

VERTEX PHARMACEUTICALS INC / MA

Form 10-Q

July 31, 2014

Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 10-Q

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

FOR THE QUARTERLY PERIOD ENDED JUNE 30, 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

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

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

238,081,533

Class

Outstanding at July 25, 2014

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED
FORM 10-Q
FOR THE QUARTER ENDED JUNE 30, 2014

TABLE OF CONTENTS

	Page
<u>Part I. Financial Information</u>	
<u>Item 1. Financial Statements</u>	<u>2</u>
<u>Condensed Consolidated Financial Statements (unaudited)</u>	<u>2</u>
<u>Condensed Consolidated Statements of Operations - Three and Six Months Ended June 30, 2014 and 2013</u>	<u>2</u>
<u>Condensed Consolidated Statements of Comprehensive Income (Loss) - Three and Six Months Ended June 30, 2014 and 2013</u>	<u>3</u>
<u>Condensed Consolidated Balance Sheets - June 30, 2014 and December 31, 2013</u>	<u>4</u>
<u>Condensed Consolidated Statements of Shareholders' Equity and Noncontrolling Interest - Six Months Ended June 30, 2014 and 2013</u>	<u>5</u>
<u>Condensed Consolidated Statements of Cash Flows - Six Months Ended June 30, 2014 and 2013</u>	<u>6</u>
<u>Notes to Condensed Consolidated Financial Statements</u>	<u>7</u>
<u>Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	<u>25</u>
<u>Item 3. Quantitative and Qualitative Disclosures About Market Risk</u>	<u>37</u>
<u>Item 4. Controls and Procedures</u>	<u>37</u>
<u>Part II. Other Information</u>	
<u>Item 1. Legal Proceedings</u>	<u>38</u>
<u>Item 1A. Risk Factors</u>	<u>38</u>
<u>Item 2. Unregistered Sales of Equity Securities and Use of Proceeds</u>	<u>39</u>
<u>Item 6. Exhibits</u>	<u>39</u>
<u>Signatures</u>	<u>40</u>

“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,” “INCIVIK[®]” 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.

Table of Contents

Part I. Financial Information

Item 1. Financial Statements

VERTEX PHARMACEUTICALS INCORPORATED

Condensed Consolidated Statements of Operations

(unaudited)

(in thousands, except per share amounts)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
Revenues:				
Product revenues, net	\$122,319	\$254,789	\$225,780	\$522,170
Royalty revenues	13,015	49,120	23,748	92,693
Collaborative revenues	3,087	6,841	7,344	24,255
Total revenues	138,421	310,750	256,872	639,118
Costs and expenses:				
Cost of product revenues	9,655	24,695	18,227	55,650
Royalty expenses	7,645	13,236	14,549	25,024
Research and development expenses	224,780	222,455	463,743	440,550
Sales, general and administrative expenses	77,446	106,521	151,658	199,400
Restructuring expenses	(270)	776	5,918	815
Intangible asset impairment charge	—	—	—	412,900
Total costs and expenses	319,256	367,683	654,095	1,134,339
Loss from operations	(180,835)	(56,933)	(397,223)	(495,221)
Interest expense, net	(15,585)	(6,551)	(31,302)	(10,016)
Other income (expense), net	37,731	(27)	38,182	(1,214)
Loss before provision for (benefit from) income taxes	(158,689)	(63,511)	(390,343)	(506,451)
Provision for (benefit from) income taxes	693	(1,799)	1,496	(132,112)
Net loss	(159,382)	(61,712)	(391,839)	(374,339)
Net loss attributable to noncontrolling interest (Alios)	—	4,547	—	9,158
Net loss attributable to Vertex	\$(159,382)	\$(57,165)	\$(391,839)	\$(365,181)
Net loss per share attributable to Vertex common shareholders:				
Basic	\$(0.68)	\$(0.26)	\$(1.68)	\$(1.67)
Diluted	\$(0.68)	\$(0.26)	\$(1.68)	\$(1.67)
Shares used in per share calculations:				
Basic	233,808	222,053	233,353	218,795
Diluted	233,808	222,053	233,353	218,795

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

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Condensed Consolidated Statements of Comprehensive Income (Loss)

(unaudited)

(in thousands)

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2014	2013	2014	2013
Net loss	\$(159,382)	\$(61,712)	\$(391,839)	\$(374,339)
Changes in other comprehensive loss:				
Unrealized holding gains (losses) on marketable securities	82	(170)	55	(159)
Unrealized losses on foreign currency forward contracts	(89)	—	(125)	—
Foreign currency translation adjustment	281	89	353	(521)
Total changes in other comprehensive loss	274	(81)	283	(680)
Comprehensive loss	(159,108)	(61,793)	(391,556)	(375,019)
Comprehensive loss attributable to noncontrolling interest (Alios)	—	4,547	—	9,158
Comprehensive loss attributable to Vertex	\$(159,108)	\$(57,246)	\$(391,556)	\$(365,861)

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

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Condensed Consolidated Balance Sheets

(unaudited)

(in thousands, except share and per share amounts)

	June 30, 2014	December 31, 2013
Assets		
Current assets:		
Cash and cash equivalents	\$420,558	\$569,299
Marketable securities, available for sale	798,603	895,777
Accounts receivable, net	81,842	85,517
Inventories	11,982	14,147
Prepaid expenses and other current assets	34,399	23,836
Total current assets	1,347,384	1,588,576
Restricted cash	129	130
Property and equipment, net	730,000	696,911
Goodwill	30,992	30,992
Other assets	9,315	2,432
Total assets	\$2,117,820	\$2,319,041
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	\$55,363	\$49,327
Accrued expenses	237,789	271,077
Deferred revenues, current portion	27,174	21,510
Accrued restructuring expenses, current portion	8,498	14,286
Capital lease obligations, current portion	19,707	16,893
Other liabilities, current portion	14,442	24,736
Total current liabilities	362,973	397,829
Deferred revenues, excluding current portion	38,105	49,459
Accrued restructuring expenses, excluding current portion	10,486	14,067
Capital lease obligations, excluding current portion	45,053	48,754
Construction financing lease obligation, excluding current portion	473,268	440,937
Other liabilities, excluding current portion	15,666	11,590
Total liabilities	945,551	962,636
Commitments and contingencies		
Shareholders' equity:		
Preferred stock, \$0.01 par value; 1,000,000 shares authorized; none issued and outstanding at June 30, 2014 and December 31, 2013	—	—
Common stock, \$0.01 par value; 300,000,000 shares authorized at June 30, 2014 and December 31, 2013; 237,331,086 and 233,788,852 shares issued and outstanding at June 30, 2014 and December 31, 2013, respectively	2,347	2,320
Additional paid-in capital	5,528,679	5,321,286
Accumulated other comprehensive loss	(23)	(306)
Accumulated deficit	(4,358,734)	(3,966,895)
Total shareholders' equity	1,172,269	1,356,405
Total liabilities and shareholders' equity	\$2,117,820	\$2,319,041

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

Table of Contents

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 (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 losses on marketable securities				(159)	)		(159)		(159)	
Foreign currency translation adjustment				(521)	)		(521)		(521)	
Net loss						(365,181)	(365,181)	(9,158)	(374,339)	
Issuance of common stock under benefit plans	6,614	68	213,733				213,801	(72)	213,729	
Convertible senior subordinated notes (due 2015)	8,276	83	402,182				402,265		402,265	
conversion										
Stock-based compensation expense			73,068				73,068	238	73,306	
Change in liquidation value of noncontrolling interest								(684)	(684)	684
Balance, June 30, 2013	232,177	\$2,300	\$5,208,431	\$(1,230)	)	\$(3,887,048)	\$1,322,453	\$186,996	\$1,509,449	\$39,214
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 securities				55			55		55	
				(125)	)		(125)		(125)	

Unrealized losses on foreign currency forward contracts										
Foreign currency translation adjustment				353		353		353		
Net loss					(391,839)	(391,839)		(391,839)		
Issuance of common stock under benefit plans	3,542	27	117,920			117,947		117,947		
Stock-based compensation expense			89,473			89,473		89,473		
Balance, June 30, 2014	237,331	\$2,347	\$5,528,679	\$(23)	\$(4,358,734)	\$1,172,269	\$—	\$1,172,269	\$—	

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

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Condensed Consolidated Statements of Cash Flows

(unaudited)

(in thousands)

	Six Months Ended June 30,	
	2014	2013
Cash flows from operating activities:		
Net loss	\$(391,839)	\$(374,339)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization expense	29,960	21,245
Stock-based compensation expense	89,024	72,625
Other non-cash based compensation expense	—	5,857
Intangible asset impairment charge	—	412,900
Deferred income taxes	—	(130,661)
Write-down of inventories to net realizable value	—	5,083
Other non-cash items, net	22	7,455
Changes in operating assets and liabilities:		
Accounts receivable, net	2,518	(18,462)
Inventories	1,194	6,620
Prepaid expenses and other assets	(17,538)	(18,152)
Accounts payable	7,671	(53,374)
Accrued expenses and other liabilities	(9,459)	4,616
Accrued restructuring expense	(9,369)	(1,276)
Deferred revenues	(5,866)	(6,842)
Net cash used in operating activities	(303,682)	(66,705)
Cash flows from investing activities:		
Purchases of marketable securities	(703,977)	(898,706)
Sales and maturities of marketable securities	801,206	830,906
Expenditures for property and equipment	(27,227)	(18,408)
Decrease in restricted cash and cash equivalents	1	31,812
Decrease in restricted cash and cash equivalents (Alios)	—	11,695
Decrease (increase) in deposits	(528)	414
Net cash provided by (used in) investing activities	69,475	(42,287)
Cash flows from financing activities:		
Issuances of common stock from employee benefit plans	117,947	207,872
Payments to redeem secured notes (due 2015)	—	(158)
Payments on capital lease obligations	(11,884)	(12,246)
Payments on construction financing lease obligation	(30,292)	(44,115)
Payments returned related to construction financing lease obligation	8,050	—
Net cash provided by financing activities	83,821	151,353
Effect of changes in exchange rates on cash	1,645	(521)
Net (decrease) increase in cash and cash equivalents	(148,741)	41,840
Cash and cash equivalents—beginning of period	569,299	489,407
Cash and cash equivalents—end of period	\$420,558	\$531,247
Supplemental disclosure of cash flow information:		
Cash paid for interest	\$31,933	\$7,142
Cash paid for income taxes	\$798	\$—
Conversion of convertible senior subordinated notes (due 2015) for common stock	\$—	\$399,842

Edgar Filing: VERTEX PHARMACEUTICALS INC / MA - Form 10-Q

Unamortized deferred debt issuance costs exchanged	\$—	\$4,230
Capitalization of costs related to construction financing lease obligation	\$25,564	\$130,222
Assets acquired under capital lease	\$8,985	\$21,576

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

Table of Contents

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. In addition, the condensed consolidated financial statements for the period from June 13, 2011 through December 31, 2013, reflect the operations of Alios BioPharma, Inc. ("Alios"), a collaborator that was a variable interest entity (a "VIE") for which the Company was deemed under applicable accounting guidance to have a variable interest and be the primary beneficiary. As of December 31, 2013, the Company deconsolidated Alios, and the Company's consolidated balance sheets as of June 30, 2014 and December 31, 2013 exclude Alios. All material intercompany balances and transactions have been eliminated. The Company operates in one segment, pharmaceuticals.

Certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted. These interim financial statements, in the opinion of management, reflect all normal recurring adjustments necessary for a fair presentation of the financial position and results of operations for the interim periods ended June 30, 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 and the income tax provision. 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 six months ended June 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

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

Company is evaluating the new guidance and the expected effect on the Company's condensed consolidated financial statements.

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 six months ended June 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	4,513	26,464	379	859	32,215
Adjustments related to prior period sales	(8	) 3,861	4,124	1	7,978
Credits/payments made	(5,271	) (57,617	) (4,160	) (1,586	) (68,634
Balance at June 30, 2014	\$769	\$40,952	\$16,142	\$829	\$58,692

C. Collaborative Arrangements**Janssen Pharmaceutica NV**

In 2006, the Company entered into a collaboration agreement (the "Janssen NV 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 NV 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 NV 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 NV Agreement through November 2013. As of November 2013, the effective date of the 2013 Janssen NV Amendment, there was \$32.1 million remaining in deferred revenues related to this up-front license payment.

Table of Contents

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 NV 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 NV Amendment was a material modification to the Janssen NV 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 NV 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 NV 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 June 30, 2014, the remaining deferred revenue balance related to the Janssen NV collaboration was \$4.2 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 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 six months ended June 30, 2014 and 2013, the Company recognized the following revenues attributable to the Janssen NV collaboration:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
	(in thousands)			
Royalty revenues (INCIVO)	\$5,698	\$44,070	\$10,633	\$83,114
Collaborative revenues:				
Up-front and amendment payments revenues	\$—	\$3,107	\$—	\$6,214
Net reimbursement for telaprevir development costs	1,483	37	2,872	9
Reimbursement for manufacturing services	—	—	—	10,299
Total collaborative revenues attributable to the Janssen NV collaboration	\$1,483	\$3,144	\$2,872	\$16,522
Total revenues attributable to the Janssen NV collaboration	\$7,181	\$47,214	\$13,505	\$99,636

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 six months ended June 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 financial support for (i) development activities for VX-661, a corrector compound discovered under the collaboration, and

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

(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 six months ended June 30, 2014 and 2013, the Company recognized the following revenues attributable to the CFFT collaboration:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
	(in thousands)			
Collaborative revenues attributable to the CFFT collaboration	\$1,604	\$4,244	\$4,472	\$7,803

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 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 in the first quarter of 2012 and began marketing KALYDECO in certain countries in the European Union in the third quarter of 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.

License and Collaboration Agreement

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. The Company does not expect to conduct any further development activities with respect to VX-135 and plans to seek to outlicense its rights to VX-135.

Under the terms of the Alios Agreement, the Company received exclusive worldwide rights to ALS-2200 (now formulated as VX-135) and ALS-2158, a second HCV nucleotide analogue discovered by Alios that was developed pursuant to the Alios agreement through the third quarter of 2012. Alios and the Company began clinical development of ALS-2200 (VX-135) and ALS-2158 in December 2011. The Company is responsible for all costs related to development, commercialization and manufacturing of each compound licensed to the Company pursuant to the Alios Agreement and provided funding to Alios to conduct the Phase 1 clinical trials for ALS-2200 and ALS-2158. In addition, the Company provided funding for a research program, which ended in 2013, directed to the discovery of

additional HCV nucleotide analogues that act on the HCV polymerase.

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

Upon entering into the Alios Agreement, the Company paid Alios a \$60.0 million up-front payment. As of June 30, 2014, Alios also had earned an aggregate of \$60.0 million in development milestone payments pursuant to the Alios Agreement. The Alios Agreement provides for development milestone payments if VX-135 is approved and commercialized. In addition, Alios is eligible to receive commercial milestone payments, as well as tiered royalties on net sales of VX-135.

The Company may terminate the Alios Agreement upon 60 days' notice to Alios. The Alios Agreement also may be terminated by either party for a material breach by the other, or if the Company challenges certain Alios patents, in each case subject to notice and cure provisions. Unless earlier terminated, the Alios Agreement will continue in effect until the expiration of the Company's royalty obligations, which expire on a country-by-country basis on the later of (a) the date the last-to-expire patent covering a licensed product expires or (b) 10 years after the first commercial sale in the applicable country.

Under applicable accounting guidance, the Company determined that Alios was a VIE, its license to VX-135 and ALS-2158 was a variable interest in Alios, that Alios was a business and that the Company was Alios' primary beneficiary for the period from June 13, 2011 through December 31, 2013. The Company based these determinations on, among other factors, the significance to Alios of the licensed compounds and on the Company's power, through the joint steering committee for the licensed compounds established under the Alios Agreement, to direct the activities that most significantly affect the economic performance of Alios.

Accordingly, the Company consolidated Alios' financial statements with the Company's condensed consolidated financial statements from June 13, 2011 through December 31, 2013. However, the Company's interests in Alios were limited to those accorded to the Company in the Alios Agreement. In particular, the Company did not acquire any equity interest in Alios, any interest in Alios' cash and cash equivalents or any control over Alios' activities that do not relate to the Alios Agreement. Alios does not have any right to the Company's assets except as provided in the Alios Agreement.

As of December 31, 2013, the Company determined that it 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. The Company deconsolidated Alios based on this conclusion as of December 31, 2013.

The Company continues to have significant continuing involvement with Alios due to the Alios Agreement, as amended; therefore, the deconsolidation of Alios is not presented as discontinued operations in the Company's condensed consolidated financial statements as of June 30, 2014. The Company will evaluate whether it continues to have significant continuing involvement with Alios for a period of one year from the December 31, 2013 deconsolidation date.

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. A summary of net loss attributable to noncontrolling interest (Alios) for the three and six months ended June 30, 2013 is as follows:

	Three Months Ended June 30, 2013 (in thousands)	Six Months Ended June 30, 2013
Loss before benefit from income taxes	\$6,824	\$12,121
Decrease in fair value of contingent milestone and royalty payments	80	2,820
Benefit from income taxes	(2,357)	(5,783)
Net loss attributable to noncontrolling interest (Alios)	\$4,547	\$9,158

The Company did not have a corresponding net loss (income) attributable to noncontrolling interest (Alios) for the three and six months ended June 30, 2014 due to the deconsolidation of Alios.

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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.

Janssen Pharmaceuticals, Inc.

On June 11, 2014, the Company entered into a license, development and commercialization agreement (the "Janssen Inc. Agreement") with Janssen Pharmaceuticals, Inc. ("Janssen Inc.") pursuant to which it granted Janssen Inc. an exclusive worldwide license to develop and commercialize VX-787 and a backup compound referred to as VX-353, for the treatment of influenza. In connection with the execution of the Janssen Inc. Agreement, the Company received from Janssen Inc. an up-front payment of \$30.0 million in the third quarter of 2014. In addition, Vertex has the potential to receive development and commercial milestone payments as well as royalties on any future product sales. Janssen Inc. will be responsible for costs related to the development and commercialization of the compounds. Janssen Inc. may terminate the Janssen Inc. Agreement, subject to certain exceptions, upon six months' notice. The Janssen Inc. Agreement also may be terminated by either party for a material breach by the other, subject to notice and cure provisions. Unless earlier terminated, the Janssen Inc. Agreement will continue in effect until the expiration of Janssen Inc.'s royalty obligations, which expire on a country-by-country basis on the later of (i) the date the last-to-expire patent covering a licensed product expires or (ii) ten years after the first commercial sale in the applicable country.

The Company will evaluate the deliverables pursuant to the Janssen Inc. Agreement under multiple element arrangement guidance for collaborative arrangements during the third quarter of 2014. The collaboration with Janssen Inc. 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 for the three and six months ended June 30, 2014.

D. Net Loss Per Share Attributable to Vertex Common Shareholders

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

	Three Months Ended June 30, 2014		Six Months Ended June 30, 2014	
	2013		2013	
	(in thousands)			
Stock options	14,549	16,802	14,549	16,802
Unvested restricted stock and restricted stock units	2,584	2,600	2,584	2,600

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

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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 June 30, 2014, the Company's investments were in money market funds, government-sponsored enterprise securities, corporate debt securities and commercial paper.

As of June 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 six months ended June 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:

	Fair Value Measurements as of June 30, 2014			
	Total (in thousands)	Fair Value Hierarchy		Level 3
		Level 1	Level 2	
Financial assets carried at fair value:				
Cash equivalents:				
Money market funds	\$ 123,469	\$ 123,469	\$—	\$—
Marketable securities:				
Government-sponsored enterprise securities	497,587	497,587	—	—
Commercial paper	69,243	—	69,243	—
Corporate debt securities	231,773	—	231,773	—
Total	\$922,072	\$621,056	\$301,016	\$—

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

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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 June 30, 2014				
Cash and cash equivalents:				
Cash and money market funds	\$420,558	\$—	\$—	\$420,558
Total cash and cash equivalents	\$420,558	\$—	\$—	\$420,558
Marketable securities:				
Government-sponsored enterprise securities (due within 1 year)	\$497,601	\$8	\$(22)	\$497,587
Commercial paper (due within 1 year)	69,144	99	—	69,243
Corporate debt securities (due within 1 year)	216,426	33	(26)	216,433
Corporate debt securities (due after 1 year through 5 years)	15,335	5	—	15,340
Total marketable securities	\$798,506	\$145	\$(48)	\$798,603
Total cash, cash equivalents and marketable securities	\$1,219,064	\$145	\$(48)	\$1,219,161

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

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 Losses on Foreign Currency Forward Contracts	Total
Balance at December 31, 2013	\$(325)	\$42	\$(23)	\$(306)
Other comprehensive income (loss) before reclassifications	353	55	(108)	300
Amounts reclassified from accumulated other comprehensive loss	—	—	(17)	(17)
	\$353	\$55	\$(125)	\$283

Net current period other comprehensive income
(loss)

Balance at June 30, 2014	\$28	\$97	\$(148	) \$(23	)
--------------------------	------	------	--------	---------	---

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

	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	(521)	) (159)	) —	(680)
Amounts reclassified from accumulated other comprehensive loss	—	—	—	—
Net current period other comprehensive loss	\$(521)	) \$(159)	) \$—	\$(680)
Balance at June 30, 2013	\$(1,267)	) \$37	\$—	\$(1,230)

H. Hedging

In December 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 included foreign currency forward contracts that were 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 June 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 June 30, 2014 (in thousands)	As of December 31, 2013
Foreign Currency		
Euro	\$23,334	\$17,468
British pound sterling	16,921	—
Total foreign currency forward contracts	\$40,255	\$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:

15

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

	As of June 30, 2014 (in thousands)	As of December 31, 2013
Fair value - assets	\$50	\$—
Fair value - liabilities	(198	) (23
Net carrying value	\$(148	) \$(23

I. Inventories

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

	As of June 30, 2014 (in thousands)	As of December 31, 2013
Raw materials	\$—	\$489
Work-in-process	11,013	9,981
Finished goods	969	3,677
Total	\$11,982	\$14,147

J. Intangible Assets and Goodwill

Intangible Assets

As of June 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. Two of the Company's competitors received approval in the fourth quarter of 2013 for new treatment regimens for HCV infection that include pegylated-interferon and ribavirin, and several of the Company's competitors are conducting Phase 3 clinical trials evaluating all-oral combinations of their drug candidates for the treatment of HCV infection.

ViroChem Acquisition

The Company determined that the fair value of the VX-222 intangible asset of \$412.9 million 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 six months ended June 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 six months ended June 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 \$1.30 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.

Goodwill

As of June 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 six months ended June 30, 2014 or 2013.

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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 leases, 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 in 2011.

Property and equipment, net, included \$522.2 million and \$503.4 million as of June 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 \$473.6 million and \$440.9 million as of June 30,

2014 and December 31, 2013, respectively.

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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 June 30, 2014:

Year	(in thousands)
2014	\$9,519
2015	20,792
2016	14,254
2017	13,129
2018	13,027
2019	3,047
Thereafter	—
Total payments	\$73,768
Less: amount representing interest	(9,008)
Present value of payments	\$64,760

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 September 2014.

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 certain 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 their equity awards as of June 30, 2014. The Company 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.

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

During the three and six months ended June 30, 2014 and 2013, the Company recognized the following stock-based compensation expense:

	Three Months Ended June 30, 2014		Six Months Ended June 30, 2014		2013	
	(in thousands)					
Stock-based compensation expense by type of award:						
Stock options	\$26,985	\$29,949	\$52,112	\$49,623		
Restricted stock and restricted stock units	14,020	9,732	33,013	19,110		
ESPP share issuances	1,681	2,051	4,348	4,573		
Less stock-based compensation expense capitalized to inventories	(242	) (382	) (449	) (681	)	)
Total stock-based compensation expense included in costs and expenses	\$42,444	\$41,350	\$89,024	\$72,625		

Stock-based compensation expense by line item:

Research and development expenses	\$27,253	\$25,740	\$60,153	\$45,089
Sales, general and administrative expenses	15,191	15,610	28,871	27,536
Total stock-based compensation expense included in costs and expenses	\$42,444	\$41,350	\$89,024	\$72,625

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 June 30, 2014	
	Unrecognized Expense, Net of Estimated Forfeitures (in thousands)	Weighted-average Recognition Period (in years)
Type of award:		
Stock options	\$158,362	2.33
Restricted stock and restricted stock units	\$102,006	2.14
ESPP share issuances	\$4,082	0.58

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

	Options Outstanding			Options Exercisable		
Range of Exercise Prices	Number Outstanding (in thousands)	Weighted-average Remaining Contractual Life (in years)	Weighted-average Exercise Price (per share)	Number Exercisable (in thousands)	Weighted-average Exercise Price (per share)	
\$10.41–\$20.00	368	2.01	\$15.59	368	\$15.59	
\$20.01–\$30.00	889	5.42	\$29.46	708	\$29.35	
\$30.01–\$40.00	4,876	5.17	\$36.20	3,758	\$35.73	
\$40.01–\$50.00	3,508	8.37	\$46.32	890	\$46.55	
\$50.01–\$60.00	1,348	7.34	\$54.11	800	\$54.63	
\$60.01–\$70.00	129	9.15	\$65.71	20	\$64.33	
\$70.01–\$80.00	2,095	9.60	\$76.73	308	\$74.18	
\$80.01–\$88.18	1,336	9.02	\$83.11	375	\$82.53	
Total	14,549	7.10	\$49.77	7,227	\$41.65	

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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 June 30, 2014, the Company had \$53.9 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 six months ended June 30, 2014, the Company recorded a net provision for income taxes of \$0.7 million and \$1.5 million, respectively, related to state income taxes and income earned in various foreign jurisdictions. For the three and six months ended June 30, 2013, the Company recorded a benefit from income taxes of \$1.8 million and \$132.1 million, respectively. The benefit from income taxes in the six months ended June 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 June 30, 2014 and December 31, 2013, the Company had unrecognized tax benefits of \$3.0 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 June 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 June 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 2010 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. The Company concluded an 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 June 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

20

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

Company has not used more than 50% of this space since it adopted the plan to restructure its operations in 2003. This unused laboratory and office space currently is subleased to third parties.

The activities related to the restructuring liability for the three and six months ended June 30, 2014 and 2013 were as follows:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
	(in thousands)			
Liability, beginning of the period	\$18,324	\$22,459	\$19,115	\$23,328
Cash payments	(3,960)	(3,849)	(7,822)	(7,422)
Cash received from subleases	2,689	2,666	5,378	5,331
Restructuring (income) expense	(2,117)	776	(1,735)	815
Liability, end of the period	\$14,936	\$22,052	\$14,936	\$22,052
Fan Pier Move Restructuring				

In connection with the relocation of its Massachusetts operations to Fan Pier in Boston, Massachusetts, the Company is incurring restructuring charges related to its remaining lease obligations at its facilities in Cambridge, Massachusetts, which will 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 Company started incurring these charges in the fourth quarter of 2013 and expects them to continue through April 2018. The majority of these restructuring charges relate to cease use charges that the Company expects to incur in the third quarter of 2014 once it has vacated the buildings in Cambridge in their entirety. Once the Company completes the relocation, the continuing charges will relate to the difference between the Company's estimated future cash flows related to its lease obligations and its actual cash flows.

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

	Three Months Ended	Six Months Ended June
	June 30, 2014	30, 2014
	(in thousands)	
Liability, beginning of the period	\$3,722	\$797
Cash payments	(2,143)	(4,377)
Restructuring expense	1,677	6,836
Liability, end of the period	\$3,256	\$3,256
Strategic Restructuring		

In October 2013, the Company adopted a restructuring plan. The restructuring plan 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.

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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

	Three Months Ended June 30, 2014	Six Months Ended June 30, 2014
	(in thousands)	
Liability, beginning of the period	\$ 1,821	\$ 8,441
Cash payments	(1,199	) (8,466
Restructuring expense	170	817
Liability, end of the period	\$ 792	\$ 792

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 for the three and six months ended June 30, 2014 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

City of Bristol Pension Fund v. Vertex Pharmaceuticals Incorporated, et al.

On September 6, 2012, a purported shareholder class action, City of Bristol Pension Fund 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 June 28, 2012, all in violation of Section 10(b) of the Securities Exchange Act of 1934, as amended, and Rule 10b-5 promulgated thereunder. By order dated December 12, 2012, the court appointed the City of Bristol lead plaintiff and appointed the City of Bristol's attorneys lead counsel. The plaintiffs filed an amended complaint on February 11, 2013. The Company filed a motion to dismiss the complaint on April 12, 2013. On May 28, 2013, the plaintiffs filed an opposition to the Company's motion to dismiss the complaint. On June 27, 2013, the Company filed a reply in further support of the Company's motion to dismiss the plaintiffs' complaint. The court conducted a hearing on the Company's motion to dismiss on November 25, 2013, and the court dismissed the plaintiffs' complaint on March 31, 2014. The plaintiffs filed a motion (i) for reconsideration and (ii) to file a second amended complaint on April 28, 2014. On May 23, 2014, the court denied the plaintiffs' motion and dismissed the complaint with prejudice.

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. The Company believes the claims to be

without merit and intends to vigorously defend the litigation. As of June 30, 2014, the Company has not recorded any reserves for this purported class action.

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

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

U. Subsequent Events

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. 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 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, or 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 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 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

Table of Contents

VERTEX PHARMACEUTICALS INCORPORATED

Notes to Condensed Consolidated Financial Statements

(unaudited)

Company may prepay the loans, 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.

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.

Table of Contents

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 positive 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. We plan to submit a New Drug Application, or NDA, to 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 in the fourth quarter of 2014.

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 and Europe are being treated with KALYDECO. In June 2014, we signed a letter of intent with the pan-Canadian Pricing Alliance to enable the public reimbursement of KALYDECO for the treatment of eligible Canadians with CF six years of age and older who have the G551D mutation in their CFTR gene. Patients in the Canadian provinces of Ontario and Alberta are now able to receive KALYDECO under public reimbursement programs, and discussions are ongoing in the remaining Canadian provinces and territories. KALYDECO also is approved in Australia for the treatment of patients with CF six years of age and older who have the G551D mutation in their CFTR gene, and we are in discussions with Australia's Therapeutic Goods Administration regarding public reimbursement of KALYDECO.

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. In Canada, we also recently received approval for KALYDECO for the treatment of this patient group and patients with CF who have the G970R mutation in their CFTR gene.

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.

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 ppFEV₁. 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 in June 2014 and an MAA variation to the EMA in July 2014 seeking approval of KALYDECO in patients with CF 18 years of age and older who have the R117H mutation on at least one allele in their CFTR gene. We believe there are approximately 700 patients with CF 18 years of age or older who have the R117H mutation in their CFTR gene in North America,

Europe and Australia.

Our 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, is complete, and we expect data from this clinical trial in the third quarter of 2014. The primary endpoint of this clinical trial is safety, and secondary endpoints

Table of Contents

include pharmacokinetics, change in sweat chloride and change in weight. If this clinical trial is successful, we plan to submit an NDA and an MAA variation based on this clinical trial in the fourth quarter of 2014. We believe there are approximately 300 children with CF two to five years of age who have the mutations evaluated in this clinical trial in North America, Europe and Australia.

In the second quarter of 2014, we announced data from a two-part proof-of-concept clinical trial of ivacaftor in 24 patients with CF who have a residual function mutation. This clinical trial was the first to evaluate the use of ivacaftor in multiple residual function mutations in their CFTR gene. Based on the data from the clinical trial, we plan to initiate a larger Phase 3 clinical trial in patients with residual function mutations that will evaluate longer-duration treatment with ivacaftor, subject to discussions with regulatory authorities. In North America, Europe and Australia, more than 3,000 patients with CF six years of age and older have non-R117H mutations that result in residual function.

Lumacaftor in Combination with Ivacaftor

We are evaluating combinations of lumacaftor and ivacaftor, our most advanced investigational CFTR corrector compound. 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. Based on the data from TRAFFIC and TRANSPORT, we plan to submit an NDA to the FDA and an MAA to the EMA for lumacaftor in combination with ivacaftor in patients with CF 12 years of age and older who have two copies (homozygous) of the F508del mutation in their CFTR gene in the fourth quarter of 2014. In June 2014, the FDA granted the combination of lumacaftor and ivacaftor Orphan Drug Designation. The combination of lumacaftor and ivacaftor also recently received Orphan designation in Europe. We believe that there are more than 22,000 patients with CF 12 years of age and older who have two copies of the F508del mutation in North America, Europe and Australia.

TRAFFIC and TRANSPORT

TRAFFIC and TRANSPORT evaluated patients with CF 12 years of age and older who have two copies (homozygous) of the F508del mutation in their CFTR gene and included two combination treatment groups and one placebo group. The combination treatment groups evaluated lumacaftor dosed at either 600 mg once daily or 400 mg every 12 hours (q12h) in combination with ivacaftor dosed at 250 mg q12h. 1,108 patients enrolled and received at least one dose of study drug in the two clinical trials. The primary endpoint of TRAFFIC and TRANSPORT was the mean absolute change from baseline in ppFEV₁ at the end of the 24-week treatment period as assessed by the average change in lung function at Week 16 and at Week 24.

All four treatment arms within TRAFFIC and TRANSPORT met their primary endpoint. Additionally, statistically significant mean absolute and relative improvements in lung function were observed for all four treatment groups, both within group and versus placebo, at all time points within the clinical trials (Weeks 2, 4, 8, 16 and 24). As patients in the clinical trials continued to be treated with their standard CF medicines, improvements observed for patients in the combination treatment arms were in addition to any benefits experienced with the use of other CF medicines. The mean baseline lung function of patients was approximately 61 ppFEV₁ for patients who received the combination regimen and for patients who received placebo. Detailed data from each arm of TRAFFIC and TRANSPORT are provided below:

		TRAFFIC Trial		TRANSPORT Trial	
Change in ppFEV ₁	Treatment Difference	Placebo (n=184)	Lumacaftor (600 mg once daily) + Ivacaftor (250 mg q12h) (n=183)	Placebo (n=187)	Lumacaftor (600 mg once daily) + Ivacaftor (250 mg q12h) (n=185)
			Lumacaftor (400 mg q12h) + Ivacaftor (250 mg q12h) (n=182)		Lumacaftor (400 mg q12h) + Ivacaftor (250 mg q12h) (n=187)
Mean Absolute Change		N/A	4.0 (p<0.0001)	N/A	2.6 (p=0.0003)
			3.6		2.5
					3.0 (p<0.0001)
					2.9

Edgar Filing: VERTEX PHARMACEUTICALS INC / MA - Form 10-Q

(percentage Within points)	Group	-0.44 (p=0.4002)	(p<0.0001)	(p<0.0001)	-0.15 (p=0.7744)	(p<0.0001)	(p<0.0001)
Mean	Treatment	N/A	6.7%	4.3%	N/A	4.4%	5.3%
Relative	Difference	N/A	(p<0.0001)	(p=0.0006)	N/A	(p=0.0007)	(p<0.0001)
Change	Within	-0.34%	6.4%	4.0%	0.0%	4.4%	5.3%
(%)	Group	(p=0.7113)	(p<0.0001)	(p<0.0001)	(p=0.9983)	(p<0.0001)	(p<0.0001)

Within TRAFFIC and TRANSPORT, patients who received the combination regimens experienced a 28 to 43 percent decrease in the rate of pulmonary exacerbations (events of worsening signs and symptoms of the disease requiring

Table of Contents

treatment with antibiotics) over the 24-week treatment period compared to placebo. Detailed data for all key secondary endpoints from each arm of the clinical trials are provided below:

		TRAFFIC Trial			TRANSPORT Trial		
Key Secondary Endpoints		Placebo (n=184)	Lumacaftor (600 mg once daily) + Ivacaftor (250 mg q12h) (n=183)	Lumacaftor (400 mg q12h) + Ivacaftor (250 mg q12h) (n=182)	Placebo (n=187)	Lumacaftor (600 mg once daily) + Ivacaftor (250 mg q12h) (n=185)	Lumacaftor (400 mg q12h) + Ivacaftor (250 mg q12h) (n=187)
Change in Body Mass Index	Treatment Difference	N/A	+0.16 (p=0.1122)	+0.13 (p=0.1938)	N/A	+0.41 (p<0.0001)	+0.36 (p=0.0001)
	Within Group	+0.19 (p=0.0065)	+0.35 (p<0.0001)	+0.32 (p<0.0001)	+0.07 (p=0.2892)	+0.48 (p<0.0001)	+0.43 (p<0.0001)
	Treatment Difference	N/A	+3.9 (p=0.0168)	+1.5 (p=0.3569)	N/A	+2.2 (p=0.1651)	+2.9 (p=0.0736)
Change in CFQ-R	Within Group	+1.1 (p=0.3423)	+5.0 (p<0.0001)	+2.6 (p=0.0295)	+2.8 (p=0.0152)	+5.0 (p<0.0001)	+5.7 (p<0.0001)
	Patients with 5% or Greater Relative Improvement in ppFEV ₁	% 22%	46%	37%	23%	46%	41%
Number of Pulmonary Exacerbations	Odds Ratio	N/A	2.94 (p<0.0001)	2.06 (p=0.0023)	N/A	2.96 (p<0.0001)	2.38 (p=0.0001)
	Number of Events (rate per 48 weeks)	112 (1.07)	79 (0.77)	73 (0.71)	139 (1.18)	94 (0.82)	79 (0.67)
	Rate Ratio	N/A	0.72 (p=0.0491)	0.66 (p=0.0169)	N/A	0.69 (p=0.0116)	0.57 (p=0.0002)

The combination regimens were generally well tolerated. The most common adverse events, regardless of treatment group, were infective pulmonary exacerbation, cough, headache and increased sputum, and adverse events that occurred more frequently in patients who received the combination regimens than those who received placebo were generally respiratory in nature and included dyspnea and respiration abnormal. 4.2 percent of all patients who received combination therapy, regardless of dosing group, discontinued treatment because of adverse events compared to 1.6 percent of those who received placebo. Across TRAFFIC and TRANSPORT, elevated liver enzymes (greater than three times the upper limit of normal) were observed in 5.2 percent of patients who received combination therapy compared to 5.1 percent of those who received placebo. Seven patients who received combination therapy experienced serious adverse events related to abnormal liver function tests, compared to zero patients who received placebo. Following discontinuation or interruption of the combination treatment, liver function tests returned to baseline for six of the seven patients and the seventh patient's liver function tests improved substantially.

Exploratory Clinical Trial in Patients Heterozygous for the F508del Mutation

In the third quarter of 2014, we completed a Phase 2, 8-week exploratory clinical trial of lumacaftor in combination with ivacaftor in 125 patients with CF 18 years of age and older who have one copy (heterozygous) of the F508del mutation and a second mutation in their CFTR gene that is not expected to respond to either ivacaftor or VX-809 alone.

The clinical trial evaluated a twice daily (q12h) combination of VX-809 (400mg) and ivacaftor (250mg) compared to placebo. The primary endpoints were safety, tolerability and mean absolute change in ppFEV₁ from baseline at Day 56, and key secondary endpoints included absolute change in body mass index (BMI), absolute change in patient-reported respiratory symptoms as reported in the CF questionnaire-revised (CFQ-R) and absolute change in

sweat chloride, among others.

In the clinical trial, the within-group mean absolute change in ppFEV₁ for the patients who received the combination regimen was -0.62 percentage points (p=0.4550) compared to -1.23 percentage points (p=0.1287) for those who received placebo. The mean absolute treatment difference was 0.61 percentage points (p=0.5978) at day 56. The clinical trial did not meet its primary efficacy endpoint. For patients who received the combination, the mean absolute improvement in CFQ-R at day 56 was +6.48 points (p=0.0131) versus placebo. Additionally, there was a -11.03 mmol/L (p < 0.0001) decrease in sweat chloride at day 56 for those who received the combination compared to those who received placebo. There was no increase observed in body mass index, or BMI.

Table of Contents

Safety results from this clinical trial were consistent with the Phase 3 TRAFFIC and TRANSPORT clinical trials in patients with two copies of the F508del mutation. The combination regimen was generally well tolerated. The most common adverse events, regardless of treatment group, were respiration abnormal, infective pulmonary exacerbation, cough, increased sputum and headache, and adverse events that occurred more frequently in patients who received the combination regimen than those who received placebo were generally respiratory in nature and included dyspnea and respiration abnormal, as well as gastroesophageal reflux. 6.5 percent of patients who received combination therapy discontinued treatment because of adverse events compared to 0.0 percent of those who received placebo.

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. In this clinical trial, VX-661 was generally well-tolerated when dosed in combination with KALYDECO, and all 18 patients completed the clinical trial. The most common adverse events in the treatment group were cough, pulmonary exacerbation, headache and upper respiratory tract infection. One serious adverse event of arthritis occurred in the VX-661 treatment arm and was deemed unrelated to VX-661 or KALYDECO.

The baseline lung function and sweat chloride levels for patients who were randomized to receive VX-661 and KALYDECO were 59.1 ppFEV₁ and 52.9 mmol/L, respectively. A summary of the lung function and sweat chloride data for patients who received VX-661 in combination with KALYDECO is provided below:

VX-661 + KALYDECO (Within-Group; n=14)	Day 0 Through Day 28 (End of VX-661 Treatment)	Day 28 to Day 56 (4 Weeks Following the End of VX-661 Treatment)
Mean Absolute Change in Lung Function (ppFEV ₁)	+4.6 percentage points (p=0.012)	-3.4 percentage points (p=0.010)
Mean Relative Change in Lung Function (ppFEV ₁)	+7.3% (p=0.012)	-5.4% (p=0.008)
Sweat Chloride	-7.02 mmol/L (p=0.053)	+12.26 mmol/L (p=0.001)

Additional clinical trials of longer duration and with additional patients will be required to further validate the results of this clinical trial in patients with CF who have the G551D mutation in their CFTR gene.

We are dosing patients in a 12-week clinical trial of VX-661 in combination with ivacaftor in patients with CF who are homozygous for 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 evaluate multiple development pathways for VX-661 in combination with ivacaftor, including the potential evaluation of this combination in patients with one copy of the F508del mutation and one copy of a mutation in their CFTR gene known to respond to ivacaftor and in patients with CF who have two copies of the F508del mutation. Additionally, VX-661 and ivacaftor may be evaluated in combination with, or without, a next-generation corrector in patients with one copy of the F508del mutation and a mutation that is not expected to respond to ivacaftor or a first-generation corrector alone.

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 2012 and 2013, we recognized most of our product revenues based on INCIVEK sales and focused a large portion of our resources on commercializing INCIVEK and seeking to develop other drug candidates for the treatment of HCV infection. Our INCIVEK net product revenues declined rapidly over the course of 2013 and represented approximately 8% of our net product revenues in the second quarter of 2014. In 2013, in response to declining sales of INCIVEK and increased competition, we reduced our focus on marketing INCIVEK, eliminating the U.S. field-based sales force that had

Table of Contents

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 April 2014, we amended our collaboration with Alios BioPharma, Inc., or Alios, and following this amendment, we have no further obligations to continue development of VX-135. We do not plan to further develop VX-135 independently and are seeking to outlicense our rights to VX-135.

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, multiple sclerosis 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 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 priority as new information becomes available and 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 the promotion of a culture of compliance. Among other laws, regulations and standards, we are subject to various U.S. federal and state 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.

Recent Financing and Business Development Activities

Business Development

In June 2014, we entered into a license, development and commercialization agreement with Janssen Pharmaceuticals, Inc., or Janssen Inc., pursuant to which we granted Janssen Inc. an exclusive worldwide license to develop and commercialize VX-787 and a backup compound referred to as VX-353, for the treatment of influenza.

Table of Contents

Pursuant to this agreement, Janssen Inc. paid us an up-front payment of \$30.0 million in the third quarter of 2014. In addition, we have the potential to receive development and commercial milestone payments as well as royalties on any future product sales. Janssen Inc. is responsible for costs related to the development and commercialization of the compounds.

We are seeking to license or acquire drugs, drug candidates and other technologies that have the potential to add to our pipeline, enhance research and development programs or to provide us with new commercial opportunities. We also are planning to seek to outlicense our rights to VX-135 and VX-509, our JAK3 inhibitor, which we have evaluated as a potential treatment for patients with rheumatoid arthritis in a Phase 2 clinical trial.

Credit Agreement

In July 2014, we entered into a credit agreement that provides for a \$300.0 million senior secured term loan. The credit agreement also provides that, subject to satisfaction of certain conditions, we may request that the lenders establish an incremental senior secured term loan facility in an aggregate amount not to exceed \$200.0 million. The loan initially bears interest at a rate of 7.2% per annum but is subject to adjustment over the course of the loan and matures in July 2017. Interest is payable quarterly and on the maturity date. 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 the maturity date.

Table of Contents

RESULTS OF OPERATIONS

	Three Months Ended		Increase/(Decrease)			Six Months Ended		Increase/(Decrease)	
	June 30, 2014 (in thousands)	2013	\$	%		June 30, 2014 (in thousands)	2013	\$	%
Revenues	\$138,421	\$310,750	\$(172,329)	(55)%		\$256,872	\$639,118	\$(382,246)	(60)%
Operating costs and expenses	\$319,256	\$367,683	\$(48,427)	(13)%		\$654,095	\$1,134,339	\$(480,244)	(42)%
Other items, net	\$21,453	\$(4,779)	N/A	N/A		\$5,384	\$120,882	\$(115,498)	(96)%
Net loss attributable to noncontrolling interest (Alios)	\$—	\$4,547	\$(4,547)	(100)%		\$—	\$9,158	\$(9,158)	(100)%
Net loss attributable to Vertex	\$(159,382)	\$(57,165)	\$102,217	179%		\$(391,839)	\$(365,181)	\$26,658	7%
Net Loss Attributable to Vertex									

Net loss attributable to Vertex was \$159.4 million in the second quarter of 2014 compared to a net loss attributable to Vertex of \$57.2 million in the second quarter of 2013. Our revenues decreased in the second quarter of 2014 as compared to the second quarter of 2013 due to decreased INCIVEK net product revenues partially offset by increased KALYDECO net product revenues. Our operating costs and expenses decreased in the second quarter of 2014 as compared to the second quarter of 2013 primarily due to reductions in sales, general and administrative expenses and cost of product revenues.

Net loss attributable to Vertex was \$391.8 million in the first half of 2014 compared to net loss attributable to Vertex of \$365.2 million in the first half of 2013. Our revenues decreased in the first half of 2014 as compared to the first half of 2013 due to decreased INCIVEK net product revenues partially offset by increased KALYDECO net product revenues. Our operating costs and expenses decreased from \$1.1 billion in the first half of 2013 to \$654.1 million in the first half of 2014. The decrease in operating expenses in the first half of 2014 compared to the first half of 2013 was primarily due to a \$412.9 million impairment charge related to VX-222, a non-nucleoside HCV polymerase inhibitor, we recorded in the first quarter of 2013, which was included in operating costs and expenses. 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 first half of 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.

Net Loss Attributable to Vertex per Diluted Share

Net loss attributable to Vertex was \$0.68 per diluted share in the second quarter of 2014 as compared to net loss attributable to Vertex of \$0.26 per diluted share in the second quarter of 2013. Net loss attributable to Vertex was \$1.68 per diluted share in the first half of 2014 as compared to net loss attributable to Vertex of \$1.67 per diluted share in the first half of 2013.

Table of Contents

Revenues

	Three Months Ended		Increase/(Decrease)			Six Months Ended		Increase/(Decrease)		
	June 30,					June 30,				
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
Product revenues, net	\$122,319	\$254,789	\$(132,470)	(52)	%	\$225,780	\$522,170	\$(296,390)	(57)	%
Royalty revenues	13,015	49,120	(36,105)	(74)	%	23,748	92,693	(68,945)	(74)	%
Collaborative revenues	3,087	6,841	(3,754)	(55)	%	7,344	24,255	(16,911)	(70)	%
Total revenues	\$138,421	\$310,750	\$(172,329)	(55)	%	\$256,872	\$639,118	\$(382,246)	(60)	%

Product Revenues, Net

	Three Months Ended		Increase/(Decrease)			Six Months Ended		Increase/(Decrease)		
	June 30,					June 30,				
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
KALYDECO	\$113,055	\$98,973	\$14,082	14	%	\$212,570	\$160,800	\$51,770	32	%
INCIVEK	9,264	155,816	(146,552)	(94)	%	13,210	361,370	(348,160)	(96)	%
Total product revenues, net	\$122,319	\$254,789	\$(132,470)	(52)	%	\$225,780	\$522,170	\$(296,390)	(57)	%

Our total net product revenues decreased in the second quarter and first half of 2014 as compared to the second quarter and first half of 2013 due to decreased INCIVEK net product revenues, partially offset by increased KALYDECO net product revenues.

We began marketing KALYDECO in the United States in the first quarter of 2012 and in certain international markets in the third quarter of 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 \$113.1 million in the second quarter of 2014 including \$50.0 million of net product revenues from international markets and were \$212.6 million in the first half of 2014, including \$93.9 million of net product revenues from international markets. The increase in KALYDECO net product revenues in the second quarter of 2014 compared to the second quarter of 2013 was primarily due to additional patients being treated with KALYDECO as a result of label-expansion. The increase in KALYDECO net product revenues in the first half of 2014 as compared to the first half of 2013 was primarily due to additional European countries beginning to provide reimbursement for KALYDECO in the second quarter of 2013. 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.

We plan to submit an NDA to the FDA and an MAA to the EMA for lumacaftor in combination with ivacaftor in the fourth quarter of 2014. 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 been declining over the past two years and were \$9.3 million in the second quarter of 2014 and \$13.2 million in the first half of 2014. In future periods, we expect INCIVEK net product revenues to continue to represent a small portion of our total net product revenues.

Royalty Revenues

Our royalty revenues were \$13.0 million and \$23.7 million, respectively, in the second quarter and first half of 2014 compared to \$49.1 million and \$92.7 million, respectively, in the second quarter and first half of 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 royalties on net sales of INCIVO terminated, subject to the continued payment of certain third-party royalties on its

net sales of INCIVO.

Table of Contents

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.

Collaborative Revenues

	Three Months Ended June 30,		Six Months Ended June 30,	
	2014	2013	2014	2013
	(in thousands)		(in thousands)	
Janssen NV	\$1,483	\$3,144	\$2,872	\$16,522
CFFT and other	1,604	3,697	4,472	7,733
Total collaborative revenues	\$3,087	\$6,841	\$7,344	\$24,255

Our collaborative revenues for the second quarter and the first half of 2014 related to net reimbursements from Janssen NV for our remaining telaprevir development costs and research funding provided by CFFT. Our collaborative revenues from Janssen NV for the first half of 2013 included \$10.3 million in reimbursements for manufacturing services. We do not expect to recognize significant collaborative revenues related to the Janssen NV collaboration in future periods.

In June 2014, we entered into an agreement with Janssen Inc. pursuant to which we outlicensed VX-787 to Janssen, Inc. We expect to recognize collaborative revenues related to this agreement in the second half of 2014.

Operating Costs and Expenses

	Three Months Ended June 30,		Increase/(Decrease)		Six Months Ended June 30,		Increase/(Decrease)	
	2014	2013	\$	%	2014	2013	\$	%
	(in thousands)				(in thousands)			
Cost of product revenues	\$9,655	\$24,695	\$(15,040)	(61)%	\$18,227	\$55,650	\$(37,423)	(67)%
Royalty expenses	7,645	13,236	\$(5,591)	(42)%	14,549	25,024	\$(10,475)	(42)%
Research and development expenses	224,780	222,455	\$2,325	1%	463,743	440,550	\$23,193	5%
Sales, general and administrative expenses	77,446	106,521	\$(29,075)	(27)%	151,658	199,400	\$(47,742)	(24)%
Restructuring expense (credit)	(270)	776	N/A	N/A	5,918	815	\$5,103	626%
Intangible asset impairment charge	—	—	N/A	N/A	—	412,900	\$(412,900)	(100)%
Total costs and expenses	\$319,256	\$367,683	\$(48,427)	(13)%	\$654,095	\$1,134,339	\$(480,244)	(42)%

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 second quarter of 2014 as compared to the second quarter of 2013 and in the first half of 2014 as compared to the first half of 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 first half of 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 second quarter of 2014 decreased by \$5.6 million, or 42%, as compared to the second quarter of 2013, and decreased by \$10.5 million, or 42% in the first half of 2014 as compared to the first half of 2013 as a result of decreased INCIVO sales by Janssen NV.

Table of Contents

Research and Development Expenses

	Three Months Ended		Increase/(Decrease)			Six Months Ended		Increase/(Decrease)		
	June 30, 2014 (in thousands)	2013	\$	%		June 30, 2014 (in thousands)	2013	\$	%	
Research expenses	\$65,342	\$64,740	\$602	1	%	\$132,365	\$126,083	\$6,282	5	%
Development expenses	159,438	157,715	1,723	1	%	331,378	314,467	16,911	5	%
Total research and development expenses	\$224,780	\$222,455	\$2,325	1	%	\$463,743	\$440,550	\$23,193	5	%

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

To date, we have incurred \$6.9 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. We received data from a Phase 3 clinical development program evaluating lumacaftor in combination with ivacaftor in June 2014 and plan to submit an NDA to the FDA and an MAA to the EMA in the fourth quarter of 2014. 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 until at least mid-2015. We cannot make a meaningful estimate when, if ever, our other clinical development programs will generate revenues and cash flows.

Research Expenses

	Three Months Ended		Increase/(Decrease)			Six Months Ended		Increase/(Decrease)		
	June 30, 2014 (in thousands)	2013	\$	%		June 30, 2014 (in thousands)	2013	\$	%	
Research Expenses:										
Salary and benefits	\$20,983	\$22,935	\$(1,952)	(9)	%	\$41,386	\$44,595	\$(3,209)	(7)	%
Stock-based compensation	8,837	7,849	988	13	%	20,891	14,675	6,216	42	%

expense										
Laboratory										
supplies and other	11,974	11,425	549	5	%	22,701	22,075	626	3	%
direct expenses										
Contractual										
services	3,604	5,609	(2,005)	(36)	)%	6,664	11,256	(4,592)	(41)	)%
Infrastructure costs	19,944	16,922	3,022	18	%	40,723	33,482	7,241	22	%
Total research										
expenses	\$65,342	\$64,740	\$602	1	%	\$132,365	\$126,083	\$6,282	5	%

Table of Contents

We maintain a substantial investment in research activities. Our research expenses in the second quarter and the first half of 2014 were consistent with our research expenses for the second quarter and the first half of 2013. Increased infrastructure costs and stock-based compensation expenses were largely offset by decreased salary and benefits and contractual services expenses in the 2014 periods as compared to the 2013 periods. 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 June 30,		Increase/(Decrease)			Six Months Ended June 30,		Increase/(Decrease)		
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
Development Expenses:										
Salary and benefits	\$38,772	\$45,248	\$(6,476)	(14)%		\$80,734	\$88,395	\$(7,661)	(9)%	
Stock-based compensation expense	18,416	17,891	525	3%		39,262	30,414	8,848	29%	
Laboratory supplies and other direct expenses	21,385	10,563	10,822	102%		35,870	21,527	14,343	67%	
Contractual services	48,322	50,422	(2,100)	(4)%		108,058	104,962	3,096	3%	
Drug supply costs	1,557	5,376	(3,819)	(71)%		4,524	14,976	(10,452)	(70)%	
Infrastructure costs	30,986	28,215	2,771	10%		62,930	54,193	8,737	16%	
Total development expenses	\$159,438	\$157,715	\$1,723	1%		\$331,378	\$314,467	\$16,911	5%	

Our development expenses increased by \$1.7 million, or 1%, in the second quarter of 2014 as compared to the second quarter of 2013, and increased by \$16.9 million, or 5%, in the first half of 2014 as compared to the first half of 2013. We expect that our development expenses will decrease in the second half of 2014 due to the completion in the first half of 2014 of our Phase 3 clinical development program for ivacaftor in combination with lumacaftor.

Sales, General and Administrative Expenses

	Three Months Ended June 30,		Increase/(Decrease)			Six Months Ended June 30,		Increase/(Decrease)		
	2014	2013	\$	%		2014	2013	\$	%	
	(in thousands)					(in thousands)				
Sales, general and administrative expenses	\$77,446	\$106,521	\$(29,075)	(27)%		\$151,658	\$199,400	\$(47,742)	(24)%	

Sales, general and administrative expenses decreased by 27% in the second quarter of 2014 as compared to the second quarter of 2013 and decreased by 24% in the first half of 2014 as compared to the first half of 2013, due primarily to decreased headcount following our October 2013 restructuring activities.

Restructuring Expense

We recorded a restructuring credit of \$0.3 million in the second quarter of 2014 as compared to restructuring expenses of \$0.8 million in the second quarter of 2013 and restructuring expenses of \$5.9 million in the first half of 2014 as compared to restructuring expenses of \$0.8 million in the first half of 2013. Our restructuring expenses in the first half of 2014 primarily related to the relocation of our corporate headquarters to Boston, Massachusetts from Cambridge, Massachusetts. We expect to incur approximately \$50 million in additional restructuring charges, primarily related to the relocation of our corporate headquarters, during the remainder of 2014.

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 first half of 2013. We did not record any intangible asset impairment charges in the second quarter or first half of 2014.

Table of Contents

Other Items

Interest Expense, net

Interest expense, net was \$15.6 million and \$31.3 million in the second quarter and first half of 2014, respectively, compared to \$6.6 million and \$10.0 million in the second quarter and first half of 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. We expect to incur approximately \$30 million of additional interest expense associated with the leases for our corporate headquarters during the second half of 2014. In addition, we expect that our interest expense, net will increase in the second half of 2014 as compared to the first half of 2014 due to approximately \$10 million in interest expense that we will incur in the second half of 2014 based on the \$300.0 million we borrowed in July 2014 pursuant to our credit agreement.

Other Income (Expense), net

Other income (expense), net was \$37.7 million and \$38.2 million in the second quarter and first half of 2014, respectively, compared to \$27 thousand and \$1.2 million in the second quarter and first half of 2013, respectively. Other income (expense), net in the second quarter and first half of 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 net provision for income taxes of \$0.7 million and \$1.5 million in the second quarter and first half of 2014, respectively. In the second quarter of 2013, we recorded a net benefit from income taxes of \$1.8 million. In the first half of 2013, we recorded a benefit from income taxes of \$132.1 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 first half of 2013.

Noncontrolling Interest (Alios)

We consolidated Alios from June 2011 through December 2013. A summary of net loss (income) attributable to noncontrolling interest (Alios) in the second quarter and first half of 2013 is as follows:

	Three Months Ended June 30, 2013 (in thousands)	Six Months Ended June 30, 2013
Loss before benefit from income taxes	\$6,824	\$ 12,121
Decrease in fair value of contingent milestone and royalty payments	80	2,820
Benefit from income taxes	(2,357	) (5,783
Net loss attributable to noncontrolling interest (Alios)	\$4,547	\$ 9,158

As a result of the deconsolidation of Alios in December 2013, we did not record any net loss (income) attributable to noncontrolling interest (Alios) in the second quarter and first half of 2014 and do not expect to record any net loss (income) attributable to noncontrolling interest (Alios) in future periods.

LIQUIDITY AND CAPITAL RESOURCES

As of June 30, 2014, we had cash, cash equivalents and marketable securities of \$1.22 billion, which represented a decrease of \$245.9 million from \$1.47 billion as of December 31, 2013. This decrease was due to cash expenditures we made during the first half of 2014 related to, among other things, research and development expenses and sales, general and administrative expenses and \$39.1 million for capital expenditures, partially offset by cash receipts from product sales, royalties, a one-time cash payment of \$36.7 million from our landlord pursuant to the terms of the leases for our corporate headquarters and \$117.9 million in cash we received from issuances of common stock pursuant to our employee benefit plans. 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.

Table of Contents

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 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 July 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 first half of 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 first half of 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 June 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 June 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 June 30, 2014 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II. Other Information

Item 1. Legal Proceedings

City of Bristol Pension Fund v. Vertex Pharmaceuticals Incorporated, et al.

On September 6, 2012, a purported shareholder class action, *City of Bristol Pension Fund 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 June 28, 2012, all in violation of Section 10(b) of the Securities Exchange Act of 1934, as amended, and Rule 10b-5 promulgated thereunder. By order dated December 12, 2012, the court appointed the City of Bristol lead plaintiff and appointed the City of Bristol's attorneys lead counsel. The plaintiffs filed an amended complaint on February 11, 2013. We filed a motion to dismiss the complaint on April 12, 2013. On May 28, 2013, the plaintiffs filed an opposition to our motion to dismiss the complaint. On June 27, 2013, we filed a reply in further support of our motion to dismiss the plaintiffs' complaint. The court conducted a hearing on our motion to dismiss on November 25, 2013, and the court dismissed the plaintiffs' complaint on March 31, 2014. The plaintiffs filed a motion (i) for reconsideration and (ii) to file a second amended complaint on April 28, 2014. On May 23, 2014, the Court denied the plaintiffs' motion and clarified that their Complaint had been dismissed with prejudice.

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. 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. We plan to submit a New Drug Application, or NDA, in the United States and a Marketing Authorization Application, or MAA, in Europe for this potential combination regimen in the fourth quarter of 2014. 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.

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

Table of Contents

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.

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 June 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 June 30, 2014:

Period

Edgar Filing: VERTEX PHARMACEUTICALS INC / MA - Form 10-Q

	Total Number of Shares Purchased	Average Price Paid per Share	Total Number of Shares Purchased as Part of Publicly Announced Plans or Programs	Maximum Number of Shares that May Yet be Purchased Under the Plans or Programs
April 1, 2014 to April 30, 2014	52,238	\$0.01	—	—
May 1, 2014 to May 31, 2014	137,533	\$0.01	—	—
June 1, 2014 to June 30, 2014	42,399	\$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.

Item 6. Exhibits

Exhibit Number Exhibit Description

10.1	2013 Stock and Option Plan, as amended. (1)
10.2	Credit Agreement, dated as of July 9, 2014, among Vertex Pharmaceuticals Incorporated, Macquarie US Trading LLC and the other lenders party thereto.
31.1	Certification of the Chief Executive Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of the Chief Financial Officer under Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of the Chief Executive Officer and the Chief Financial Officer under Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance
101.SCH	XBRL Taxonomy Extension Schema
101.CAL	XBRL Taxonomy Extension Calculation
101.LAB	XBRL Taxonomy Extension Labels
101.PRE	XBRL Taxonomy Extension Presentation
101.DEF	XBRL Taxonomy Extension Definition

(1) Incorporated by reference to Appendix A to Vertex's definitive proxy statement, filed on March 28, 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

July 31, 2014

By: /s/ Ian F. Smith

Ian F. Smith

Executive Vice President and Chief Financial Officer

(principal financial officer and
duly authorized officer)