

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

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): October 25, 2013

VERTEX PHARMACEUTICALS INCORPORATED

(Exact name of registrant as specified in its charter)

MASSACHUSETTS

(State or other jurisdiction of incorporation)

000-19319

(Commission File Number)

04-3039129

(IRS Employer Identification No.)

130 Waverly Street

Cambridge, Massachusetts 02139

(Address of principal executive offices) (Zip Code)

(617) 341-6100

(Registrant's telephone number, including area code)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Item 2.02. Results of Operations and Financial Condition.

On October 29, 2013, we issued a press release in which we reported our consolidated financial results for the quarter ended September 30, 2013. A copy of that press release is attached to this Current Report on Form 8-K as Exhibit 99.1 and is incorporated herein by reference.

The information set forth in Exhibit 99.1 shall not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, and shall not be incorporated by reference into any registration statement or other document filed under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

Item 2.05. Costs Associated With Exit or Disposal Activities.

On October 29, 2013, we announced a reduction of our workforce primarily related to the support of INCIVEK following the continued and rapid decline in the number of people being treated with INCIVEK as other new medicines for the treatment of hepatitis C virus infection near approval. This action resulted from our decision to focus our investment on future opportunities in cystic fibrosis and other research and development programs, including VX-135 as part of all-oral regimens for hepatitis C virus infection.

As part of this restructuring, we are eliminating approximately 370 full-time positions globally, representing approximately a 15% reduction in our workforce. Approximately 175 positions are being eliminated in Massachusetts. Following the changes, we expect to have approximately 1,800 employees worldwide, including approximately 1,300 in Massachusetts. We estimate that we will incur aggregate restructuring charges of \$35.0 million to \$45.0 million, including \$20.0 million to \$25.0 million for employee severance and benefit costs, \$6.0 million to \$8.0 million in assets associated with this restructuring that have become impaired and approximately \$9.0 million to \$12.0 million for other costs. Approximately 75% of the restructuring charges are expected to result in cash outlays.

We committed to this course of action on October 25, 2013. We recorded \$11.4 million of these restructuring charges in the third quarter of 2013. We expect the activities related to this restructuring to be completed during the first half of 2014.

Special Note Regarding Forward-looking Statements

This Current Report on Form 8-K contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, statements regarding Vertex's expectations regarding the effects of the restructuring on its operations. While Vertex believes the forward-looking statements contained in this Current Report on Form 8-K are accurate, those statements are subject to risks and uncertainties that could cause actual outcomes to vary materially from the outcomes referenced in the forward-looking statements. These risks and uncertainties include, among other things, that the actual effects of the restructuring could vary materially from Vertex's expectations and the other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this Current Report on Form 8-K as new information becomes available.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

<u>Exhibit</u>	<u>Description of Document</u>
99.1	Press Release, dated October 29, 2013

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 hereunto duly authorized.

VERTEX PHARMACEUTICALS INCORPORATED

(Registrant)

Date: October 29, 2013

/s/ Kenneth L. Horton

Kenneth L. Horton

Executive Vice President and Chief Legal Officer

Vertex Reports Third Quarter 2013 Financial Results and Provides Financial Outlook for 2014

-Third quarter 2013 total revenues of \$222 million, including net product revenues of \$101 million for KALYDECO in cystic fibrosis and \$86 million for INCIVEK in hepatitis C-

-Cash, cash equivalents and marketable securities of approximately \$1.42 billion on September 30, 2013-

-Company reduces workforce and focuses investment on future opportunities in cystic fibrosis and other research and early development programs, including all-oral regimens in hepatitis C; expected reduction of \$150 million to \$200 million in 2014 non-GAAP operating expenses compared to expected 2013 non-GAAP operating expenses of approximately \$1.1 billion-

CAMBRIDGE, Mass.-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended September 30, 2013. Vertex reported total third quarter 2013 revenues of \$222 million, including net product revenues of \$101 million from KALYDECO[™] (ivacaftor) and \$86 million from INCIVEK[®] (telaprevir). The GAAP net loss attributable to Vertex was \$(124.1) million, or \$(0.54) per share, for the third quarter of 2013, including certain charges of \$49.7 million, comprised primarily of stock-based compensation expense and restructuring charges. Non-GAAP net loss attributable to Vertex for the third quarter of 2013 was \$(74.4) million, or \$(0.32) per diluted share. The company reported \$1.42 billion in cash, cash equivalents and marketable securities as of September 30, 2013. The company also today provided updated financial guidance for 2013.

Vertex also announced that it would focus its investment on future opportunities in cystic fibrosis (CF) and other research and early development programs, including VX-135 as part of all-oral regimens for hepatitis C. The company is reducing its workforce related to the support of INCIVEK following the continued and rapid decline in the number of people being treated with INCIVEK as other new medicines for hepatitis C near approval. These changes are expected to generate a reduction in 2014 non-GAAP operating expenses of approximately \$150 million to \$200 million compared to anticipated 2013 non-GAAP operating expenses of approximately \$1.1 billion.

"Our business is at a unique point in its evolution. We have a tremendous opportunity ahead of us to further transform the treatment of cystic fibrosis, which continues to be the company's highest priority development program," commented Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Following the continued decline in the number of people starting treatment with INCIVEK, we

today took the difficult step to reduce our workforce supporting this medicine, enabling us to focus our investment on key programs in cystic fibrosis and other diseases to position the company for future growth."

Vertex continues to advance key development programs for the treatment of CF and for all-oral regimens for hepatitis C and has multiple ongoing and planned studies for these programs. The company today provided the following updates:

Cystic Fibrosis

Vertex is conducting multiple studies aimed at helping more people with CF and enhancing the clinical benefit for these patients with our approved and investigational medicines. The company recently provided a comprehensive overview of its ongoing and planned studies in CF, including multiple ongoing label-expansion studies for ivacaftor, ongoing and planned Phase 2 and Phase 3 combination studies of lumacaftor (VX-809) and ivacaftor, and VX-661 and ivacaftor, and research efforts aimed at beginning clinical development of a next-generation corrector. The company's two Phase 3 studies evaluating a combination of ivacaftor and lumacaftor in people with CF who have two copies of the F508del mutation are now fully enrolled. Data from these studies are expected in mid-2014, and Vertex plans to submit a New Drug Application (NDA) in the U.S. and a Marketing Authorization Application (MAA) in Europe in the second half of 2014 for the combination of lumacaftor and ivacaftor. These and other updates were made as part of a press release issued on October 17, 2013.

Hepatitis C

Vertex's strategy in hepatitis C is to develop new all-oral treatment regimens of 12 weeks or less in duration with a goal of providing a high viral cure rate and improved tolerability for multiple hepatitis C genotypes. Vertex is conducting the following studies of VX-135, its nucleotide analogue hepatitis C virus (HCV) polymerase inhibitor:

- ***Study of VX-135 in Combination with Daclatasvir:*** Vertex and Bristol Myers Squibb Company (BMS) are conducting a Phase 2 study of VX-135 in combination with daclatasvir, an NS5A replication complex inhibitor being developed by BMS, in New Zealand. Safety and efficacy results from the first part of the study are expected to be available in early 2014 to inform future development plans for this combination.
- ***VX-135 in Combination with Simeprevir:*** A drug-drug interaction study of VX-135 in combination with simeprevir in healthy volunteers is complete. Simeprevir (TMC435) is a once-daily investigational hepatitis C protease inhibitor being jointly developed by Janssen R&D Ireland and Medivir AB. Vertex and Janssen are currently discussing the design of additional studies of VX-135 in combination with simeprevir in patients with genotype 1 hepatitis C.

- **Studies of VX-135 in Combination with Ribavirin:** Dosing of VX-135 in combination with ribavirin is complete in two Phase 2 studies. These studies were conducted to generate safety data for VX-135 in combination with ribavirin and were not intended to evaluate the combination of VX-135 and ribavirin as a therapeutic regimen.
 - Vertex recently completed a 12-week Phase 2 study of VX-135 dosed at 100 mg and 200 mg in combination with ribavirin being conducted in Europe. Ten patients with genotype 1 hepatitis C were enrolled in each dose group and all 20 patients completed 12 weeks of treatment. Both the 100 mg and 200 mg doses were well tolerated, no serious adverse events were reported and no liver or cardiac safety issues were identified. As previously reported, 70 percent and 80 percent of patients in the 100 mg and 200 mg dosing arms, respectively, had undetectable HCV RNA within four weeks of initiating treatment. SVR12 rates were 10 percent and 50 percent for the 100 mg and 200 mg groups, respectively. These data will be presented as a poster at the 64th American Association for the Study of Liver Diseases Annual Meeting (AASLD), November 1-5, 2013 in Washington, D.C.
 - Dosing of 100 mg of VX-135 in combination with ribavirin as part of a 12-week Phase 2 study in the United States is complete. Ten patients with genotype 1 hepatitis C were enrolled in this dose group, and all 10 patients completed 12 weeks of treatment. The 100 mg dose was well tolerated, no serious adverse events were reported and no liver or cardiac safety issues were identified. All patients achieved undetectable HCV RNA during the 12-week dosing period, and 60 percent of patients had undetectable HCV RNA within four weeks of initiating treatment. The SVR4 rate was 10 percent.
 - Further evaluation of VX-135 in the U.S. is subject to resolution with the FDA regarding the partial clinical hold on VX-135. The company intends to provide further data to the FDA, including SVR data from ongoing studies of VX-135 dosed at 100 mg and 200 mg in combination with ribavirin and with daclatasvir, through the first quarter of 2014.

Autoimmune Diseases

Vertex's strategy in autoimmune diseases is to maximize the value of VX-509, an investigational oral, selective Janus kinase 3 (JAK3) inhibitor, across multiple autoimmune diseases globally. Vertex is actively pursuing collaborative opportunities to support further global development of VX-509. In a press release issued on October 18, 2013, Vertex announced 12-week results from an ongoing Phase 2b study of VX-509 dosed once or twice daily in people with active rheumatoid arthritis (RA) taking methotrexate. The study met its primary endpoints of both the proportion of people who achieved at least a 20 percent improvement in signs and symptoms of RA, as measured by the ACR improvement criteria (ACR20), and the change from baseline in

Disease Activity Score for 28 joints (DAS28). These results were accepted for presentation at the ACR annual meeting, being held October 25-30 in San Diego, CA. The presentation of the results will take place today in the "ACR Late-Breaking Abstract Oral Session" from 2:30 to 4:00 p.m. PT.

Workforce Reduction and Investment Focus on Future Opportunities in Cystic Fibrosis and Other Key Research and Development Programs

As part of a reduction in Vertex's global workforce and the resulting investment focus on future opportunities in cystic fibrosis and other high-potential research and development programs, Vertex expects to incur total restructuring charges of approximately \$35 million to \$45 million in 2013, including a restructuring charge of approximately \$11 million in the third quarter of 2013. Vertex anticipates a reduction in 2014 non-GAAP operating expenses of approximately \$150 million to \$200 million compared to anticipated 2013 non-GAAP operating expenses of approximately \$1.1 billion. The company is eliminating 370 positions, primarily related to the support of INCIVEK, representing an approximately 15 percent reduction in the company's global workforce. Approximately 175 positions are being eliminated in Massachusetts.

Third Quarter 2013 Financial Results

Total Revenues: Total revenues for the third quarter of 2013 were \$221.7 million, compared with \$336.0 million in total revenues for the third quarter of 2012. The components of total revenues for the third quarter and first nine months of 2013 and 2012 were:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Product revenues, net	(in millions)		(in millions)	
INCIVEK revenues, net	\$ 85.6	\$ 254.3	\$ 447.0	\$ 939.0
KALYDECO revenues, net	101.1	49.2	261.8	113.1
Total product revenues, net	186.7	303.5	708.8	1,052.1
Royalty revenues				
Royalty revenues from INCIVO	21.0	20.0	104.3	80.8
Other royalty revenues	6.0	5.6	15.4	17.2
Total royalty revenues	27.0	25.6	119.7	98.0
Collaborative revenues	8.0	6.9	32.3	42.9
Total revenues	\$ 221.7	\$ 336.0	\$ 860.8	\$ 1,193.0

A table of the components of total revenues on a quarterly basis since the third quarter of 2012 is provided following the Condensed Consolidated Statements of Operations Data.

- **Net Product Revenues from INCIVEK**

Vertex's third quarter 2013 net product revenues from INCIVEK were \$85.6 million, compared to \$254.3 million for the third quarter of 2012. The reduced revenues from INCIVEK were due to fewer HCV patients initiating treatment in the third quarter of 2013 compared to the third quarter of 2012 as well as a reduction in channel inventory and a reduced realized price due to changes in the payer mix. Vertex expects a continued decline in INCIVEK revenues as people with hepatitis C await new treatment options.

- **Net Product Revenues from KALYDECO**

Vertex's third quarter 2013 net product revenues from KALYDECO were \$101.1 million, compared to \$49.2 million for the third quarter of 2012. The increased revenues, compared to the third quarter of 2012, resulted primarily from the rapid uptake of KALYDECO in eligible patients in Europe following the conclusion of reimbursement discussions. Nearly all eligible patients with the G551D mutation in the United States and Europe have started treatment with KALYDECO. In 2014, further growth in KALYDECO revenues is dependent on completion of reimbursement discussions in Australia and Canada for eligible patients with the G551D mutation and on the potential approval of ivacaftor for use in people with non-G551D gating mutations, as well as in people with CF who have the R117H mutation.

- **Royalty Revenues from INCIVO[®]**

Vertex recognized \$21.0 million in INCIVO royalty revenues for the third quarter of 2013 from our collaborator Janssen, compared to \$20.0 million in INCIVO royalty revenues for the third quarter of 2012.

Cost of Product Revenues: Cost of product revenues was \$20.0 million for the third quarter of 2013, compared to cost of product revenues of \$30.7 million for the third quarter of 2012.

Research and Development (R&D) Expenses: R&D expenses were \$228.6 million for the third quarter of 2013, including \$26.9 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, compared to \$200.2 million for the third quarter of 2012, including \$21.3 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex. The increase in Vertex's R&D investment is principally due to progression and expansion of clinical development programs in cystic fibrosis and development of all-oral hepatitis C treatment regimens, including initiation of a pivotal program for a combination of lumacaftor and ivacaftor.

Sales, General and Administrative (SG&A) Expenses: SG&A expenses were \$87.8 million for the third quarter of 2013, including \$13.4 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, compared to \$97.7 million for the third quarter of 2012, including \$10.9 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex. This decrease in SG&A expenses resulted primarily from reduced HCV marketing and commercial expenses, partially offset by increased investment to support the expanded global use of KALYDECO.

GAAP Net Loss Attributable to Vertex: Vertex's third quarter 2013 GAAP net loss was \$(124.1) million, or \$(0.54) per share, including certain charges of \$49.7 million, comprised primarily of stock-based compensation expense and a restructuring charge. Vertex's GAAP net loss for the third quarter of 2012 was \$(57.5) million, or \$(0.27) per diluted share, including \$85.7 million in certain charges.

Non-GAAP Net Income (Loss) Attributable to Vertex: Vertex's third quarter 2013 non-GAAP net loss was \$(74.4) million, or \$(0.32) per diluted share. Vertex's non-GAAP net income for the third quarter of 2012 was \$28.2 million, or \$0.13 per diluted share. The decrease in the company's third quarter 2013 non-GAAP net income (loss), compared to the third quarter of 2012, is primarily attributable to a decrease in total revenues, specifically decreased INCIVEK revenues due to fewer HCV patients initiating treatment. Total non-GAAP operating expenses for the third quarter of 2013 were consistent with the third quarter of 2012.

Cash Position: As of September 30, 2013, Vertex had \$1.42 billion in cash, cash equivalents and marketable securities compared to \$1.32 billion in cash, cash equivalents and marketable securities as of December 31, 2012.

2013 Financial Guidance

This section contains forward-looking guidance about the financial outlook for Vertex Pharmaceuticals.

Vertex today updated its financial guidance for 2013 total net revenues and 2013 KALYDECO net revenues. The company now expects lower 2013 total net revenues in the range of \$1.0 billion to \$1.05 billion. The company also now expects higher total 2013 KALYDECO net revenues in the range of \$360 million to \$365 million.

The company also today updated its financial guidance for total 2013 non-GAAP operating expenses, excluding cost of revenues, stock-based compensation expense, restructuring charges, intangible asset impairment charges, certain interest expenses related to the 2015 Notes, transition costs related to the relocation of our corporate headquarters and Alios expenses related to the accounting for the collaboration

with Vertex. Vertex now expects total 2013 non-GAAP operating expenses of approximately \$1.1 billion, which is within the range provided previously for total 2013 non-GAAP operating expenses.

Vertex expects to end 2013 with a cash position of approximately \$1.3 billion. Based on the reduction in global workforce and the resulting investment focus announced today in a separate press release, the company anticipates a reduction in 2014 non-GAAP operating expenses of approximately \$150 million to \$200 million compared to anticipated 2013 non-GAAP operating expenses of approximately \$1.1 billion.

Non-GAAP Financial Measures

In this press release, Vertex's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, Vertex provides its non-GAAP net income (loss) for the periods ending September 30, 2013 and 2012 excluding stock-based compensation expense, restructuring expense, inventory reserves, intangible asset impairment charges, net of tax, certain interest expenses related to the 2015 Notes and charges related to changes in the fair value of expected future payments under Vertex's collaboration with Alios. These results are provided as a complement to results provided in accordance with GAAP because management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding its financial position. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally, and to manage the company's business and to evaluate its performance. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the attached financial statements.

Vertex Pharmaceuticals Incorporated
Third Quarter and Nine Month Results
Condensed Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2013	2012	2013	2012
Revenues:				
Product revenues, net	\$ 186,653	\$ 303,501	\$ 708,823	\$ 1,052,149
Royalty revenues	27,012	25,586	119,705	98,047
Collaborative revenues	8,035	6,919	32,290	42,852
Total revenues	<u>221,700</u>	<u>336,006</u>	<u>860,818</u>	<u>1,193,048</u>
Costs and expenses:				
Cost of product revenues (Note 1)	20,048	30,680	75,698	161,147
Royalty expenses	7,291	7,856	32,315	31,023
Research and development expenses (R&D)	228,567	200,161	669,117	593,076
Sales, general and administrative expenses (SG&A)	87,804	97,684	287,204	326,344
Restructuring expense (Note 2)	12,048	696	12,863	1,650
Intangible asset impairment charge (Note 3)	—	—	412,900	—
Total costs and expenses	<u>355,758</u>	<u>337,077</u>	<u>1,490,097</u>	<u>1,113,240</u>
Income (loss) from operations	(134,058)	(1,071)	(629,279)	79,808
Other income (expense), net (Note 4)	4,652	(4,041)	(6,578)	(11,417)
Income (loss) before provision for (benefit from) income taxes	<u>(129,406)</u>	<u>(5,112)</u>	<u>(635,857)</u>	<u>68,391</u>
Provision for (benefit from) income taxes (Note 3)	(751)	21,355	(132,863)	41,450
Net income (loss)	<u>(128,655)</u>	<u>(26,467)</u>	<u>(502,994)</u>	<u>26,941</u>
Net loss (income) attributable to noncontrolling interest (Note 5)	4,530	(31,076)	13,688	(57,825)
Net income (loss) attributable to Vertex	<u>\$ (124,125)</u>	<u>\$ (57,543)</u>	<u>\$ (489,306)</u>	<u>\$ (30,884)</u>
Net loss per share attributable to Vertex common shareholders:				
Basic	\$ (0.54)	\$ (0.27)	\$ (2.20)	\$ (0.15)
Diluted	\$ (0.54)	\$ (0.27)	\$ (2.20)	\$ (0.15)
Shares used in per share calculations:				
Basic	230,505	213,767	222,764	211,053
Diluted	230,505	213,767	222,764	211,053

Consolidated Revenues
(in millions)
(unaudited)

	Three Months Ended				
	September 30, 2013	June 30, 2013	March 31, 2013	December 31, 2012	September 30, 2012
Product revenues, net					
INCIVEK revenues, net	\$ 85.6	\$ 155.8	\$ 205.6	\$ 222.8	\$ 254.3
KALYDECO revenues, net	101.1	99.0	61.8	58.5	49.2
Total product revenues, net	186.7	254.8	267.4	281.3	303.5
Royalty revenues					
Royalty revenues from INCIVO	21.0	44.1	39.0	36.8	20.0
Other royalty revenues	6.0	5.0	4.5	6.7	5.6
Total royalty revenues	27.0	49.1	43.6	43.5	25.6
Collaborative revenues					
	8.0	6.8	17.4	9.2	6.9
Total revenues	\$ 221.7	\$ 310.8	\$ 328.4	\$ 334.0	\$ 336.0

Reconciliation of GAAP to Non-GAAP Financial Information-Third Quarter
(in thousands, except per share amounts)

Three Months Ended September 30, 2013

	GAAP	Adjustments			Non-GAAP
		Alios Transaction	Stock-based Compensation Expense	Inventory Write-off and Restructuring Expenses	
Income (loss) from operations	\$ (134,058)	\$ 9,052	\$ 31,197	\$ 17,324	\$ (76,485)
Other income (expense), net	4,652	4	—	—	4,656
Income (loss) before provision for (benefit from) income taxes	(129,406)	9,056	31,197	17,324	(71,829)
Provision for (benefit from) income taxes	(751)	3,306	—	—	2,555
Net income (loss)	(128,655)	5,750	31,197	17,324	(74,384)
Net loss (income) attributable to noncontrolling interest (Alios)	4,530	(4,530)	—	—	—
Net income (loss) attributable to Vertex	<u>\$ (124,125)</u>	<u>\$ 1,220</u>	<u>\$ 31,197</u>	<u>\$ 17,324</u>	<u>\$ (74,384)</u>
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 6)	\$ (0.54)				\$ (0.32)

Three Months Ended September 30, 2012

	GAAP	Adjustments			Non-GAAP
		Alios Transaction	Stock-based Compensation Expense	Restructuring Expenses	
Income (loss) from operations	(1,071)	4,624	27,484	696	31,733
Other income (expense), net	(4,041)	466	—	—	(3,575)
Income (loss) before provision for (benefit from) income taxes	(5,112)	5,090	27,484	696	28,158
Provision for (benefit from) income taxes	21,355	(21,394)	—	—	(39)
Net income (loss)	(26,467)	26,484	27,484	696	28,197
Net loss (income) attributable to noncontrolling interest (Alios)	(31,076)	31,076	—	—	—
Net income (loss) attributable to Vertex	<u>(57,543)</u>	<u>57,560</u>	<u>27,484</u>	<u>696</u>	<u>28,197</u>
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 6)	\$ (0.27)				\$ 0.13

Reconciliation of GAAP to Non-GAAP Financial Information-Third Quarter
(in thousands)
(unaudited)

	Three Months Ended September 30,	
	2013	2012
GAAP total costs and expenses	\$ 355,758	\$ 337,077
Adjustments:		
Cost of product revenues (Note 1) and royalty expenses	(27,339)	(38,536)
Stock-based compensation expense	(31,197)	(27,484)
Alios transaction (Note 5)	(9,052)	(4,624)
Restructuring expenses (Note 2)	(12,048)	(696)
Non-GAAP total costs and expenses	\$ 276,122	\$ 265,737
GAAP research and development expenses	\$ 228,567	\$ 200,161
Adjustments:		
Stock-based compensation expense	(19,137)	(17,396)
Alios transaction (Note 5)	(7,725)	(3,862)
Non-GAAP research and development expenses	\$ 201,705	\$ 178,903
GAAP sales, general, and administrative expenses	\$ 87,804	\$ 97,684
Adjustments:		
Stock-based compensation expense	(12,060)	(10,088)
Alios transaction (Note 5)	(1,327)	(762)
Non-GAAP sales, general, and administrative expenses	\$ 74,417	\$ 86,834

Reconciliation of GAAP to Non-GAAP Financial Information-Nine Month
(in thousands, except per share amounts)
(unaudited)

Nine Months Ended September 30, 2013

	GAAP	Adjustments			Non-GAAP	
		Alios Transaction	Stock-based Compensation Expense	Inventory Write-off, Intangible Asset and Restructuring Charges		Debt Conversion Costs
Income (loss) from operations	\$ (629,279)	\$ 21,348	\$ 103,613	\$ 436,121	\$ —	\$ (68,197)
Other income (expense), net	(6,578)	(171)	—	—	3,908	(2,841)
Income (loss) before provision for (benefit from) income taxes	(635,857)	21,177	103,613	436,121	3,908	(71,038)
Provision for (benefit from) income taxes	(132,863)	9,089	—	127,586	—	3,812
Net income (loss)	(502,994)	12,088	103,613	308,535	3,908	(74,850)
Net loss (income) attributable to noncontrolling interest (Alios)	13,688	(13,688)	—	—	—	—
Net income (loss) attributable to Vertex	<u>\$ (489,306)</u>	<u>\$ (1,600)</u>	<u>\$ 103,613</u>	<u>\$ 308,535</u>	<u>\$ 3,908</u>	<u>\$ (74,850)</u>
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 6)	\$ (2.20)					\$ (0.34)

Nine Months Ended September 30, 2012

	GAAP	Adjustments			Non-GAAP	
		Alios Transaction	Stock-based Compensation Expense	Inventory Write-off and Restructuring Charges		
Income (loss) from operations	\$ 79,808	\$ 14,356	\$ 86,280	\$ 79,650	\$ 260,094	
Other income (expense), net	(11,417)	225	—	—	(11,192)	
Income (loss) before provision for (benefit from) income taxes	68,391	14,581	86,280	79,650	248,902	
Provision for (benefit from) income taxes	41,450	(40,354)	—	1,239	2,335	
Net income (loss)	26,941	54,935	86,280	78,411	246,567	
Net loss (income) attributable to noncontrolling interest (Alios)	(57,825)	57,825	—	—	—	
Net income (loss) attributable to Vertex	<u>\$ (30,884)</u>	<u>\$ 112,760</u>	<u>\$ 86,280</u>	<u>\$ 78,411</u>	<u>\$ 246,567</u>	
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 6)	\$ (0.15)					\$ 1.15

Reconciliation of GAAP to Non-GAAP Financial Information-Nine Month
(in thousands)
(unaudited)

	<u>Nine Months Ended September 30,</u>	
	<u>2013</u>	<u>2012</u>
GAAP total costs and expenses	\$ 1,490,097	\$ 1,113,240
Adjustments:		
Cost of product revenues (Note 1) and royalty expenses	(108,013)	(192,170)
Stock-based compensation expense	(103,613)	(86,280)
Alios transaction (Note 5)	(21,348)	(14,356)
Intangible asset impairment charge (Note 3) and restructuring expenses (Note 2)	<u>(425,763)</u>	<u>(1,650)</u>
Non-GAAP total costs and expenses	\$ 831,360	\$ 818,784
 GAAP research and development expenses	 \$ 669,117	 \$ 593,076
Adjustments:		
Stock-based compensation expense	(64,110)	(54,223)
Alios transaction (Note 5)	<u>(17,339)</u>	<u>(11,480)</u>
Non-GAAP research and development expenses	\$ 587,668	\$ 527,373
 GAAP sales, general, and administrative expenses	 \$ 287,204	 \$ 326,344
Adjustments:		
Stock-based compensation expense	(39,503)	(32,057)
Alios transaction (Note 5)	<u>(4,009)</u>	<u>(2,876)</u>
Non-GAAP sales, general, and administrative expenses	\$ 243,692	\$ 291,411

Condensed Consolidated Balance Sheets Data

(in thousands)

(unaudited)

	September 30, 2013	December 31, 2012
Assets		
Cash, cash equivalents and marketable securities	\$ 1,422,650	\$ 1,321,215
Restricted cash and cash equivalents (Alios) (Note 5)	51,059	69,983
Accounts receivable, net	120,281	143,250
Inventories (Note 1)	13,543	30,464
Other current assets	41,105	24,673
Restricted cash	127	31,934
Property and equipment, net	648,924	433,609
Intangible assets (Note 3)	250,600	663,500
Goodwill	30,992	30,992
Other non-current assets	3,474	9,668
Total assets	\$ 2,582,755	\$ 2,759,288
Liabilities and Shareholders' Equity		
Other liabilities	\$ 418,798	\$ 429,372
Accrued restructuring expense (Note 2)	26,138	23,328
Deferred tax liability (Note 3)	150,203	280,367
Deferred revenues	108,361	123,808
Construction financing lease obligation	392,569	268,031
Convertible notes (due 2015) (Note 4)	—	400,000
Noncontrolling interest (Alios) (Note 5)	221,792	235,202
Shareholders' equity (Vertex)	1,264,894	999,180
Total liabilities and shareholders' equity	\$ 2,582,755	\$ 2,759,288
Common shares outstanding	233,592	217,287

Note 1: In the three and nine months ended September 30, 2013, the company recorded within cost of product revenues reserves for excess and obsolete inventories of \$5.3 million and \$10.4 million, respectively. In the nine months ended September 30, 2012, the company recorded within cost of product revenues reserves for excess and obsolete inventories of \$78.0 million.

Note 2: On October 29, 2013, the company announced a restructuring in which it recorded \$11.4 million in restructuring expenses during the three months ended September 30, 2013. The company expects to record the majority of the remaining expenses associated with this restructuring in the fourth quarter of 2013.

Note 3: As of September 30, 2013, the intangible assets and deferred tax liability reflected in the condensed consolidated balance sheet relate to the company's collaboration agreement with Alios BioPharma, Inc. (Alios).

In the first quarter of 2013, the company determined that the value of VX-222 had become impaired and that the fair value of VX-222 was zero as of March 31, 2013. This resulted in a \$412.9 million impairment charge. In connection with this impairment charge, the company recorded a credit of \$127.6 million in its provision for income taxes.

Note 4: In the second quarter of 2013, the company elected to redeem \$400.0 million in aggregate principal amount of 3.35% convertible senior subordinated notes due 2015 (“2015 Notes”). In response, the holders of the 2015 Notes converted their 2015 Notes into approximately 8.2 million shares of the company's common stock. In accordance with the terms of the 2015 Notes, the company made additional make-whole interest payments of \$6.7 million, payable in shares of the company's common stock.

Note 5: The company has consolidated the financial statements of its collaborator Alios as of September 30, 2013, December 31, 2012, and for the three and nine months ended September 30, 2013 and 2012. The company's interest and obligations with respect to Alios' assets and liabilities are limited to those accorded to the company in its collaboration agreement with Alios. Restricted cash and cash equivalents (Alios) reflects Alios' cash and cash equivalents, which Vertex does not have any interest in and which will not be used to fund the collaboration. Each reporting period Vertex estimates the fair value of the contingent milestone payments and royalties payable by Vertex to Alios. Any increase in the fair value of these contingent milestone and royalty payments results in a decrease in net income attributable to Vertex (or an increase in net loss attributable to Vertex) on a dollar-for-dollar basis.

Note 6: Shares used in non-GAAP net income (loss) per diluted share attributable to Vertex common shareholders were 230,505,000 and 217,797,000 for the three months ended September 30, 2013 and 2012, respectively, and 222,764,000 and 214,580,000 for the nine months ended September 30, 2013 and 2012, respectively.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO™ (ivacaftor)

Ivacaftor (150mg tablets) is indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a *G551D* mutation in the *CFTR* gene.

Ivacaftor is not for use in people with CF due to other mutations in the *CFTR* gene. It is not effective in patients with CF with 2 copies of the *F508del* mutation (*F508del/F508del*) in the *CFTR* gene. The efficacy and safety of ivacaftor in children younger than 6 years of age have not been evaluated.

Elevated liver enzymes (transaminases; ALT and AST) have been reported in patients receiving ivacaftor. It is recommended that ALT and AST be assessed prior to initiating ivacaftor, every 3 months during the first year of treatment, and annually thereafter. Patients who develop increased transaminase levels should be closely monitored until the abnormalities resolve. Dosing should be interrupted in patients with ALT or AST of greater than 5 times the upper limit of normal. Following resolution of transaminase elevations, consider the benefits and risks of resuming ivacaftor dosing.

Use of ivacaftor with medicines that are strong CYP3A inducers, such as the antibiotics rifampin and rifabutin; seizure medications (phenobarbital, carbamazepine, or phenytoin); and the herbal supplement St. John's Wort, substantially decreases exposure of ivacaftor which may diminish effectiveness. Therefore, co-administration is not recommended.

The dose of ivacaftor must be adjusted when used concomitantly with potent and moderate CYP3A inhibitors. The dose of ivacaftor must be adjusted when used in patients with moderate or severe hepatic disease.

Ivacaftor can cause serious adverse reactions including abdominal pain and high liver enzymes in the blood. The most common side effects associated with ivacaftor include headache; upper respiratory tract infection (the common cold), including sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; and dizziness. These are not all the possible side effects of ivacaftor. A list of the adverse

reactions can be found in the product labeling for each country where ivacaftor is approved. Patients should tell their healthcare providers about any side effect that bothers them or does not go away.

Please see full U.S. Prescribing Information for KALYDECO at www.KALYDECO.com, the EU Summary of Product Characteristics for KALYDECO at <http://goo.gl/N3Tz4>, the Canadian Product Monograph for KALYDECO at www.vrtx.ca and the Australian Consumer Medical Information and Product Information for KALYDECO at <http://bit.ly/18wlMld>.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR INCIVEK (telaprevir)

INCIVEK® (telaprevir) is a prescription medicine used with the medicines peginterferon alfa and ribavirin to treat chronic (lasting a long time) hepatitis C genotype 1 infection in adults with stable liver problems, who have not been treated before or who have failed previous treatment. It is not known if INCIVEK is safe and effective in children under 18 years of age.

Important Safety Information

INCIVEK® (telaprevir) should always be used in combination with peginterferon alfa and ribavirin. INCIVEK combination treatment may cause serious side effects including skin rash and serious skin reactions, anemia (low red blood cell count) that can be severe, and birth defects or death of an unborn baby.

Skin rashes are common with INCIVEK combination treatment. Sometimes these skin rashes and other skin reactions can become serious, require treatment in a hospital, and may lead to death. Patients should call their healthcare provider right away if they develop any skin changes or itching during treatment with INCIVEK. Their healthcare provider will decide if they need treatment or if they need to stop INCIVEK or any of their other medicines. Patients should not stop taking INCIVEK combination treatment without talking with their healthcare provider first.

Patients' healthcare providers will do blood tests regularly to check for anemia. If anemia is severe, the healthcare providers may tell them to stop taking INCIVEK.

INCIVEK combined with peginterferon alfa and ribavirin may cause birth defects or death of an unborn baby. Therefore, a patient should not take INCIVEK combination treatment if she is pregnant or may become pregnant, or if he is a man with a sexual partner who is pregnant. Females who can become pregnant and females whose male partner takes these medicines must have a negative pregnancy test before starting treatment, every month during treatment, and for 6 months after treatment ends. Patients must use two forms of effective birth control during treatment and for 6 months after all treatment has ended. These two forms of birth control should not contain hormones, as these may not work during treatment with INCIVEK.

INCIVEK and other medicines can affect each other and can also cause side effects that can be serious or life-threatening. There are certain medicines patients cannot take with INCIVEK combination treatment. Patients should tell their healthcare providers about all the medicines they take, including prescription and **over-the-counter** medicines, vitamins and herbal supplements.

The most common side effects of INCIVEK combination treatment include itching, nausea, diarrhea, vomiting, anal or rectal problems (including hemorrhoids, discomfort, burning or itching around or near the anus), taste changes and tiredness. There are other possible side effects of INCIVEK, and side effects associated with peginterferon alfa and ribavirin also apply to INCIVEK combination treatment. Patients should tell their healthcare provider about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information including Boxed Warning, and the Medication Guide for INCIVEK available at www.INCIVEK.com.

About Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. Vertex scientists and our collaborators are working on new medicines to cure or significantly advance the treatment of cystic fibrosis, hepatitis C, rheumatoid arthritis and other life-threatening diseases. In addition to our clinical development programs, Vertex has more than a dozen ongoing preclinical programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For four years in a row, Science magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit www.vrtx.com.

Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Leiden's statements in the third paragraph of the press release, the information provided in the sections captioned "Workforce Reduction and Investment Focus on Future Opportunities in Cystic Fibrosis and Other Key Research and Development Programs" and "2013 Financial Guidance" and statements regarding (i) expected non-GAAP operating expense in 2013 and 2014; (ii) the company focusing its investment on future opportunities in cystic fibrosis and other research and development programs, including VX-135; (iii) the timing of receipt of data from studies, including the Phase 3 studies of lumacaftor and ivacaftor and studies of VX-135 in combination with daclatasvir; (iv) the research efforts aimed at beginning clinical development of a next generation corrector; (v) the timing of potential regulatory submissions to the FDA and in Europe; (vi) the company's intent to provide further data to the FDA regarding VX-135; (vii) Vertex pursuing collaborative opportunities to support further global development of VX-509; (viii) expectations regarding the restructuring charges and (ix) expectations regarding future KALYDECO and INCIVEK revenues. While Vertex believes the forward-looking statements contained in this press release are accurate, there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2013 revenues and financial results, and its 2013 and 2014 non-GAAP operating expenses may be incorrect (including because one or more of the company's assumptions underlying its revenue or expense expectations may not be realized), that the outcomes of Vertex's ongoing and planned clinical studies may be delayed or may not support registration or further development of its compounds due to safety, efficacy, or other reasons, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

Conference Call and Webcast

The company will host a conference call and webcast at 8:30 a.m. ET. To access this call, dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The conference call will be webcast live, and a link to the webcast may be accessed from the "Vertex Events" page of Vertex's website at www.vrtx.com.

A replay of the conference call and webcast will be archived on the company's website until November 5, 2013. To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast.

(VRTX-GEN)

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