Three Months Ended September 30, 2014		2013	Nine Months Ended September 30, 2014		2013
	(in thousands)					
Liability, beginning of the period	\$3,256	\$—		\$797	\$—	
Cash payments	(2,266	) (80	)	(6,643	) (80	)
Restructuring expense	39,752	80		46,588	80	
Liability, end of the period	\$40,742	\$—		\$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. The most significant activity commenced in October 2013 when the Company adopted a restructuring plan that included (i) a workforce reduction primarily related to the commercial support of INCIVEK following the continued and rapid decline in the number of patients being treated with INCIVEK as new medicines for the treatment of HCV infection neared approval and (ii) the write-off of certain assets. This action resulted from the Company's decision to focus its investment on future opportunities in 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, 2014 were as follows:

	Three Months Ended September 30, 2014		2013	Nine Months Ended September 30, 2014		2013
	(in thousands)					
Liability, beginning of the period	\$792	\$—		\$8,441	\$—	
Cash payments	(399	) —		(8,865	) —	
Impairment of property and equipment	—	(6,650	)	—	(6,650	)
Restructuring expense	1,555	11,444		2,372	11,444	
Liability, end of the period	\$1,948	\$4,794		\$1,948	\$4,794	

## Q. 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 in 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 condensed 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 is not subject to refund.

## R. 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 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. The Company believes the claims to be without merit and intends to vigorously defend the litigation. As of September 30, 2014, the Company has not recorded any reserves for this purported class action.

#### S. 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, 2014 or December 31, 2013.

#### T. Guarantees

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

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

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or settle claims related to these indemnification provisions. Accordingly, the Company believes the estimated fair value of these indemnification arrangements is minimal.

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### 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 small molecule drugs. We invest in scientific innovation to create transformative medicines for patients with serious diseases in specialty markets. Our business is focused on developing and commercializing therapies for the treatment of cystic fibrosis, or CF, and advancing our other research and early-stage development programs, while maintaining our financial strength.

We have marketed KALYDECO (ivacaftor) in the United States, European Union and Canada since it was approved in 2012 for the treatment of patients six years of age and older with CF who have specific genetic mutations in their cystic fibrosis transmembrane conductance regulator, or CFTR, gene. In June 2014, we announced data from two Phase 3 clinical trials, referred to as TRAFFIC and TRANSPORT, of lumacaftor, a CFTR corrector compound, in combination with ivacaftor, a CFTR potentiator compound. In TRAFFIC and TRANSPORT, we evaluated the combination regimen in patients 12 years of age and older with CF who have two copies (homozygous) of the F508del mutation in their CFTR gene, which is the most prevalent form of CF. All four treatment arms in TRAFFIC and TRANSPORT met their primary endpoints of mean absolute improvement in lung function as compared to placebo. In November 2014, we submitted a New Drug Application, or NDA, to the United States Food and Drug Administration, or FDA, and a Marketing Authorization Application, or MAA, to the European Medicines Agency, or EMA, for lumacaftor in combination with ivacaftor.

#### Cystic Fibrosis

Our plan is to (i) increase the number of patients eligible for treatment with ivacaftor, (ii) seek marketing approval for lumacaftor in combination with ivacaftor for the treatment of patients with CF who have two copies of the F508del mutation in their CFTR gene and (iii) research and develop earlier-stage compounds for the treatment of CF.

#### Ivacaftor

KALYDECO was approved in 2012 in the United States, European Union and Canada as a treatment for patients with CF six years of age and older who have the G551D mutation in their CFTR gene. We believe that most patients with CF six years of age and older who have the G551D mutation in the United States, Europe and Canada are being treated with KALYDECO. In Australia, we expect KALYDECO to be listed on the Pharmaceutical Benefits Scheme as of December 1, 2014. We believe there are approximately 250 patients six years of age and older who will be eligible for treatment with KALYDECO in Australia. In February 2014, the FDA approved KALYDECO for the treatment of patients with CF six years of age and older who have one of eight other mutations in their CFTR gene, which were studied in our first Phase 3 label-expansion clinical trial for ivacaftor. In July 2014, the European Commission approved KALYDECO for this patient group.

We are seeking to further expand the number of patients eligible for treatment with ivacaftor by (i) evaluating ivacaftor as a potential treatment for patients with CF who have residual CFTR function, including patients with CF who have the R117H mutation in their CFTR gene and (ii) evaluating ivacaftor as a potential treatment for patients with CF two to five years of age with specific mutations in their CFTR gene.

We have completed a Phase 3 clinical trial to evaluate ivacaftor as a treatment for children with CF two to five years of age with specific gating mutations in their CFTR gene, including the G551D mutation, and have submitted an NDA and an MAA line extension application based on this clinical trial. We believe there are approximately 300 children with CF two to five years of age who have gating mutations in North America, Europe and Australia.

Our Phase 3 clinical trial to evaluate ivacaftor in patients with the R117H mutation in their CFTR gene did not meet its primary endpoint of a statistically significant absolute change from baseline in percent predicted forced expiratory volume in one second, or ppFEV<sub>1</sub>. However, a pre-specified subgroup analysis demonstrated a statistically significant clinical benefit in patients with CF 18 years of age and older who have the R117H mutation on at least one allele.

Based on these data, we submitted a supplemental New Drug Application, or sNDA, to the FDA and an MAA variation to the EMA seeking approval of KALYDECO in patients with CF who have the R117H mutation on at least one allele in their CFTR gene. In October 2014, the FDA's Pulmonary Allergy Drugs Advisory Committee voted 13-2 to recommend approval of KALYDECO in patients with CF six years of age and older who have the R117H mutation in their CFTR gene. The FDA is not bound by the committee's recommendation, but often follows its advice. The

FDA is expected to make a decision on the sNDA by December 30, 2014 under the Prescription Drug User Fee Act. Lumacaftor in Combination with Ivacaftor

In the second quarter of 2014, we completed TRAFFIC and TRANSPORT, which were Phase 3 randomized, double-blind, placebo-controlled clinical trials of lumacaftor in combination with ivacaftor. All four treatment arms in TRAFFIC and

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TRANSPORT met their primary endpoints of mean absolute improvement in ppFEV<sub>1</sub> as compared to placebo. In October 2014, we obtained the first interim data from a rollover clinical trial in which patients from TRAFFIC and TRANSPORT received a combination regimen. At the time of the interim analysis, approximately 25% of patients within each arm of the rollover clinical trial had received an additional 24 weeks of treatment. The first interim data from this clinical trial showed that the initial improvements in lung function observed in the 24-week TRAFFIC and TRANSPORT clinical trials were sustained through 48 weeks of treatment with lumacaftor in combination with ivacaftor. The pattern of response observed after the initiation of combination dosing across all patients who received placebo in TRAFFIC and TRANSPORT and subsequently received a combination regimen in the rollover clinical trial was similar to that seen in patients who received a combination regimen in TRAFFIC and TRANSPORT. At the time of the interim analysis, the safety and tolerability results, including the rate of serious adverse events, were consistent with those observed in the TRAFFIC and TRANSPORT. Based on the data from TRAFFIC and TRANSPORT, in November 2014, we submitted an NDA to the FDA and an MAA to the EMA for lumacaftor in combination with ivacaftor in patients with CF twelve years of age and older who have two copies (homozygous) of the F508del mutation in their CFTR gene. The FDA submission includes a request for Priority Review and the European Committee for Medicinal Products for Human Use has granted our request for Accelerated Assessment of the MAA. We believe that there are approximately 22,000 patients twelve years of age and older who have two copies of the F508del mutation in North America, Europe and Australia, including approximately 8,500 in the United States and approximately 12,000 in Europe.

### Ivacaftor in Combination with VX-661

We are evaluating VX-661, a second investigational CFTR corrector, in combination with ivacaftor, in Phase 2 clinical development. VX-661 was granted Orphan Drug Designation by the FDA in 2014. In May 2014, we announced data from a Phase 2 double-blind clinical trial evaluating VX-661 in combination with KALYDECO in patients with CF 12 years of age and older who have one copy of the G551D mutation and one copy of the F508del mutation in their CFTR gene. VX-661 currently is being evaluated in combination with ivacaftor as part of a fully-enrolled 12-week Phase 2b clinical trial in patients 18 years of age and older who have two copies of the F508del mutation in their CFTR gene. This clinical trial is designed to evaluate safety, efficacy and pharmacokinetics to characterize VX-661 for further development.

Based on the data from the clinical trial evaluating VX-661 in combination with KALYDECO in patients who have the G551D mutation in their CFTR gene and pending data from the ongoing 12-week clinical trial in patients homozygous for the F508del mutation and discussions with regulatory authorities, we plan to initiate Phase 3 clinical trials in the first half of 2015 to evaluate VX-661 in combination with ivacaftor. In these Phase 3 clinical trials, we expect to evaluate patients with CF who have (i) two copies of the F508del mutation, (ii) one copy of the F508del mutation and a second mutation in their CFTR gene known to result in a gating defect in the CFTR protein, (iii) one copy of the F508del mutation and a second mutation in their CFTR gene that is known to result in residual function in the CFTR protein and (iv) one copy of the F508del mutation and a second mutation in their CFTR gene that results in minimal CFTR function. As part of the Phase 3 program, we also plan to evaluate ivacaftor monotherapy in patients with a mutation in their CFTR gene that is known to result in residual function in the CFTR protein.

### Next-generation CFTR Corrector Compounds

We also are seeking to identify and develop next-generation CFTR corrector compounds that could be evaluated in regimens combining ivacaftor with two CFTR corrector compounds. We have multiple next-generation correctors in the lead-optimization stage of research and expect to begin clinical development of a next-generation corrector in 2015.

### HCV Infection

In 2013, in response to declining sales of INCIVEK (telaprevir), our product for the treatment of genotype 1 HCV infection and increased competition, we reduced our focus on marketing INCIVEK and eliminated the U.S. field-based sales force that had been promoting INCIVEK. In addition, in the first quarter of 2013 and fourth quarter of 2013, we incurred intangible asset impairment charges of \$412.9 million and \$250.6 million, respectively, related to drug candidates for the treatment of HCV infection. In the fourth quarter of 2014, we provided notice of termination of the collaboration with Alios BioPharma, Inc. that related to the development of HCV nucleotide analogues, and we

are in the process of winding-down all remaining activities relating to the field of HCV infection.

#### Research and Early-Stage Development

We are engaged in a number of other research and early-stage development programs, including programs in the areas of oncology, neurology and other serious and rare diseases. We plan to continue investing in our research programs as well as our early-stage development programs and fostering scientific innovation in order to identify and develop transformative



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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.

### Drug Discovery and Development

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

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

### Regulatory Compliance

Our marketing of pharmaceutical products is subject to extensive and complex laws and regulations. We have a corporate compliance program designed to actively identify, prevent and mitigate risk through the implementation of compliance policies and systems, and through the promotion of a culture of compliance. Among other laws, regulations and standards, we are subject to various U.S. federal and state laws, and comparable foreign laws pertaining to health care fraud and abuse, including anti-kickback and false claims 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 in exchange for, or 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.

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## RESULTS OF OPERATIONS

	Three Months Ended September 30,		Increase/(Decrease)		Nine Months Ended September 30,		Increase/(Decrease)	
	2014	2013	\$	%	2014	2013	\$	%
	(in thousands)				(in thousands)			
Revenues	\$178,987	\$221,700	\$(42,713 )	(19 )%	\$435,859	\$860,818	\$(424,959 )	(49 )%
Operating costs and expenses	\$321,190	\$345,256	\$(24,066 )	(7 )%	\$974,646	\$1,460,545	\$(485,899 )	(33 )%
Other items, net	\$(27,857 )	\$(576 )	N/A	N/A	\$(23,112 )	\$110,414	N/A	N/A
Net loss attributable to Vertex	\$(170,060 )	\$(124,132 )	\$45,928	37 %	\$(561,899 )	\$(489,313 )	\$72,586	15 %

## Net Loss Attributable to Vertex

Net loss attributable to Vertex was \$170.1 million in the third quarter of 2014 compared to a net loss attributable to Vertex of \$124.1 million in the third quarter of 2013. Our revenues decreased in the third quarter of 2014 as compared to the third quarter of 2013 due to decreased INCIVEK net product revenues partially offset by increased KALYDECO net product revenues and increased collaborative revenues. Our operating costs and expenses decreased in the third quarter of 2014 as compared to the third quarter of 2013 primarily due to reductions in research and development expenses, sales, general and administrative expenses and cost of product revenues.

Net loss attributable to Vertex was \$561.9 million in the nine months ended September 30, 2014 compared to a net loss attributable to Vertex of \$489.3 million in the nine months ended September 30, 2013. Our revenues decreased in the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013 due to decreased INCIVEK net product revenues partially offset by increased KAYDECO net product revenues. Our operating expenses decreased in the nine months ended September 30, 2014 compared to the nine months ended September 30, 2013 primarily due to (i) reductions in research and development expenses, sales, general and administrative expenses and cost of product revenues and (ii) a \$412.9 million impairment charge that we recorded in the first quarter of 2013. In connection with this impairment charge, we recorded a benefit from income taxes of \$127.6 million in the first quarter of 2013, which was included in other items, net. The net effect of the impairment charge and the benefit from income taxes was to increase net loss attributable to Vertex in the nine months ended September 30, 2013 by \$285.3 million.

We have incurred and expect to continue to incur net losses on a quarterly basis. In order to execute our business plan and become profitable, we need to obtain approval to market lumacaftor in combination with ivacaftor on a timely basis and to effectively market this combination in the United States and international markets.

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

Diluted net loss per share attributable to Vertex common shareholders was \$0.72 in the third quarter of 2014 as compared to a diluted net loss per share attributable to Vertex common shareholders of \$0.54 in the third quarter of 2013. Diluted net loss per share attributable to Vertex common shareholders was \$2.40 in the nine months ended September 30, 2014 as compared to a diluted net loss per share attributable to Vertex common shareholders of \$2.20 in the nine months ended September 30, 2013.

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## Revenues

	Three Months Ended		Increase/(Decrease)			Nine Months Ended		Increase/(Decrease)		
	September 30,					September 30,				
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
Product revenues, net	\$ 137,099	\$ 186,653	\$(49,554 )	(27 )	%	\$ 362,879	\$ 708,823	\$(345,944 )	(49 )	%
Royalty revenues	8,386	27,012	(18,626 )	(69 )	%	32,134	119,705	(87,571 )	(73 )	%
Collaborative revenues	33,502	8,035	25,467	317	%	40,846	32,290	8,556	26	%
Total revenues	\$ 178,987	\$ 221,700	\$(42,713 )	(19 )	%	\$ 435,859	\$ 860,818	\$(424,959 )	(49 )	%
Product Revenues, Net										
	Three Months Ended		Increase/(Decrease)			Nine Months Ended		Increase/(Decrease)		
	September 30,					September 30,				
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
KALYDECO	\$ 126,801	\$ 101,061	\$ 25,740	25	%	\$ 339,371	\$ 261,861	\$ 77,510	30	%
INCIVEK	10,298	85,592	(75,294 )	(88 )	%	23,508	446,962	(423,454 )	(95 )	%
Total product revenues, net	\$ 137,099	\$ 186,653	\$(49,554 )	(27 )	%	\$ 362,879	\$ 708,823	\$(345,944 )	(49 )	%

Our total net product revenues decreased in the third quarter of 2014 and nine months ended September 30, 2014 as compared to the third quarter of 2013 and nine months ended September 30, 2013 due to decreased INCIVEK net product revenues, partially offset by increased KALYDECO net product revenues.

We began marketing KALYDECO in the United States and certain international markets in 2012. The FDA approved the first label expansion for KALYDECO in the first quarter of 2014 and the European Commission approved a similar label expansion in July 2014. KALYDECO net product revenues were \$126.8 million in the third quarter of 2014, including \$52.9 million of net product revenues from international markets, and were \$339.4 million in the nine months ended September 30, 2014, including \$146.8 million of net product revenues from international markets. The increase in KALYDECO net product revenues in the third quarter of 2014, compared to the third quarter of 2013, was primarily due to additional patients being treated with KALYDECO as a result of label-expansions. A portion of the increase in KALYDECO net product was due to an adjustment to our distribution model in the United States, which resulted in the acceleration of ordering by certain of our specialty pharmacies and distributors in the third quarter of 2014. As a result, we expect that KALYDECO net product revenues will decrease in the fourth quarter of 2014 as compared to the third quarter of 2014. The increase in KALYDECO net product revenues in the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013 was primarily due to additional European countries beginning to provide reimbursement for KALYDECO in the second quarter of 2013 and label-expansions. Future increases in KALYDECO net product revenues are dependent on (i) the potential for obtaining public reimbursement for the cost of KALYDECO in additional markets, and (ii) potential additional label expansions that could increase the number of patients with CF who are eligible for treatment with KALYDECO. In November 2014, we submitted an NDA to the FDA and an MAA to the EMA for lumacaftor in combination with ivacaftor. Obtaining regulatory approval can be a lengthy, time consuming and uncertain process. Even if we are successful in obtaining marketing approval on a timely basis, we currently do not expect to recognize revenue from lumacaftor in combination with ivacaftor until at least mid-2015.

INCIVEK net product revenues have declined significantly in 2014 compared to 2013 and were \$10.3 million in the third quarter of 2014 and \$23.5 million in the nine months ended September 30, 2014. In future periods, we do not expect significant INCIVEK net product revenues.

## Royalty Revenues

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. Our royalty revenues were \$8.4 million and \$32.1 million, respectively, in the third quarter of 2014 and nine months ended September 30, 2014 compared to \$27.0 million and \$119.7 million, respectively, in the third quarter of 2013 and nine months ended September 30, 2013. The decreased royalty revenues in the 2014 periods compared to the 2013 periods were primarily due to the amendment to our collaboration agreement with Janssen NV in the fourth quarter of 2013, under which Janssen NV's obligations to pay us

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royalties on net sales of INCIVO terminated, subject to the continued payment of certain third-party royalties on its net sales of INCIVO.

## Collaborative Revenues

	Three Months Ended September 30, 2014		2013	Nine Months Ended September 30, 2014		2013
	(in thousands)			(in thousands)		
Janssen Inc.	\$30,000		\$—	\$30,000		\$—
Janssen NV	1,390		4,520	4,262		21,042
CFFT and other	2,112		3,515	6,584		11,248
Total collaborative revenues	\$33,502		\$8,035	\$40,846		\$32,290

Our collaborative revenues for the third quarter of 2014 and nine months ended September 30, 2014 related primarily to (i) a \$30.0 million upfront payment received in July 2014 from Janssen Inc. related to our outlicense of VX-787, (ii) net reimbursements from Janssen NV for our remaining telaprevir development costs and (iii) funding provided by CFFT. Our collaborative revenues from Janssen NV for the nine months ended September 30, 2013 included \$10.3 million in reimbursements for manufacturing services. We do not expect to recognize significant collaborative revenues related to the Janssen NV or CFFT collaborations in future periods.

## Operating Costs and Expenses

	Three Months Ended September 30, 2014		Increase/(Decrease)		Nine Months Ended September 30, 2014		Increase/(Decrease)	
	(in thousands)		\$	%	(in thousands)		\$	%
Cost of product revenues	\$10,208	\$20,048	\$(9,840)	(49)%	\$28,435	\$75,698	\$(47,263)	(62)%
Royalty expenses	3,976	7,291	(3,315)	(45)%	18,525	32,315	(13,790)	(43)%
Research and development expenses	190,939	219,442	(28,503)	(13)%	654,043	643,636	10,407	2%
Sales, general and administrative expenses	75,224	86,427	(11,203)	(13)%	226,882	283,133	(56,251)	(20)%
Restructuring expenses	40,843	12,048	28,795	239%	46,761	12,863	33,898	264%
Intangible asset impairment charge	—	—	N/A	N/A	—	412,900	(412,900)	(100)%
Total costs and expenses	\$321,190	\$345,256	\$(24,066)	(7)%	\$974,646	\$1,460,545	\$(485,899)	(33)%

## 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 KALYDECO and INCIVEK. Cost of product revenues decreased in the third quarter of 2014 as compared to the third quarter of 2013, and in the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013, primarily due to decreased product revenues from INCIVEK. In addition, a \$9.3 million commercial milestone payment payable under our agreement with CFFT was recognized in the first quarter of 2013 and is included in cost of product revenues for the nine months ended September 30, 2013.

## Royalty Expenses

Royalty expenses include third-party royalties payable upon net sales of telaprevir by our collaborators and royalty expenses related to a subroyalty payable to a third party on net sales of an HIV protease inhibitor sold by GlaxoSmithKline. Royalty expenses in the third quarter of 2014 decreased by \$3.3 million, or 45%, as compared to

the third quarter of 2013, and decreased by \$13.8 million, or 43%, in the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013, as a result of decreased INCIVO sales by Janssen NV.

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## Research and Development Expenses

	Three Months Ended September 30,		Increase/(Decrease)			Nine Months Ended September 30,		Increase/(Decrease)		
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
Research expenses	\$63,460	\$55,561	\$7,899	14	%	\$195,825	\$171,958	\$23,867	14	%
Development expenses	127,479	163,881	(36,402)	(22)	)%	458,218	471,678	(13,460)	(3)	)%
Total research and development expenses	\$190,939	\$219,442	\$(28,503)	(13)	)%	\$654,043	\$643,636	\$10,407	2	%

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.

To date, we have incurred \$7.1 billion in research and development expenses associated with drug discovery and development. The successful development of our drug candidates is highly uncertain and subject to a number of risks. In addition, the duration of clinical trials may vary substantially according to the type, complexity and novelty of the drug candidate and the disease indication being targeted. The FDA and comparable agencies in foreign countries impose substantial requirements on the introduction of therapeutic pharmaceutical products, typically requiring lengthy and detailed laboratory and clinical testing procedures, sampling activities and other costly and time-consuming procedures. Data obtained from nonclinical and clinical activities at any step in the testing process may be adverse and lead to discontinuation or redirection of development activities. Data obtained from these activities also are susceptible to varying interpretations, which could delay, limit or prevent regulatory approval. The duration and cost of discovery, nonclinical studies and clinical trials may vary significantly over the life of a project and are difficult to predict. Therefore, accurate and meaningful estimates of the ultimate costs to bring our drug candidates to market are not available.

In 2014, costs related to our CF programs have 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. In November 2014 we submitted an NDA to the FDA and an MAA to the EMA for lumacaftor in combination with ivacaftor. Obtaining regulatory approval can be a lengthy, time-consuming and uncertain process. Even if we are successful in obtaining marketing approval on a timely basis, we currently do not expect to recognize revenues from lumacaftor in combination with ivacaftor in the United States until at least mid-2015 and expect that it will take longer to obtain approval and third-party reimbursement for the combination therapy in international markets. We cannot make a meaningful estimate of 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)		
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
Research Expenses:										
Salary and benefits	\$21,575	\$19,388	\$2,187	11	%	\$63,017	\$61,205	\$1,812	3	%
	11,523	6,535	4,988	76	%	32,414	21,121	11,293	53	%

Stock-based  
compensation  
expense

Laboratory supplies and other direct expenses	7,988	8,596	(608)	(7)%	27,963	26,078	1,885	7	%
Outsourced services	2,895	4,739	(1,844)	(39)%	12,229	13,769	(1,540)	(11)	%
Infrastructure costs	19,479	16,303	3,176	19%	60,202	49,785	10,417	21	%
Total research expenses	\$63,460	\$55,561	\$7,899	14%	\$195,825	\$171,958	\$23,867	14	%

We maintain a substantial investment in research activities. Our research expenses increased in the third quarter of 2014 as compared to the third quarter of 2013, and the nine months ended September 30, 2014 as compared to the nine months



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ended September 30, 2013, primarily due to increased expenses related to employees in our research organization. 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)		
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
Development Expenses:										
Salary and benefits	\$40,571	\$41,468	\$(897)	(2)	%	\$121,411	\$128,798	\$(7,387)	(6)	%
Stock-based compensation expense	19,608	12,620	6,988	55	%	58,870	42,979	15,891	37	%
Laboratory supplies and other direct expenses	5,392	9,468	(4,076)	(43)	%	25,040	26,215	(1,175)	(4)	%
Outsourced services	32,483	64,602	(32,119)	(50)	%	156,018	167,670	(11,652)	(7)	%
Drug supply costs	2,253	9,773	(7,520)	(77)	%	6,777	25,873	(19,096)	(74)	%
Infrastructure costs	27,172	25,950	1,222	5	%	90,102	80,143	9,959	12	%
Total development expenses	\$127,479	\$163,881	\$(36,402)	(22)	%	\$458,218	\$471,678	\$(13,460)	(3)	%

Our development expenses decreased by \$36.4 million, or 22%, in the third quarter of 2014 as compared to the third quarter of 2013, and decreased by \$13.5 million, or 3%, in the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013. Our decreased development expenses in each of the periods was primarily due to decreased outsourced services expenses and drug supply costs partially offset by increased stock-based compensation expense. The significant decrease in outsourced services expenses in the third quarter of 2014 and the nine months ended September 30, 2014 was largely attributable to decreased clinical trial expenses resulting from the completion of the TRAFFIC and TRANSPORT clinical trials in the first half of 2014. We expect our development expenses for outsourced activities to increase in future periods as compared to the third quarter of 2014 due to activities related to clinical trials we plan to initiate in 2015.

**Sales, General and Administrative Expenses**

	Three Months Ended September 30,		Increase/(Decrease)			Nine Months Ended September 30,		Increase/(Decrease)		
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
Sales, general and administrative expenses	\$75,224	\$86,427	\$(11,203)	(13)	%	\$226,882	\$283,133	\$(56,251)	(20)	%

Sales, general and administrative expenses decreased by 13% in the third quarter of 2014 as compared to the third quarter of 2013 and decreased by 20% in the nine months ended September 30, 2014 as compared to the nine months ended September 30, 2013, due primarily to decreased headcount following our October 2013 restructuring activities.

**Restructuring Expense**

We recorded restructuring expenses of \$40.8 million in the third quarter of 2014 as compared to restructuring expenses of \$12.0 million in the third quarter of 2013 and restructuring expenses of \$46.8 million in the nine months ended September 30, 2014 as compared to restructuring expenses of \$12.9 million in the nine months ended September 30, 2013. Our restructuring expenses in the third quarter of 2014 and nine months ended September 30,

2014 primarily related to the relocation of our corporate headquarters to Boston, Massachusetts from Cambridge, Massachusetts.

**Intangible Asset Impairment Charge**

In the first quarter of 2013, we recorded a \$412.9 million impairment charge related to VX-222. In connection with this impairment charge, we recorded a credit of \$127.6 million in our provision for income taxes in the first quarter of 2013. The net effect on net loss attributable to Vertex related to this impairment charge was \$285.3 million in the nine months ended September 30, 2013. We did not record any intangible asset impairment charges in the third quarter of 2014 or the nine months ended September 30, 2014.

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### Other Items

#### Interest Expense, Net

Interest expense, net was \$20.4 million and \$51.7 million in the third quarter of 2014 and nine months ended September 30, 2014, respectively, compared to \$0.1 million and \$10.1 million in the third quarter of 2013 and nine months ended September 30, 2013, respectively. The increases in interest expense, net during the 2014 periods as compared to the 2013 periods was primarily due to interest expense associated with the leases for our corporate headquarters and the \$300.0 million we borrowed in July 2014 pursuant to our credit agreement. During the fourth quarter of 2014, 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 Income (Expense), Net

Other income (expense), net was \$(4.0) million and \$34.2 million in the third quarter of 2014 and nine months ended September 30, 2014, respectively, compared to \$4.8 million and \$3.4 million in the third quarter of 2013 and nine months ended September 30, 2013, respectively. Other income (expense), net in the third quarter was primarily due to realized and unrealized foreign exchange losses. 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 \$3.4 million and \$4.9 million in the third quarter of 2014 and nine months ended September 30, 2014, respectively. In the third quarter of 2013, we recorded a provision for income taxes of \$2.6 million. In the nine months ended September 30, 2013, we recorded a benefit from income taxes of \$123.8 million primarily due to the impairment of VX-222. In connection with the VX-222 impairment charge, we wrote-off the associated deferred tax liability of \$127.6 million as a benefit in our condensed consolidated statements of operations during the nine months ended September 30, 2013.

#### Discontinued Operations, Net of Tax

Our loss from discontinued operations was \$7.2 million and \$20.3 million including benefits from income taxes of \$3.3 million and \$9.1 million in the third quarter of 2013 and nine months ended September 30, 2013, respectively. These losses related to Alios BioPharma, Inc., a variable interest entity that we consolidated from June 2011 through December 2013. Our loss from discontinued operations, net of tax in the third quarter of 2014 and nine months ended September 30, 2014 was not significant. As of September 30, 2014, we concluded that we no longer had significant continuing involvement with Alios; therefore, 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, 2014, we had cash, cash equivalents and marketable securities of \$1.48 billion, which represented an increase of \$12.7 million from \$1.47 billion as of December 31, 2013. This increase was due to cash receipts from product sales and royalties, the upfront payment of \$30.0 million from Janssen Inc., a one-time cash payment of \$36.7 million from our landlord pursuant to the terms of the leases for our corporate headquarters, \$300.0 million we borrowed pursuant to a credit agreement we entered into in the third quarter of 2014 and \$201.3 million in cash we received from issuances of common stock pursuant to our employee benefit plans, largely offset by cash expenditures we made during the nine months ended September 30, 2014 related to, among other things, research and development expenses and sales, general and administrative expenses and \$53.7 million for capital expenditures. We expect to continue to incur losses on a quarterly basis until we can substantially increase revenues as a result of potential future regulatory approvals, the timing of which are uncertain.

#### Sources of Liquidity

We intend to rely on cash flows from product sales as our primary source of liquidity. Our cash flows from product sales have been decreasing in recent periods. Our near-term cash flows from product sales will be dependent on continued sales of KALYDECO, the outcomes of our reimbursement discussions with governmental authorities in international markets and potential future regulatory approvals based on our label-expansion programs for ivacaftor. In 2015, we expect our cash flows from revenues will be dependent on our ability to obtain regulatory approval for

lumacaftor in combination with ivacaftor based on data from TRAFFIC and TRANSPORT. In addition, subject to certain conditions, we may request up to an additional \$200.0 million under the credit agreement we entered into in the third quarter of 2014. In recent periods, we also have received significant proceeds from the issuance of common stock under our employee benefit plans, but the amount and

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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.

### Future Capital Requirements

We incur substantial operating expenses to conduct research and development activities and operate our organization. In addition, we must repay the principal amount on the \$300.0 million we borrowed in the third quarter of 2014 as follows: \$15.0 million in the second half of 2015, \$105.0 million in 2016 and \$180.0 million in 2017. We also have substantial facility and capital lease obligations, including leases for two buildings in Boston, Massachusetts that continue through 2028. We expect that cash flows from KALYDECO together with our current cash, cash equivalents and marketable securities will be sufficient to fund our operations for at least the next twelve months. The adequacy of our available funds to meet our future operating and capital requirements will depend on many factors, including the amounts of future revenues generated by KALYDECO, potential revenues from lumacaftor in combination with ivacaftor, and the potential introduction or one or more of our other drug candidates to the market, and the number, breadth, cost and prospects of our research and development programs.

### Financing Strategy

In the third quarter of 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. Although we do not have any plans to do so in the near term, we may raise additional capital through public offerings or private placements of our securities. In addition, we may raise additional capital through securing new collaborative agreements or other methods of financing. We will continue to manage our capital structure and to 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, 2013, which was filed with the Securities and Exchange Commission, or SEC, on February 11, 2014. There have been no material changes from the contractual commitments and obligations previously disclosed in that Annual Report on Form 10-K, other than the \$300.0 million borrowed under the credit agreement we entered into in the third quarter of 2014.

### 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, 2014, 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, 2013, which was filed with the SEC on February 11, 2014.

### RECENT ACCOUNTING PRONOUNCEMENTS

Refer to Note A, "Basis of Presentation and Accounting Policies," in the accompanying notes to the condensed consolidated financial statements for a discussion of recent accounting pronouncements. There were no new accounting pronouncements adopted during the nine months ended September 30, 2014 that had a material effect on our financial statements.



In the second quarter of 2014, the Financial Accounting Standards Board issued amended guidance applicable to revenue recognition which will be effective for us for the year ending December 31, 2017. Early adoption is not permitted. The new guidance applies a more principle-based approach to recognizing revenue. The new guidance must be adopted using either a full retrospective approach for all periods presented or a modified retrospective approach. We are in process of evaluating the new guidance and determining the expected effect on our consolidated 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

We invest our cash in a variety of financial instruments, principally money market funds, securities issued by the U.S. government and its agencies, 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. The change in fair value of these foreign currency forward contracts included in accumulated other comprehensive loss and the gross fair value of foreign currency forward assets and liabilities included on the condensed consolidated balance sheet as of September 30, 2014 were not material.

### 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, 2014 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, 2014 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting other than the implementation of a new human resources software platform and the upgrade of our Oracle enterprise resource planning system, together with related adjustments to our systems controls, in the third quarter of 2014.

## 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 believe the claims to be without merit and intend to vigorously defend the litigation.

#### 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, 2013, which was filed with the SEC on February 11, 2014. There have been no material changes from the risk factors previously disclosed in that Annual Report on Form 10-K, except that:

Our business and future net product revenues depend heavily on the success of lumacaftor in combination with ivacaftor, which has not been approved by the FDA or the European Commission. If we are unable to obtain marketing approval or experience material delays in obtaining marketing approval for lumacaftor in combination with ivacaftor our business will be materially harmed.

We believe that a significant portion of the value attributed to our company by investors is based on the commercial potential of lumacaftor in combination with ivacaftor. In November 2014, we submitted an NDA in the United States and an MAA, in Europe for this potential combination regimen. Obtaining approval of an NDA or an MAA is a lengthy, expensive and uncertain process, and we may not be successful. Obtaining marketing approval for the combination of lumacaftor and ivacaftor in one country or region does not ensure that we will be able to obtain marketing approval in any other country or region.

Obtaining approval to market the combination of lumacaftor and ivacaftor will depend on many factors, including: whether or not the FDA and European regulatory authorities determine that the evidence gathered in well-controlled clinical trials, other clinical trials and nonclinical studies demonstrates that lumacaftor in combination with ivacaftor is safe and effective as a treatment for patients with CF 12 years of age and older who have two copies of the F508del mutation;

whether or not the FDA and European regulatory authorities are satisfied that the manufacturing facilities, processes and controls for the combination of lumacaftor and ivacaftor are adequate, that the labeling is satisfactory and that plans for post-marketing studies, safety monitoring and risk evaluation and mitigation are sufficient; and the timing and nature of the FDA and European Medicines Agency, or EMA's, comments and questions regarding the NDA and MAA for the combination of lumacaftor and ivacaftor, the scheduling and recommendations of any advisory committee meeting to consider the combination of lumacaftor and ivacaftor, the time required to respond to the FDA or EMA's comments and questions and to obtain the final labeling for the combination of lumacaftor and ivacaftor and any other delays that may be associated with the NDA and MAA review process.

Even if a product is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming clinical trials or reporting as conditions of approval. If we experience material delays in obtaining marketing approval for the combination of lumacaftor and ivacaftor in either the United States or Europe, our future net product revenues and cash flows will be adversely effected. If we do not obtain approval to market the combination of lumacaftor and ivacaftor in the United States or Europe, our business will be materially harmed. Additionally, even if the combination of lumacaftor and ivacaftor receives marketing approval, coverage and reimbursement may not be available and, even if it is available, the level of reimbursement may not be satisfactory. The regulations that govern pricing, coverage and reimbursement for drugs vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing

review period begins after marketing approval is granted. Adverse pricing limitations or a delay in obtaining coverage and reimbursement will hinder our future net product revenues and may harm our business.

Our indebtedness could materially and adversely affect our financial condition and the terms of our credit agreement impose restrictions on our business, reducing our operational flexibility and creating default risks.

In July 2014, we entered into a credit agreement that provides for a \$300.0 million senior secured term loan. We are required to repay principal on the loan in installments of \$15.0 million per quarter from October 1, 2015 through July 1, 2016 and in installments of \$60.0 million per quarter from October 1, 2016 through July 9, 2017.

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Our indebtedness could have important consequences to our business, including increasing our vulnerability to general adverse financial, business, economic and industry conditions, as well as other factors that are beyond our control. Beginning in October 2015, we will be required to begin repayment of the principal amount of our indebtedness, thereby reducing the availability of future cash flows to fund working capital, capital expenditures, acquisitions, research and development efforts and other general corporate purposes.

The credit agreement requires that we 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 our ability and the ability of our 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. As a result, we may be restricted from engaging in business activities that may otherwise improve our business. Failure to comply with the covenants could result in an event of default that could trigger acceleration of our indebtedness, which would require us to repay all amounts owing under the credit agreement and/or our capital leases and could have a material adverse impact on our business.

Additionally, our obligations under the facilities are unconditionally guaranteed by certain of our domestic subsidiaries. All obligations under the facilities, and the guarantees of those obligations, are secured, subject to certain exceptions, by substantially all of our assets and the assets of all guarantors, including the pledge of all or a portion of the equity interests of certain of our subsidiaries. If we fail to satisfy our obligations under the credit agreement or are unable to obtain sufficient funds to make payments, the lenders could foreclose on our pledged collateral.

### **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 INCIVEK;
- our expectations regarding clinical trials, development timelines and regulatory authority filings and submissions for ivacaftor, lumacaftor and VX-661, including the expected NDA and MAA filings for lumacaftor in combination with ivacaftor;
- our ability to successfully market KALYDECO, lumacaftor in combination with ivacaftor, if approved, 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 and VX-661, 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.

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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, 2013, which was filed with the SEC on February 11, 2014, and above in this Item 1A of this Quarterly Report on Form 10-Q for the quarter ended September 30, 2014. 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, 2014:

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, 2014 to July 31, 2014	15,524	\$0.01	—	—
August 1, 2014 to August 31, 2014	47,971	\$0.01	—	—
September 1, 2014 to September 30, 2014	35,720	\$0.01	—	—

The repurchases were made under the terms of our Amended and Restated 2006 Stock and Option Plan. Under this plan, 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 to the Amended and Restated 2006 Stock and Option Plan and are available for future awards under the terms of that plan.

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Item 6. Exhibits

Exhibit Number Exhibit Description

10.1	Credit Agreement, dated as of July 9, 2014, among Vertex Pharmaceuticals Incorporated, Macquarie US Trading LLC and the other lenders party thereto. (1)
31.1	Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation
101.LAB	XBRL Taxonomy Extension Labels
101.PRE	XBRL Taxonomy Extension Presentation
101.DEF	XBRL Taxonomy Extension Definition
(1) Incorporated by reference to Exhibit 10.2 to Vertex's Quarterly Report on Form 10-Q, filed on July 31, 2014.	

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

November 6, 2014

By: /s/ Ian F. Smith

Ian F. Smith

Executive Vice President and Chief Financial Officer

(principal financial officer and  
duly authorized officer)