



January 29, 2014

Vertex Reports Full-Year and Fourth Quarter 2013 Financial Results and Provides Financial Guidance for 2014

-Full-year 2013 total revenues of \$1.21 billion, including net product revenues of \$371.3 million for KALYDECO in cystic fibrosis and \$466.3 million for INCIVEK in hepatitis C-

-Cash, cash equivalents and marketable securities of approximately \$1.47 billion on December 31, 2013-

-Company expects total 2014 revenues of \$570 to \$600 million, including KALYDECO net revenues of \$470 to \$500 million, and non-GAAP operating expenses of \$900 to \$950 million-

BOSTON--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the full-year and fourth quarter ended December 31, 2013. The company also today provided detailed financial guidance for 2014.

Vertex reported total 2013 revenues of \$1.21 billion, including net product revenues of \$371.3 million from KALYDECO™ (ivacaftor) and \$466.3 million from INCIVEK® (telaprevir). The 2013 GAAP net loss attributable to Vertex was \$(445.0) million, or \$(1.98) per share. The 2013 non-GAAP net loss attributable to Vertex was \$(203.3) million, or \$(0.90) per diluted share. The non-GAAP net loss excludes 2013 gains of \$69.8 million related to the Alios collaboration, primarily attributable to the net effect of the full write-down of VX-135 and related deconsolidation of Alios (see Note 6), and \$174.4 million primarily related to the amendment of the company's collaboration with Janssen Pharmaceutica NV, and certain charges of \$486.0 million. The company reported approximately \$1.47 billion in cash, cash equivalents and marketable securities as of December 31, 2013.

Vertex reported total fourth quarter 2013 revenues of \$351.2 million, including net product revenues of \$109.5 million from KALYDECO, \$19.3 million from INCIVEK and the revenues related to the amendment of the company's collaboration with Janssen. The GAAP net income attributable to Vertex was \$44.3 million, or \$0.19 per share, for the fourth quarter of 2013. Non-GAAP net loss attributable to Vertex for the fourth quarter of 2013 was \$(128.4) million, or \$(0.56) per diluted share. The non-GAAP net loss excludes fourth quarter 2013 gains of \$68.2 million attributable to the net effect of the write-down and the related Alios deconsolidation and \$174.4 million primarily related to the Janssen amendment, and certain charges of \$69.9 million, comprised primarily of restructuring charges and stock-based compensation expense.

"Data generated throughout the coming year will provide important information to help define our future as we seek to create a sustainable global company with multiple transformative medicines," commented Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "Importantly, we enter 2014 in a strong financial position to enable continued investment in our key research and development programs for cystic fibrosis and other serious diseases. Over the coming months, we expect multiple important development and regulatory milestones that will help to define the potential of our approved and investigational CF medicines to treat more people with this disease."

On January 12, 2014, Vertex reviewed its corporate strategy for 2014 and provided a comprehensive update on key research and development programs. The company today provided the following updates:

Cystic Fibrosis

- **VX-661 in Combination with Ivacaftor Granted Breakthrough Therapy Designation:** Vertex today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for the combination of VX-661 and ivacaftor to treat people with cystic fibrosis (CF) who have two copies of the F508del mutation. According to the FDA, Breakthrough Therapy designation is based on preliminary clinical evidence and designed to expedite the development and review of medicines that are intended to treat a serious condition and may demonstrate substantial improvement over available therapy on one or more clinically significant endpoints. Vertex has submitted a protocol to the FDA for a 12-week Phase 2 study to evaluate the safety, efficacy and pharmacokinetics of VX-661 in combination with ivacaftor. The company is currently finalizing the design of this study and expects to begin the study in the first half of 2014. The study will enroll people with two copies of the F508del mutation.

Rheumatoid Arthritis

- **24-Week Results of Phase 2b Study of JAK3 Inhibitor VX-509:** Vertex today announced top-line 24-week results from a Phase 2b study of the Janus kinase 3 (JAK3) inhibitor VX-509 in people with moderate to severe rheumatoid arthritis (RA) who continued to receive stable doses of methotrexate during the study. As previously announced, the study met its 12-week 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). The top-line data announced today showed continued improvements in the signs and symptoms of RA from week 12 to 24 as measured by ACR20, ACR50 and ACR70. At week 24, all doses of VX-509 showed statistically significant ACR20, ACR50 and ACR70 responses versus placebo. Across the four VX-509 dose groups, the ACR20, ACR50 and ACR70 responses were between 61 and 63 percent, 38 and 47 percent and 15 and 25 percent, respectively, at 24 weeks of treatment. The ACR20, ACR50 and ACR70 responses for patients who received placebo were 17, 7 and 3 percent, respectively, at 24 weeks. Additional details on the study design and 12-week results were provided in a press release issued on October 18, 2013.

Safety results through 24 weeks of treatment were similar to results observed through 12 weeks. Through 24 weeks, the discontinuation rate due to adverse events was 9.1 percent for the pooled VX-509 treatment group and 8.5 percent for the placebo group. Overall, adverse event rates through 24 weeks were 59.9 percent in the pooled VX-509 treatment groups compared to 42.3 percent for those who received placebo. There were two deaths in the study, including one death in the VX-509 200 mg QD group in a patient with pneumonia and pancytopenia and a previously reported death in the VX-509 100 mg BID group in a patient who had cardiac failure.

In addition to the Phase 2b study in RA, Vertex has completed certain drug-drug interaction studies of VX-509 in healthy volunteers. These studies demonstrate that dosing with VX-509 inhibits CYP3A4 and indicate that dose modification with frequently prescribed medicines (e.g. atorvastatin, methylprednisone) or limited concomitant use of certain medications (e.g. oral midazolam) with VX-509 may be required.

Vertex is pursuing collaborative opportunities to support further global development of VX-509.

Full-Year 2013 Financial Results

Total Revenues: Total revenues for 2013 were \$1.21 billion, compared with \$1.53 billion in total revenues for 2012. The components of total revenues for 2013 and 2012 were:

	Twelve Months Ended December 31,	
	2013	2012
	(in millions)	
Product revenues, net		
INCIVEK revenues, net	\$ 466.3	\$ 1,161.8
KALYDECO revenues, net	371.3	171.6
Total product revenues, net	\$ 837.6	\$ 1,333.5
Royalty revenues		
Royalty revenues from INCIVO	130.7	117.6
Other royalty revenues	25.9	23.9
Total royalty revenues	\$ 156.6	\$ 141.5
Collaborative revenues	\$ 217.7	\$ 52.1
Total revenues	<u>\$ 1,212.0</u>	<u>\$ 1,527.0</u>

A table of the components of total revenues on a quarterly basis for the last five quarters is provided following the Condensed Consolidated Statements of Operations Data.

- **Net Product Revenues from INCIVEK**
Vertex's 2013 net product revenues from INCIVEK were \$466.3 million, compared to \$1.16 billion for 2012. The reduced revenues from INCIVEK were primarily due to fewer HCV patients receiving treatment with INCIVEK in 2013 compared to 2012. Vertex expects a continued decline in INCIVEK revenues as a result of increased competition.
- **Net Product Revenues from KALYDECO**
Vertex's 2013 net product revenues from KALYDECO were \$371.3 million, compared to \$171.6 million for 2012. The increased revenues, compared to 2012, resulted primarily from the rapid uptake of KALYDECO by eligible patients in Europe in 2013. 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

expansion of the KALYDECO label.

- **Royalty Revenues from INCIVO®**

Vertex recognized \$130.7 million in INCIVO royalty revenues in 2013 from the company's collaborator Janssen, compared to \$117.6 million in INCIVO royalty revenues in 2012. In November 2013, Vertex sold its rights to future royalties on INCIVO sales by Janssen for a \$152.0 million cash payment. Beginning in the first quarter of 2014, Vertex will no longer receive INCIVO royalties.

Cost of Product Revenues: Cost of product revenues was \$89.0 million in 2013, compared to cost of product revenues of \$236.7 million in 2012. The decrease in cost of product revenues was attributable to lower total product revenues and certain 2012 charges to account for excess INCIVEK inventory.

Research and Development (R&D) Expenses: R&D expenses were \$918.8 million in 2013, including certain charges of \$121.5 million, compared to \$806.2 million in 2012, including certain charges of \$87.5 million. The increase in Vertex's R&D investment is principally due to progression and expansion of clinical development programs in cystic fibrosis, including initiation of a pivotal Phase 3 program for the combination of lumacaftor and ivacaftor.

Sales, General and Administrative (SG&A) Expenses: SG&A expenses were \$362.3 million in 2013, including certain charges of \$53.2 million, compared to \$436.8 million in 2012, including certain charges of \$46.4 million. 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 2013 GAAP net loss was \$(445.0) million or \$(1.98) per share. Vertex's GAAP net loss for 2012 was \$(107.0) million, or \$(0.50) per diluted share.

Non-GAAP Net Income (Loss) Attributable to Vertex: Vertex's 2013 non-GAAP net loss was \$(203.3) million, or \$(0.90) per diluted share. The 2013 non-GAAP loss excludes 2013 gains of \$69.8 million, primarily attributable to the net effect of the full write-down of VX-135 and related deconsolidation of Alios, and \$174.4 million, primarily related to the amendment of the company's collaboration with Janssen, and certain charges of \$486.0 million, primarily related to the impairment of an intangible hepatitis C asset (VX-222), restructuring charges and stock-based compensation expense. Vertex's non-GAAP net income for 2012 was \$255.5 million, or \$1.18 per diluted share, excluding \$362.6 million in certain charges. The difference in the company's 2013 non-GAAP net loss, compared to 2012 non-GAAP net income, is primarily attributable to decreased INCIVEK revenues.

Cash Position: As of December 31, 2013, Vertex had \$1.47 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.

Fourth Quarter 2013 Financial Results

Total Revenues: Total revenues for the fourth quarter of 2013 were \$351.2 million, compared with \$334.0 million in total revenues for the fourth quarter of 2012. The components of total revenues for the fourth quarter of 2013 and 2012 were:

	Three Months Ended December 31,	
	2013	2012
	(in millions)	
Product revenues, net		
INCIVEK revenues, net	\$ 19.3	\$ 222.8
KALYDECO revenues, net	109.5	58.5
Total product revenues, net	\$ 128.8	\$ 281.3
Royalty revenues		
Royalty revenues from INCIVO	26.4	36.8
Other royalty revenues	10.5	6.7
Total royalty revenues	\$ 36.9	\$ 43.5
Collaborative revenues	\$ 185.4	\$ 9.2
Total revenues	\$ 351.2	\$ 334.0

- **Net Product Revenues from INCIVEK**

Vertex's fourth quarter 2013 net product revenues from INCIVEK were \$19.3 million, compared to \$222.8 million for the fourth quarter of 2012. The reduced revenues from INCIVEK were due to fewer HCV patients receiving treatment with INCIVEK in the fourth quarter of 2013 compared to the fourth quarter of 2012 and a reduced realized price. Vertex expects a continued decline in INCIVEK revenues as a result of increased competition.

- **Net Product Revenues from KALYDECO**

Vertex's fourth quarter 2013 net product revenues from KALYDECO were \$109.5 million, compared to \$58.5 million for the fourth quarter of 2012. The increased revenues, compared to the fourth 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 expansion of the KALYDECO label.

- **Royalty Revenues from INCIVO®**

Vertex recognized \$26.4 million in INCIVO royalty revenues for the fourth quarter of 2013 from the company's collaborator Janssen, compared to \$36.8 million in INCIVO royalty revenues for the fourth quarter of 2012. In November 2013, Vertex sold its rights to future royalties on INCIVO sales by Janssen for a \$152.0 million cash payment. Beginning in the first quarter of 2014, Vertex will no longer receive INCIVO royalties.

Cost of Product Revenues: Cost of product revenues was \$13.3 million for the fourth quarter of 2013, compared to cost of product revenues of \$75.6 million for the fourth quarter of 2012. The decrease in cost of product revenues was attributable to lower total product revenues and certain 2012 charges to account for excess INCIVEK inventory.

Research and Development (R&D) Expenses: R&D expenses were \$249.6 million for the fourth quarter of 2013, including certain charges of \$40.1 million, compared to \$213.1 million for the fourth quarter of 2012, including certain charges of \$21.8 million. The increase in Vertex's R&D investment is principally due to progression and expansion of clinical development programs in cystic fibrosis, including initiation of a pivotal Phase 3 program for the combination of lumacaftor and ivacaftor.

Sales, General and Administrative (SG&A) Expenses: SG&A expenses were \$75.2 million for the fourth quarter of 2013, including certain charges of \$9.7 million, compared to \$110.5 million for the fourth quarter of 2012, including certain charges of \$11.4 million. 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 Income (Loss) Attributable to Vertex: Vertex's fourth quarter 2013 GAAP net income was \$44.3 million, or \$0.19 per share. Vertex's GAAP net loss for the fourth quarter of 2012 was \$(76.1) million, or \$(0.35) per diluted share.

Non-GAAP Net Income (Loss) Attributable to Vertex: Vertex's fourth quarter 2013 non-GAAP net loss was \$(128.4) million, or \$(0.56) per diluted share, excluding fourth quarter 2013 gains of \$68.2 million attributable to the net effect of the full write-down of VX-135 and the related deconsolidation of Alios, and \$174.4 million primarily related to the amendment of the company's collaboration with Janssen, and certain charges of \$69.9 million, comprised primarily of restructuring charges and stock-based compensation expense. Vertex's non-GAAP net income for the fourth quarter of 2012 was \$9.0 million, or \$0.04 per diluted share, excluding \$85.1 million in certain charges. The difference in the company's fourth quarter 2013 non-GAAP net income (loss), compared to the fourth quarter of 2012, is primarily attributable to a decrease in total revenues, specifically decreased INCIVEK revenues due to fewer HCV patients receiving treatment.

2014 Financial Guidance

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

- **Total Revenues:** Vertex expects total 2014 net revenues of \$570 to \$600 million. Total revenues include net product revenues for KALYDECO and INCIVEK, as well as royalty and collaborative revenues.
 - **KALYDECO Net Revenues:** Vertex expects KALYDECO net revenues of \$470 to \$500 million for 2014.
- **Non-GAAP Operating Expenses:** Vertex expects that its 2014 non-GAAP operating expenses will be in the range of \$900 to \$950 million. The company's planned 2014 investment includes approximately \$40 to \$50 million of expense related to development of an all-oral treatment regimen for hepatitis C.
 - **Research and Development Expenses:** Vertex expects that full-year 2014 non-GAAP R&D expenses will be in the range of \$665 to \$695 million. The research component of 2014 non-GAAP R&D expenses is expected to remain consistent with 2013 at approximately \$200 million.
 - **Sales, General and Administrative Expenses:** Vertex expects that full-year 2014 non-GAAP SG&A expenses will be in the range of \$235 to \$255 million.

Vertex's expected non-GAAP operating expense excludes cost of revenues, stock-based compensation expense, restructuring charges, transition costs related to the relocation of our corporate headquarters, post-restructuring HCV collaborative revenues and development costs, and any similar expenses incurred in 2014.

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 full-year and fourth quarter non-GAAP net income (loss) for the periods ending December 31, 2013 and 2012 excluding stock-based compensation expense, restructuring expenses, inventory reserves, intangible asset impairment charges, net of tax, certain interest expenses related to the 2015 Notes, transition costs related to the relocation of our corporate headquarters, post-restructuring HCV collaborative revenues and development costs, and gains and charges related to changes in fair value of the company's HCV nucleotide analogue program and ensuing deconsolidation of 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
Fourth Quarter and Twelve Month Results
Condensed Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended		Twelve Months Ended	
	December 31,		December 31,	
	2013	2012	2013	2012
Revenues:				
Product revenues, net	\$ 128,822	\$ 281,309	\$ 837,645	\$ 1,333,458
Royalty revenues	36,887	43,451	156,592	141,498
Collaborative revenues (Note 1)	185,448	9,234	217,738	52,086
Total revenues	<u>351,157</u>	<u>333,994</u>	<u>1,211,975</u>	<u>1,527,042</u>
Costs and expenses:				
Cost of product revenues (Note 2)	13,281	75,595	88,979	236,742
Royalty expenses	8,983	12,120	41,298	43,143
Research and development expenses (R&D)	249,609	213,109	918,783	806,185
Sales, general and administrative expenses (SG&A)	75,188	110,452	362,342	436,796
Restructuring expense (Note 3)	27,658	194	40,521	1,844
Intangible asset impairment charges (Note 4) (Note 6)	250,600	—	663,500	—
Total costs and expenses	<u>625,319</u>	<u>411,470</u>	<u>2,115,423</u>	<u>1,524,710</u>
Income (loss) from operations	(274,162)	(77,476)	(903,448)	2,332
Other expense, net (Note 5)(Note 6)	(66,091)	(3,296)	(72,669)	(14,713)
Income (loss) before provision for (benefit from) income taxes	<u>(340,253)</u>	<u>(80,772)</u>	<u>(976,117)</u>	<u>(12,381)</u>
Provision for (benefit from) income taxes (Note 4)	(155,704)	(2,696)	(288,567)	38,754
Net loss	<u>(184,549)</u>	<u>(78,076)</u>	<u>(687,550)</u>	<u>(51,135)</u>
Net loss (income) attributable to noncontrolling interest	228,834	1,928	242,522	(55,897)
Net income (loss) attributable to Vertex	<u>\$ 44,285</u>	<u>\$ (76,148)</u>	<u>\$ (445,028)</u>	<u>\$ (107,032)</u>
Net income (loss) per share attributable to Vertex common shareholders:				
Basic	\$ 0.19	\$ (0.35)	\$ (1.98)	\$ (0.50)
Diluted	\$ 0.19	\$ (0.35)	\$ (1.98)	\$ (0.50)
Shares used in per share calculations:				
Basic	231,264	214,607	224,906	211,946
Diluted	235,717	214,607	224,906	211,946

Consolidated Revenues
(in millions)
(unaudited)

Three Months Ended

	December 31, 2013	September 30, 2013	June 30, 2013	March 31, 2013	December 31, 2012
Product revenues, net					
INCIVEK revenues, net	\$ 19.3	\$ 85.6	\$ 155.8	\$ 205.6	\$ 222.8
KALYDECO revenues, net	109.5	101.1	99.0	61.8	58.5
Total product revenues, net	128.8	186.7	254.8	267.4	281.3
Royalty revenues					
Royalty revenues from INCIVO	26.4	21.0	44.1	39.0	36.8
Other royalty revenues	10.5	6.0	5.0	4.5	6.7
Total royalty revenues	36.9	27.0	49.1	43.6	43.5
Collaborative revenues	185.4	8.0	6.8	17.4	9.2
Total revenues	\$ 351.2	\$ 221.7	\$ 310.8	\$ 328.4	\$ 334.0

Reconciliation of GAAP to Non-GAAP Financial Information-Fourth Quarter

(in thousands, except per share amounts)

(unaudited)

Three Months Ended December 31, 2013

	GAAP	Adjustments			Non-GAAP
		Alios Transaction (Note 6)	Stock-based Compensation Expense	Other Adjustments (Note 8)	
Income (loss) from operations	\$(274,162)	\$ 262,475	\$ 23,240	\$ (140,042)	\$(128,489)
Other income (expense), net	(66,091)	55,204	—	12,283	1,396
Income (loss) before provision for (benefit from) income taxes	(340,253)	317,679	23,240	(127,759)	(127,093)
Provision for (benefit from) income taxes	(155,704)	157,056	—	—	1,352
Net income (loss)	(184,549)	160,623	23,240	(127,759)	(128,445)
Net loss (income) attributable to noncontrolling interest (Alios)	228,834	(228,834)	—	—	—
Net income (loss) attributable to Vertex	<u>\$ 44,285</u>	<u>\$ (68,211)</u>	<u>\$ 23,240</u>	<u>\$ (127,759)</u>	<u>\$(128,445)</u>
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 7)	\$ 0.19				\$ (0.56)

Three Months Ended December 31, 2012

	GAAP	Adjustments			Non-GAAP
		Alios Transaction (Note 6)	Stock-based Compensation Expense	Other Adjustments (Note 8)	
Income (loss) from operations	\$(77,476)	\$ 5,706	\$ 27,524	\$ 55,383	\$ 11,137
Other income (expense), net	(3,296)	(243)	—	—	(3,539)
Income (loss) before provision for (benefit from) income taxes	(80,772)	5,463	27,524	55,383	7,598
Provision for (benefit from) income taxes	(2,696)	1,325	—	—	(1,371)
Net income (loss)	(78,076)	4,138	27,524	55,383	8,969
Net loss (income) attributable to noncontrolling interest (Alios)	1,928	(1,928)	—	—	—
Net income (loss) attributable to Vertex	<u>\$(76,148)</u>	<u>\$ 2,210</u>	<u>\$ 27,524</u>	<u>\$ 55,383</u>	<u>\$ 8,969</u>
Net loss per diluted share attributable to Vertex common shareholders (Note 7)	\$ (0.35)				\$ 0.04

Reconciliation of GAAP to Non-GAAP Financial Information-Fourth Quarter

(in thousands)
(unaudited)

	Three Months Ended December 31,	
	2013	2012
GAAP total costs and expenses	\$ 625,319	\$ 411,470
Adjustments:		
Cost of product revenues (Note 2) and royalty expenses	(22,264)	(87,715)
Stock-based compensation expense	(23,240)	(27,524)
Alios transaction (Note 6)	(262,475)	(5,706)
Other Adjustments (Note 8)	(42,354)	(194)
Non-GAAP total costs and expenses	\$ 274,986	\$ 290,331
GAAP research and development expenses	\$ 249,609	\$ 213,109
Adjustments:		
Stock-based compensation expense	(17,075)	(17,019)
Alios transaction (Note 6)	(9,749)	(4,784)
Other Adjustments (Note 8)	(13,254)	—
Non-GAAP research and development expenses	\$ 209,531	\$ 191,306
GAAP sales, general and administrative expenses	\$ 75,188	\$ 110,452
Adjustments:		
Stock-based compensation expense	(6,165)	(10,505)
Alios transaction (Note 6)	(2,126)	(922)
Other Adjustments (Note 8)	(1,442)	—
Non-GAAP sales, general and administrative expenses	\$ 65,455	\$ 99,025

Reconciliation of GAAP to Non-GAAP Financial Information-Twelve Months

(in thousands, except per share amounts)
(unaudited)

Twelve Months Ended December 31, 2013

	GAAP	Adjustments				Non-GAAP
		Alios Transaction (Note 6)	Stock-based Compensation Expense	Debt Conversion Costs	Other Adjustments (Note 8)	
Income (loss) from operations	\$(903,448)	\$ 283,823	\$ 126,853	\$ —	\$ 296,079	\$(196,693)
Other income (expense), net	(72,669)	55,033	—	3,908	12,283	(1,445)
Income (loss) before provision for (benefit from) income taxes	(976,117)	338,856	126,853	3,908	308,362	(198,138)
Provision for (benefit from) income taxes	(288,567)	166,145	—	—	127,586	5,164
Net income (loss)	(687,550)	172,711	126,853	3,908	180,776	(203,302)
Net loss (income) attributable to noncontrolling interest (Alios)	242,522	(242,522)	—	—	—	—
Net income (loss) attributable to Vertex	\$(445,028)	\$ (69,811)	\$ 126,853	\$ 3,908	\$ 180,776	\$(203,302)
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 7)	\$ (1.98)					\$ (0.90)

Twelve Months Ended December 31, 2012

Adjustments

Other

	GAAP	Alios Transaction (Note 6)	Stock-based Compensation Expense	Adjustments (Note 8)	Non- GAAP
Income (loss) from operations	\$ 2,332	\$ 20,062	\$ 113,804	\$ 135,033	\$271,231
Other income (expense), net	(14,713)	(18)	—	—	(14,731)
Income (loss) before provision for (benefit from) income taxes	(12,381)	20,044	113,804	135,033	256,500
Provision for (benefit from) income taxes	38,754	(39,029)	—	1,239	964
Net income (loss)	(51,135)	59,073	113,804	133,794	255,536
Net loss (income) attributable to noncontrolling interest (Alios)	(55,897)	55,897	—	—	—
Net income (loss) attributable to Vertex	<u>\$(107,032)</u>	<u>\$ 114,970</u>	<u>\$ 113,804</u>	<u>\$ 133,794</u>	<u>\$255,536</u>
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 7)	\$ (0.50)				\$ 1.18

Reconciliation of GAAP to Non-GAAP Financial Information-Twelve Months

(in thousands)
(unaudited)

	Twelve Months Ended December 31,	
	2013	2012
GAAP total costs and expenses	\$ 2,115,423	\$ 1,524,710
Adjustments:		
Cost of product revenues (Note 2) and royalty expenses	(130,277)	(279,885)
Stock-based compensation expense	(126,853)	(113,804)
Alios transaction (Note 6)	(283,823)	(20,062)
Other Adjustments (Note 8)	(468,117)	(1,844)
Non-GAAP total costs and expenses	\$ 1,106,353	\$ 1,109,115
GAAP research and development expenses	\$ 918,783	\$ 806,185
Adjustments:		
Stock-based compensation expense	(81,204)	(71,242)
Alios transaction (Note 6)	(27,088)	(16,264)
Other Adjustments (Note 8)	(13,254)	—
Non-GAAP research and development expenses	\$ 797,237	\$ 718,679
GAAP sales, general and administrative expenses	\$ 362,342	\$ 436,796
Adjustments:		
Stock-based compensation expense	(45,649)	(42,562)
Alios transaction (Note 6)	(6,135)	(3,798)
Other Adjustments (Note 8)	(1,442)	—
Non-GAAP sales, general and administrative expenses	\$ 309,116	\$ 390,436

Condensed Consolidated Balance Sheets Data

(in thousands)
(unaudited)

	December 31, 2013	December 31, 2012
Assets		
Cash, cash equivalents and marketable securities	\$ 1,465,076	\$ 1,321,215
Restricted cash and cash equivalents (Alios) (Note 6)	—	69,983
Accounts receivable, net	85,517	143,250
Inventories (Note 2)	14,147	30,464

Other current assets	23,836	24,673
Restricted cash	130	31,934
Property and equipment, net	696,911	433,609
Intangible assets (Note 4)	—	663,500
Goodwill	30,992	30,992
Other non-current assets	2,432	9,668
Total assets	\$ 2,319,041	\$ 2,759,288

Liabilities and Shareholders' Equity

Other liabilities	\$ 422,377	\$ 429,372
Accrued restructuring expense	28,353	23,328
Deferred tax liability (Note 4)	—	280,367
Deferred revenues	70,969	123,808
Construction financing lease obligation	440,937	268,031
Convertible notes (due 2015) (Note 5)	—	400,000
Noncontrolling interest (Alios) (Note 6)	—	235,202
Shareholders' equity (Vertex)	1,356,405	999,180
Total liabilities and shareholders' equity	\$ 2,319,041	\$ 2,759,288

Common shares outstanding	233,789	217,287
---------------------------	---------	---------

Note 1: During the fourth quarter of 2013, the company sold its product royalty rights relating to INCIVO (telaprevir) to Janssen Pharmaceutica NV. Under this amendment to the company's collaboration agreement with Janssen, Janssen made a \$152.0 million cash payment to Vertex in the fourth quarter of 2013 and will cease paying royalties to Vertex on INCIVO sales beginning in 2014. As a result of the amendment, Janssen will have sole authority to execute INCIVO marketing and promotion activities in these regions.

Note 2: In the twelve months ended December 31, 2013, the company recorded within cost of product revenues reserves for excess and obsolete inventories of \$10.4 million. In the three and twelve months ended December 31, 2012, the company recorded within cost of product revenues reserves for excess and obsolete inventories of \$55.2 million and \$133.2 million, respectively.

Note 3: The Company recorded \$27.5 million and \$39.0 million in restructuring expenses during the three and twelve months ended December 31, 2013, respectively, in connection with its 2013 restructuring.

Note 4: As of December 31, 2013, there were no intangible assets or deferred tax liability reflected in the condensed consolidated balance sheet.

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.

In the fourth quarter of 2013, the company determined that the value of the HCV nucleotide analogue program related to the company's collaboration agreement with Alios BioPharma, Inc. had become impaired and that the fair value of the intangible asset was zero as of December 31, 2013. This resulted in a \$250.6 million impairment charge. Accordingly, a benefit for income taxes of \$102.1 million was recorded in the fourth quarter of 2013.

Note 5: 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 6: The company has consolidated the financial statements of its collaborator Alios from the second quarter of 2011 through December 31, 2013. The company determined that it would no longer consolidate Alios as of December 31, 2013. During the period the company consolidated Alios, the company's interest and obligations with respect to Alios' assets and liabilities were limited to those accorded to the company in its collaboration agreement with Alios. Restricted cash and cash equivalents (Alios) as of December 31, 2012 reflected Alios' cash and cash equivalents, which Vertex did not have any interest in and which was not available to fund the collaboration.

The net effect of the impairment charge, the tax benefit, and the related deconsolidation was a gain of approximately \$68.2 million attributable to Vertex in 2013. The gain of \$68.2 million is approximately the difference between (i) losses we recorded in 2011 and 2012 based on estimated increases in the fair value of potential milestone and royalty payments payable by us to Alios and (ii) the upfront and milestone payments that we made to Alios pursuant to the Alios collaboration.

Note 7: Shares used in non-GAAP net income (loss) per diluted share attributable to Vertex common shareholders were 231,264,000 and 217,291,000 for the three months ended December 31, 2013 and 2012, respectively, and 224,906,000 and 215,263,000 for the twelve months ended December 31, 2013 and 2012, respectively.

Note 8: In the three and twelve months ended December 31, 2013, "Other Adjustments" consists of (i) gains of \$174.4 million related to post-restructuring HCV collaborative revenues and development costs, (ii) an intangible asset impairment charge, net of tax, related to VX-222, of \$0.0 and \$285.3 million, respectively, (iii) restructuring expenses of \$27.7 and \$40.5 million, respectively, (iv) \$19.0 million of transition costs related to the relocation of our corporate headquarters and (v) inventory reserves of \$0.0 and \$10.4 million, respectively.

In the three and twelve months ended December 31, 2012, "Other Adjustments" consists of (i) inventory reserves, net of tax, of \$55.2 and \$132.0 million, respectively and (ii) restructuring expenses of \$0.2 and \$1.8 million, 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 (ivacaftor) at <http://bit.ly/18wIMld>.

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 fourth paragraph of the press release, the information provided in the section captioned "2014 Financial Guidance," and the information provided regarding (i) Vertex's plans regarding the design and initiation of a Phase 2 study of VX-661 in combination with ivacaftor; (ii) the suggestion that use of VX-509 with certain other medicines may require dose modification or limited concomitant use; (iii) Vertex pursuing collaborative opportunities to support further global development of VX-509; and (iv) Vertex's expectations regarding 2014 revenues, including KALYDECO and INCIVEK revenues. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and 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 2014 revenues and financial results and its 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 data from the company's development programs 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 today at 5:00 p.m. ET. To access this call, dial (866) 501-1537 (U.S.) or +1 (720) 545-0001 (International). The call will also be available as a live and archived webcast on Vertex's website at www.vrtx.com in the "Investors" section under "Events and Presentations." To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast.

(VRTX-GEN)

Vertex Pharmaceuticals Incorporated

Investors:

Michael Partridge, 617-341-6108

or

Kelly Lewis, 617-961-7530

or

Media:

Zach Barber, 617-341-6992

mediainfo@vrtx.com

Source: Vertex Pharmaceuticals Incorporated

News Provided by Acquire Media