October 28, 2014

Vertex Reports Third Quarter 2014 Financial Results

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended September 30, 2014. Vertex reported total third quarter 2014 GAAP revenues of $179 million, including revenues of $127 million from KALYDECO® (ivacaftor). The GAAP net loss for the third quarter of 2014 was $(170) million, or $(0.72) per share, which includes net charges of $84 million. The non-GAAP net loss for the third quarter of 2014 was $(86) million, or $(0.37) per share. The company also today provided updated financial guidance for total 2014 non-GAAP revenues, 2014 KALYDECO revenues and non-GAAP operating expenses. As of September 30, 2014, Vertex had $1.48 billion in cash, cash equivalents and marketable securities.

"With the recent progress across our research and development programs in cystic fibrosis and the expansion of the number of people being treated with KALYDECO, we have established a clear path for the future of our company," said Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "We remain focused on investing in the discovery and development of new medicines for CF and other serious and rare diseases while increasing revenues to move us to profitability and drive earnings growth."

Research and Development Updates

In a press release issued on October 9, 2014, Vertex provided a comprehensive update on its research and development programs aimed at developing combinations of medicines to treat the underlying cause of cystic fibrosis (CF) for the majority of people with the disease. Vertex today provided the following updates:

KALYDECO® (ivacaftor)

Availability of KALYDECO in Australia: In Australia, KALYDECO is expected to be listed on the Pharmaceutical Benefits Scheme (PBS) as of December 1, which will provide reimbursement for all eligible patients to begin and remain on treatment with KALYDECO. There are approximately 250 people age 6 years and older who are expected to be eligible for treatment with KALYDECO in Australia.

R117H Mutation: Based on data from a Phase 3 study, Vertex submitted a supplemental New Drug Application (sNDA) in the U.S. in June and a Marketing Authorization Application (MAA) variation in the E.U. in July for the approval of KALYDECO for use in people who have the R117H mutation. On October 21, the U.S. Food and Drug Administration's Pulmonary Allergy Drugs Advisory Committee (PADAC) voted 13-2 to recommend approval of KALYDECO in people with CF ages 6 and older who have the R117H mutation in the cystic fibrosis transmembrane regulatory (*CFTR*) gene, which is the indication being reviewed by the FDA. The FDA is not bound by the committee's recommendation, but often follows its advice.

Third Quarter 2014 Non-GAAP Financial Results

The third quarter 2014 non-GAAP financial results exclude stock-based compensation expense, costs related to the relocation of our corporate headquarters, hepatitis C revenues and costs and other adjustments. The third quarter 2013 non-GAAP financial results exclude stock-based compensation expense, expenses related to Alios (HCV), hepatitis C-related costs and other adjustments.

Total Non-GAAP Revenues: Total non-GAAP revenues for the third quarter of 2014 were $165.0 million, including $126.8 million in net product revenues from KALYDECO and $38.2 million from royalties and collaborative revenues, which includes revenues from a one-time payment of $30 million from Janssen related to the out-licensing of VX-787 for the treatment of influenza. The components of total non-GAAP revenues for the third quarter of 2014 were:

Three Months Ended September 30, 2014 (in millions)		
GAAP revenues	HCV related revenues	Non-GAAP revenues

Product revenues			
KALYDECO revenues, net	$ 126.8	$ —	$ 126.8
INCIVEK revenues, net	10.3	(10.3)	—
Total product revenues, net	137.1	(10.3)	126.8
Royalty revenues	8.4	(2.3)	6.1
Collaborative revenues	33.5	(1.4)	32.1
Total revenues	$ 179.0	$ (14.0)	$ 165.0

- **Net Product Revenues from KALYDECO:** Vertex's third quarter 2014 net product revenues from KALYDECO were $126.8 million compared to $101.1 million for the third quarter of 2013. The increased revenues, compared to the third quarter of 2013, resulted primarily from the use of KALYDECO in the U.S. in people with the additional mutations approved by the FDA in February 2014 and from an increase of approximately $7 million that resulted from changes in Vertex's distribution network.

Non-GAAP Operating Expenses: Total non-GAAP operating expenses for the third quarter of 2014 were $212.4 million, compared to $274.7 million for the third quarter of 2013. This reduction was primarily the result of prioritization of investment toward medicines for CF resulting in decreased R&D and SG&A expenses as follows:

- **Research and Development (R&D) Expenses**: Non-GAAP R&D expenses were $157.4 million for the third quarter of 2014, compared to $200.3 million in non-GAAP R&D expenses for the third quarter of 2013.
- **Sales, General and Administrative (SG&A) Expenses:** Non-GAAP SG&A expenses were $55.1 million for the third quarter of 2014, compared to $74.4 million in non-GAAP SG&A expenses for the third quarter of 2013.

Non-GAAP Net Income (Loss) Attributable to Vertex: Vertex's third quarter 2014 non-GAAP net loss was $(86.2) million, or $(0.37) per diluted share, compared to a non-GAAP net loss of $(74.4) million, or $(0.32) per diluted share, for the third quarter of 2013. The increased non-GAAP net loss for the third quarter of 2014 was primarily the result of a reduction in INCIVEK net product revenues, partially offset by increased KALYDECO product revenues, decreased operating expenses and the revenues from a one-time payment of $30 million from Janssen related to the out-licensing of VX-787.

Cash Position at September 30, 2014

As of September 30, 2014, Vertex had $1.48 billion in cash, cash equivalents and marketable securities compared to $1.47 billion in cash, cash equivalents and marketable securities as of December 31, 2013. In July 2014, Vertex entered into a credit agreement that provides for a secured loan of up to $500 million, $300 million of which Vertex received in July 2014 and is outstanding as of September 30, 2014.

2014 Financial Guidance

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

On May 1, 2014, Vertex provided guidance for 2014 total non-GAAP revenues of $520 to $550 million, including total 2014 KALYDECO net product revenues of $470 to $500 million. The company now expects total non-GAAP revenues to be $525 to $535 million, with KALYDECO net product revenues expected to be approximately $460 million. The KALYDECO net product revenues range was based on assumptions of the timing of label expansion and reimbursement approvals in certain countries, and the company now expects KALYDECO revenues to be below the range provided in May due primarily to the delay in the reimbursement of KALYDECO in Australia.

Additionally, Vertex provided guidance for 2014 total non-GAAP operating expenses of $890 to $930 million. 2014 total non-GAAP operating expenses are now expected to be approximately $910 to $920 million, which reflects ongoing activities to support the accelerated advancement of VX-661 into a pivotal program in the first half of 2015.

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, non-GAAP financial results exclude: (i) in 2014, stock-based compensation expense, costs related to the relocation of the company's corporate headquarters including a one-time cash payment received related to a lease agreement, hepatitis C revenues and costs and other adjustments and (ii) in 2013, stock-based compensation expense, expenses related to Alios (HCV), the impairment of VX-222, hepatitis C inventory charges and costs, certain interest expenses related to the convertible notes due 2015 and other adjustments. 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 the company's 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 information.

Third Quarter 2014 GAAP Financial Results

Total Revenues: Total revenues for the third quarter of 2014 were $179.0 million compared with $221.7 million in total revenues for the third quarter of 2013. Third quarter 2014 revenues are comprised primarily of $126.8 million in KALYDECO net revenues and an aggregate of $52.2 million in net product revenues from INCIVEK, royalty revenues and collaborative revenues, which includes revenues from a one-time payment of $30.0 million from Janssen related to the out-licensing of VX-787 for the treatment of influenza. For the third quarter of 2013, Vertex reported $101.1 million in net product revenues from KALYDECO and $120.6 million in net product revenues from INCIVEK, royalty revenues and collaborative revenues.

Operating Costs and Expenses: Total operating costs and expenses for the third quarter of 2014 were $321.2 million, including certain charges of $108.8 million, compared to $345.3 million for the third quarter of 2013, including certain charges of $70.6 million. GAAP operating costs and expenses include:

- **R&D Expenses:** R&D expenses were $190.9 million for the third quarter of 2014, including $33.6 million of certain charges, compared to $219.4 million for the third quarter of 2013, including $19.2 million of certain charges.
- **Sales, General and Administrative (SG&A) Expenses:** SG&A expenses were $75.2 million for the third quarter of 2014, including $20.2 million of certain charges, compared to $86.4 million for the third quarter of 2013, including $12.0 million of certain charges.

Net Loss Attributable to Vertex: Vertex's third quarter 2014 net loss was $(170.1) million, or $(0.72) per share, and includes net charges of $83.8 million. Vertex's third quarter 2013 net loss was $(124.1) million, or $(0.54) per share, including net charges of $49.7 million.

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,	
	2014	2013	2014	2013
Revenues:				
Product revenues, net	$ 137,099	$ 186,653	$ 362,879	$ 708,823
Royalty revenues	8,386	27,012	32,134	119,705
Collaborative revenues	33,502	8,035	40,846	32,290
Total revenues	178,987	221,700	435,859	860,818
Costs and expenses:				
Cost of product revenues	10,208	20,048	28,435	75,698
Royalty expenses	3,976	7,291	18,525	32,315
Research and development expenses (R&D)	190,939	219,442	654,043	643,636
Sales, general and administrative expenses (SG&A)	75,224	86,427	226,882	283,133
Restructuring expenses	40,843	12,048	46,761	12,863
Intangible asset impairment charge (Note 1)	—	—	—	412,900
Total costs and expenses	321,190	345,256	974,646	1,460,545
Loss from operations	(142,203)	(123,556)	(538,787)	(599,727)
Interest expense, net	(20,384)	(95)	(51,686)	(10,109)
Other income (expense), net (Note 2)	(3,990)	4,751	34,192	3,360
Loss from continuing operations before provision for (benefit from) income taxes	(166,577)	(118,900)	(556,281)	(606,476)
Provision for (benefit from) income taxes (Note 1)	3,419	2,555	4,915	(123,774)
Loss from continuing operations	(169,996)	(121,455)	(561,196)	(482,702)
Loss from discontinued operations, net of tax (Note 3)	(64)	(7,207)	(703)	(20,299)

Loss from discontinued operations attributable to noncontrolling interest (Alios) (Note 3)	—	4,530	—	13,688
Net loss from discontinued operations attributable to Vertex (Note 3)	(64)	(2,677)	(703)	(6,611)
Net loss attributable to Vertex	$(170,060)	$(124,132)	$(561,899)	$(489,313)
Net loss per share from continuing operations:				
Basic	$ (0.72)	$ (0.53)	$ (2.40)	$ (2.17)
Diluted	$ (0.72)	$ (0.53)	$ (2.40)	$ (2.17)
Net loss from discontinued operations per share attributable to Vertex common shareholders:				
Basic	$ —	$ (0.01)	$ —	$ (0.03)
Diluted	$ —	$ (0.01)	$ —	$ (0.03)
Net loss per share attributable to Vertex common shareholders:				
Basic	$ (0.72)	$ (0.54)	$ (2.40)	$ (2.20)
Diluted	$ (0.72)	$ (0.54)	$ (2.40)	$ (2.20)
Shares used in per share calculations:				
Basic	236,137	230,505	234,195	222,764
Diluted	236,137	230,505	234,195	222,764

Consolidated Revenues
(in millions)
(unaudited)

	Three Months Ended				
	September 30, 2014	**June 30, 2014**	**March 31, 2014**	**December 31, 2013**	**September 30, 2013**
Product revenues					
KALYDECO revenues, net	$ 126.8	$ 113.1	$ 99.5	$ 109.5	$ 101.1
INCIVEK revenues, net	10.3	9.3	3.9	19.3	85.6
Total product revenues, net	137.1	122.4	103.5	128.8	186.7
Royalty revenues	8.4	13.0	10.7	36.9	27.0
Collaborative revenues	33.5	3.0	4.3	185.4	8.0
Total revenues	$ 179.0	$ 138.4	$ 118.5	$ 351.2	$ 221.7

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

Three Months Ended September 30, 2014		Adjustments				
	GAAP	Stock-based compensation expense	Corporate headquarters relocation (Note 4)	HCV related costs (Note 5)	Other adjustments (Note 6)	Non-GAAP
Income (loss) from operations	$(142,203)	$ 46,136	$ 43,896	$ (4,621)	$ (1,648)	$ (58,440)
Other income (expense), net	(24,374)	—		—	—	(24,374)
Income (loss) from continuing operations before provision for (benefit from) income taxes	(166,577)	46,136	43,896	(4,621)	(1,648)	(82,814)
Provision for (benefit from) income taxes	3,419	—	—	—	—	3,419
Net income (loss) from continuing operations	(169,996)	46,136	43,896	(4,621)	(1,648)	(86,233)
Net income (loss) from discontinued operations attributable to Vertex (Note 3)	(64)	—	—	64	—	—
Net income (loss) attributable to Vertex	$(170,060)	$ 46,136	$ 43,896	$ (4,557)	$ (1,648)	$ (86,233)

Net income (loss) per diluted share attributable to Vertex common shareholders (Note 7)	$ (0.72)					$ (0.37)

Three Months Ended September 30, 2013		Adjustments				
	GAAP	Stock-based compensation expense	Alios transaction (Note 3)	HCV related costs (Note 5)	Other adjustments (Note 6)	Non-GAAP
Income (loss) from operations	$(123,556)	$ 31,197	$ —	$ 16,800	$ 524	$ (75,035)
Other income (expense), net	4,656	—	—	—		4,656
Income (loss) from continuing operations before provision for (benefit from) income taxes	(118,900)	31,197	—	16,800	524	(70,379)
Provision for (benefit from) income taxes	2,555	—	—	—	—	2,555
Net income (loss) from continuing operations	(121,455)	31,197	—	16,800	524	(72,934)
Net income (loss) from discontinued operations attributable to Vertex (Note 3)	(2,677)	—	1,220	—	—	(1,457)
Net income (loss) attributable to Vertex	$(124,132)	$ 31,197	$ 1,220	$ 16,800	$ 524	$ (74,391)
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 7)	$ (0.54)					$ (0.32)

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

	Three Months Ended September 30,	
	2014	**2013**
GAAP total costs and expenses	$ 321,190	$ 345,256
Adjustments:		
Cost of product revenues and royalty expenses	(14,184)	(27,339)
Stock-based compensation expense	(46,136)	(31,197)
Corporate headquarters relocation (Note 4)	(43,896)	—
HCV related costs (Note 5)	(6,185)	(11,524)
Other adjustments (Note 6)	1,648	(524)
Non-GAAP operating costs and expenses	$ 212,437	$ 274,672
GAAP research and development expenses	$ 190,939	$ 219,442
Adjustments:		
Stock-based compensation expense	(31,131)	(19,155)
Corporate headquarters relocation (Note 4)	(3,511)	—
HCV related costs (Note 5)	(1,494)	—
Other adjustments (Note 6)	2,580	—
Non-GAAP research and development expenses	$ 157,383	$ 200,287
GAAP sales, general and administrative expenses	$ 75,224	$ 86,427
Adjustments:		
Stock-based compensation expense	(15,005)	(12,042)
Corporate headquarters relocation (Note 4)	(633)	—
HCV related costs (Note 5)	(4,532)	—
Non-GAAP sales, general and administrative expenses	$ 55,054	$ 74,385

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

Nine Months Ended September 30, 2014	Adjustments

	GAAP	Stock-based compensation expense	Corporate headquarters relocation (Note 4)	HCV related costs (Note 5)	Other adjustments (Note 6)	Non-GAAP
Income (loss) from operations	$(538,787)	$ 135,160	$ 76,206	$ 3,650	$ 3,526	$(320,245)
Other income (expense), net	(17,494)	—	(36,685)	—	—	(54,179)
Income (loss) from continuing operations before provision for (benefit from) income taxes	(556,281)	135,160	39,521	3,650	3,526	(374,424)
Provision for (benefit from) income taxes	4,915	—	—	—	—	4,915
Net income (loss) from continuing operations	(561,196)	135,160	39,521	3,650	3,526	(379,339)
Net income (loss) from discontinued operations attributable to Vertex (Note 3)	(703)	—	—	703	—	—
Net income (loss) attributable to Vertex	$(561,899)	$ 135,160	$ 39,521	$ 4,353	$ 3,526	$(379,339)
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 7)	$ (2.40)					$ (1.62)

Nine Months Ended September 30, 2013

		Adjustments				
	GAAP	Stock-based compensation expense	Alios transaction (Note 3)	HCV related costs (Note 5)	Other adjustments (Note 6)	Non-GAAP
Income (loss) from operations	$(599,727)	$ 103,585	$ —	$ 434,782	$ 1,339	$ (60,021)
Other income (expense), net	(6,749)	—		—	3,908	(2,841)
Income (loss) from continuing operations before provision for (benefit from) income taxes	(606,476)	103,585	—	434,782	5,247	(62,862)
Provision for (benefit from) income taxes	(123,774)	—		127,586	—	3,812
Net income (loss) from continuing operations	(482,702)	103,585	—	307,196	5,247	(66,674)
Net income (loss) from discontinued operations attributable to Vertex (Note 3)	(6,611)	—	(1,600)	—	—	(8,211)
Net income (loss) attributable to Vertex	$(489,313)	$ 103,585	$ (1,600)	$ 307,196	$ 5,247	$ (74,885)
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 7)	$ (2.20)					$ (0.34)

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

	Nine Months Ended September 30,	
	2014	**2013**
GAAP total costs and expenses	$ 974,646	$ 1,460,545
Adjustments:		
Cost of product revenues and royalty expenses	(46,960)	(108,013)
Stock-based compensation expense	(135,160)	(103,585)
Corporate headquarters relocation (Note 4)	(76,206)	—
HCV related costs (Note 5)	(29,026)	(424,424)
Other adjustments (Note 6)	(3,526)	(1,339)
Non-GAAP operating costs and expenses	$ 683,768	$ 823,184
GAAP research and development expenses	654,043	643,636
Adjustments:		
Stock-based compensation expense	(91,284)	(64,100)
Corporate headquarters relocation (Note 4)	(25,094)	—
HCV related costs (Note 5)	(14,834)	—
Other adjustments (Note 6)	(4,329)	—
Non-GAAP research and development expenses	$ 518,502	$ 579,536
GAAP sales, general and administrative expenses	$ 226,882	$ 283,133

Adjustments:		
Stock-based compensation expense	(43,876)	(39,485)
Corporate headquarters relocation (Note 4)	(4,524)	—
HCV related costs (Note 5)	(13,216)	—
Non-GAAP sales, general and administrative expenses	$ 165,266	$ 243,648

Condensed Consolidated Balance Sheets Data
(in thousands)
(unaudited)

	September 30, 2014	December 31, 2013
Assets		
Cash, cash equivalents and marketable securities	$ 1,477,737	$ 1,465,076
Accounts receivable, net	114,308	85,517
Inventories	16,753	14,147
Other current assets	41,298	23,836
Restricted cash	121	130
Property and equipment, net	720,878	696,911
Goodwill	30,992	30,992
Other non-current assets	3,999	2,432
Total assets	$ 2,406,086	$ 2,319,041
Liabilities and Shareholders' Equity		
Other liabilities	$ 370,734	$ 422,377
Accrued restructuring expense	55,602	28,353
Deferred revenues	55,933	70,969
Construction financing lease obligation	473,172	440,937
Senior secured term loan	294,740	—
Shareholders' equity	1,155,905	1,356,405
Total liabilities and shareholders' equity	$ 2,406,086	$ 2,319,041
Common shares outstanding	240,238	233,789

Note 1: 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 the nine months ended September 30, 2013. In connection with this impairment charge, the company recorded a credit of $127.6 million in its provision for income taxes.

Note 2: The company recorded the effect of a one-time cash payment received related to a lease agreement in Other income (expense), net during the nine months ended September 30, 2014.

Note 3: The company reflects the effect of its relationship with its collaborator Alios in its condensed consolidated statement of operations as a loss from discontinued operations. The company consolidated the financial statements of Alios from June 13, 2011 through December 31, 2013. The company presents the effect of its relationship with Alios as discontinued operations attributable to noncontrolling interest (Alios) and discontinued operations attributable to Vertex in its condensed consolidated statements of operations.

Note 4: In the three and nine months ended September 30, 2014, "Corporate headquarters relocation" primarily consists of (i) $4.1 million and $29.6 million in transition costs related to the company's relocation, respectively, (ii) $39.8 million and $46.6 million in restructuring charges related to this relocation, respectively, and (iii) $0.0 million and $36.7 million of credits to record the effect of the one-time cash payment discussed in Note 2 above, respectively.

Note 5: In the three and nine months ended September 30, 2014, "HCV related costs" primarily consists of (i) $10.3 million and $23.5 million net product revenues related to INCIVEK, respectively, (ii) $2.3 million and $12.9 million royalty revenues related to INCIVO, respectively, and a corresponding amount of royalty expenses, (iii) $0.2 million and $11.3 million net charges related to post-restructuring HCV collaborative revenues and development costs, respectively, and (iv) $4.5 million and $13.2 million related to the 2014 pharma fee and commercial costs related to INCIVEK, respectively. In the three and nine months ended September 30, 2013, HCV related costs consisted of (1) inventory write-offs related to INCIVEK of $5.3 million and $10.4

million, respectively, (2) $11.4 million in restructuring expense related to the October 2013 strategic restructuring and (3) the first quarter of 2013 VX-222 impairment charge, net of tax discussed in Note 1 above.

Note 6: In the three and nine months ended September 30, 2014, "Other adjustments" consists of (i) credits for development cost and net charges associated with VX-509 of $2.6 million and $4.3 million, respectively, (ii) restructuring credits related to a lease obligation of $0.5 million and $2.2 million, respectively and (iii) miscellaneous restructuring charges of $1.4 million recorded in the third quarter of 2014. In the three and nine months ended September 30, 2013, "Other adjustments" consists of (1) $3.9 million of interest expense related to the 2015 Notes that were converted in the second quarter of 2013 and (2) restructuring charges related to a lease obligation of $0.5 million and $1.3 million, respectively.

Note 7: Shares used in non-GAAP net income (loss) per diluted share attributable to Vertex common shareholders were 236,137,173 and 230,504,963 for the three months ended September 30, 2014 and 2013, respectively, and 234,195,172 and 222,763,995 for the nine months ended September 30, 2014 and 2013, respectively.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO (ivacaftor)

Ivacaftor (150 mg 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.

In the United States, ivacaftor is also indicated for the treatment of CF in patients age 6 and older who have one of the following mutations in the *CFTR* gene: G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R. In Canada, ivacaftor is indicated for these same mutations and additionally for G970R.

Ivacaftor is not effective in patients with CF with 2 copies of the F508del mutation (F508del/F508del) in the *CFTR* gene. The safety and efficacy of ivacaftor in children with CF younger than 6 years of age have not been established.

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 and may diminish effectiveness. Therefore, co-administration is not recommended.

The dose of ivacaftor must be adjusted when used concomitantly with strong and moderate CYP3A inhibitors or 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 KALYDECO U.S. Prescribing Information, EU Summary of Product Characteristics, Canadian Product Monograph, Australian Consumer Medicine Information and Product Information, Swiss Prescribing Information and Patient Information, and the New Zealand Datasheet and Consumer Medicine Information.

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. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen ongoing research 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 five 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 second paragraph of the press release, the information provided in the section captioned "2014 Financial Guidance," and the information provided regarding Vertex's sNDA in the U.S. and an MAA variation in Europe for people with CF who have the R117H mutation. 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, that Vertex could experience unforeseen delays in submitting regulatory filings, that regulatory authorities may not approve, or approve on a timely basis, lumacaftor in combination with ivacaftor or ivacaftor for additional indications 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 the call, please 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 can be accessed through 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. An archived webcast will be available on the company's website.

(VRTX-GEN)

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