



April 30, 2013

Vertex Reports First Quarter 2013 Financial Results and Reviews Recent Progress in Development Programs for Cystic Fibrosis and Hepatitis C

-First quarter 2013 total revenues of \$328 million, including net product revenues of \$206 million for INCIVEK in hepatitis C and \$62 million for KALYDECO in cystic fibrosis-

-Cystic fibrosis: Enrollment ongoing in Phase 3 program for VX-809 in combination with ivacaftor for people with two copies of the F508del mutation-

-Hepatitis C: multiple all-oral combination studies ongoing with the nucleotide analogue HCV polymerase inhibitor VX-135-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended March 31, 2013. The company also today increased its KALYDECO net revenue guidance for 2013 and reiterated other components of financial guidance, as provided on January 29, 2013.

Vertex reported total first quarter 2013 revenues of \$328 million, including net product revenues of \$206 million from INCIVEK[®] (telaprevir) and \$62 million from KALYDECO[™] (ivacaftor). The GAAP net loss attributable to Vertex was \$(308.0) million, or \$(1.43) per share, for the first quarter of 2013. The company's first quarter 2013 GAAP net loss includes certain charges of \$313.8 million, comprised primarily of a one-time charge, net of a tax benefit, of \$285.3 million related to an impairment of an intangible asset. Non-GAAP net income attributable to Vertex for the first quarter of 2013 was \$5.7 million, or \$0.03 per diluted share. The company reported \$1.24 billion in cash, cash equivalents and marketable securities as of March 31, 2013.

"During the first quarter of the year, we have made significant advances across our business as we continue to execute on our strategy of developing new medicines focused on serious diseases in specialty markets," said Jeffrey Leiden, M.D., Ph.D., Chair, President and Chief Executive Officer of Vertex. "With the recent initiation of a Phase 3 program in cystic fibrosis, multiple ongoing all-oral studies in hepatitis C, label-expansion studies for ivacaftor, and significant advances in our early stage pipeline, we are well positioned for continued progress over the rest of this year and beyond."

Development Program Updates

Cystic Fibrosis

Vertex's strategy in cystic fibrosis (CF) is to provide benefit to as many CF patients as possible, and to maximize the benefit for these patients, with our approved and investigational medicines.

Continued Progression of Label-Expansion Studies for Ivacaftor Monotherapy

- Three Phase 3 label-expansion studies are ongoing for ivacaftor monotherapy, including a study in people with CF ages 6 and older who have at least one copy of the R117H mutation, a study in people with CF ages 6 and older who have at least one non-G551D *CFTR* gating mutation and a study in children with CF ages 2 to 5 who have a gating mutation. Enrollment of the study of gating mutations is complete, with the first data expected in the second half of 2013. Enrollment is ongoing in the study of children ages 2 to 5 and in the study in people with the R117H mutation. Enrollment is also ongoing in a Phase 2 proof-of-concept study evaluating ivacaftor in people with CF who have clinical evidence of residual *CFTR* function. Vertex believes that ivacaftor monotherapy may be able to treat between 10% and 15% of the estimated 70,000 CF patients worldwide, pending results of clinical studies.

Initiation of Phase 3 Studies for VX-809 in Combination with Ivacaftor

- Vertex recently initiated a Phase 3 program for VX-809 in combination with ivacaftor that consists of two 24-week Phase 3 studies in people ages 12 and older with two copies of the most common mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene, known as F508del. Enrollment in the Phase 3 program is underway. Worldwide, nearly half of people with CF have two copies of the F508del mutation. Additional details on the Phase 3 program were provided in a press release issued February 26, 2013.

Phase 2 Data for VX-661 in Combination with Ivacaftor

- Vertex recently announced data from a Phase 2 study of VX-661 in combination with ivacaftor in people with two copies of the F508del mutation. In the study, treatment with a combination of VX-661 and ivacaftor for 28 days in the two highest dose groups resulted in mean relative increases in lung function (percent predicted FEV₁) of 9.0% (p=0.01) and 7.5% (p=0.02) versus placebo. In contrast, patients who received placebo showed a 0.03% mean relative change in lung function at Day 28 (within-group). In the study, VX-661 was generally well-tolerated, both as monotherapy and in combination with ivacaftor, and most adverse events were pulmonary in nature, were mild to moderate in severity and similar between the treatment groups and those who received placebo. Additional details on these data were provided in a press release issued April 18, 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.

Multiple Ongoing Studies of VX-135 as Part of All-Oral Treatment Regimens

- Vertex is currently evaluating multiple all-oral regimens that include VX-135, Vertex's nucleotide analogue hepatitis C virus (HCV) polymerase inhibitor. Ongoing and planned studies include:
 - *Genotype 1*
 - Two Phase 2 studies of VX-135 in combination with ribavirin are currently ongoing in people with genotype 1 HCV infection. Vertex today announced that one of these studies is fully enrolled.
 - A drug-drug interaction study of VX-135 in combination with simeprevir is ongoing in healthy volunteers. Simeprevir (TMC435) is a once-daily investigational hepatitis C protease inhibitor being jointly developed by Janssen R&D Ireland and Medivir AB.
 - *Genotypes 1, 2 or 3 and People with Cirrhosis*
 - Vertex plans to conduct two Phase 2 studies of VX-135 and Bristol-Myers Squibb's NS5A replication complex inhibitor daclatasvir. An initial study in people with genotype 1 HCV infection is planned for the second quarter of 2013. Vertex plans to begin a subsequent study in people infected with genotype 1, 2 or 3 HCV, including those with cirrhosis, in the second half of 2013, pending data from the initial study.
 - Vertex expects to obtain the first data from all-oral studies of VX-135 in the second half of 2013, including data from the initial study of VX-135 with daclatasvir and from the studies of VX-135 with ribavirin.

Data for ALS-2200 (VX-135) in Genotypes 2, 3 and 4 and in People with Cirrhosis Presented at EASL

- At the 48th Annual Meeting of the European Association for the Study of the Liver (EASL), Vertex announced new data from a 7-day viral kinetic study of ALS-2200 in people with genotypes 2, 3 and 4 HCV and those with cirrhosis. The data showed significant reductions in HCV RNA after seven days of dosing with ALS-2200 (200 mg) once daily and were consistent with previously reported data in people with genotype 1 chronic HCV infection. ALS-2200 was well-tolerated in this study, there were no serious adverse events and no patients discontinued due to adverse events. Additional details on these data were provided in a press release issued April 23, 2013.

Data from CONCISE Study of Telaprevir Presented at EASL

- Also at EASL, Vertex announced new data from an interim analysis of the CONCISE study, which showed that treatment with telaprevir combination therapy for a total of 12 or 24 weeks resulted in high viral cure rates in people with genotype 1 HCV with the IL28B CC genotype who had a rapid viral response and completed at least 12 weeks of treatment. The safety profile of telaprevir combination therapy observed in the CONCISE study through the time of the interim analysis was similar to that seen in previously reported clinical trials. Additional details on these data were provided in a press release issued April 24, 2013.

Autoimmune Diseases

Vertex's strategy in autoimmune diseases is to maximize the value of VX-509 across multiple autoimmune diseases globally. The company will evaluate collaborative opportunities that provide funding and capabilities to broaden and accelerate global development of VX-509.

Enrollment Complete in Phase 2b Study of VX-509 in Rheumatoid Arthritis

- Vertex today announced that enrollment is complete in a 24-week Phase 2b study of VX-509, a selective JAK3 inhibitor, in people with moderate to severe rheumatoid arthritis (RA) receiving methotrexate. The primary endpoints of this study will be measured after 12 weeks of treatment, and data from this analysis are expected in the second half of 2013.

First Quarter 2013 Financial Results

Total Revenues: Total revenues for the first quarter of 2013 were \$328.4 million, compared with \$438.7 million in total revenues for the first quarter of 2012. The components of total revenues for the first quarter of 2013 and 2012 were:

	Three Months Ended March 31,	
	2013	2012
	(in millions)	
Product revenues		
INCIVEK revenues, net	\$ 205.6	\$ 356.9
KALYDECO revenues, net	61.8	18.4
Total product revenues, net	267.4	375.4
Royalty revenues		
Royalty revenues from INCIVO	39.0	32.9
Other royalty revenues	4.6	6.0
Total royalty revenues	43.6	39.0
Collaborative revenues	17.4	24.4
Total revenues	\$ 328.4	\$ 438.7

A table of the components of total revenues for the first quarter of 2013 and each quarter in 2012 is provided following the Condensed Consolidated Statements of Operations Data.

• Net Product Revenues from INCIVEK

Vertex's first quarter 2013 net product revenues from INCIVEK were \$205.6 million, compared to \$356.9 million for the first quarter of 2012. The reduced revenues from INCIVEK were due to fewer HCV patients initiating treatment in the first quarter of 2013 compared to the first quarter of 2012.

• Net Product Revenues from KALYDECO

Vertex's first quarter 2013 net product revenues from KALYDECO were \$61.8 million, compared to \$18.4 million for the first quarter of 2012. The increased revenues, compared to the first quarter of 2012, resulted primarily from the rapid uptake of KALYDECO in the vast majority of eligible patients in the U.S. following FDA approval in January 2012.

• Royalty Revenues from INCIVO®

Vertex recognized \$39.0 million in INCIVO royalty revenues for the first quarter of 2013 from our collaborator Janssen, compared to \$32.9 million in INCIVO royalty revenues for the first quarter of 2012. The increase in INCIVO royalties was due to expanded availability of INCIVO in international markets.

Cost of Product Revenues: Cost of product revenues was \$31.0 million for the first quarter of 2013, compared to cost of product revenues of \$25.9 million for the first quarter of 2012. The increase in cost of product revenues was due to a \$9.3 million commercial milestone related to net sales of KALYDECO under our agreement with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT).

Research and Development (R&D) Expenses: R&D expenses were \$218.1 million for the first quarter of 2013, including \$23.3 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, compared to \$196.4 million for the first quarter of 2012, including \$21.1 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, hepatitis C and rheumatoid arthritis, including initiation of a pivotal program for a combination of VX-809 and ivacaftor and advancement of all-oral studies for VX-135.

Sales, General and Administrative (SG&A) Expenses: SG&A expenses were \$92.9 million for the first quarter of 2013, including \$13.1 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, compared to \$111.1 million for the first quarter of 2012, including \$11.6 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.

GAAP Net Income (Loss) Attributable to Vertex: Vertex's first quarter 2013 GAAP net loss was \$(308.0) million, or \$(1.43) per share. The company's first quarter 2013 GAAP net loss includes certain charges of \$313.8 million, comprised primarily of a one-time charge of \$412.9 million, which was partially offset by a tax benefit of \$127.6 million, related to an impairment of an intangible hepatitis C asset (VX-222). Vertex's GAAP net income for the first quarter of 2012 was \$91.6 million, or \$0.43 per diluted share, including \$27.0 million in certain charges.

Non-GAAP Net Income Attributable to Vertex: Vertex's first quarter 2013 non-GAAP net income was \$5.7 million, or \$0.03 per diluted share. Vertex's non-GAAP net income for the first quarter of 2012 was \$118.6 million, or \$0.55 per diluted share. The decrease in the company's first quarter 2013 non-GAAP net income, compared to the first 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 first quarter of 2013 were consistent with the first quarter of 2012.

Cash Position: As of March 31, 2013, Vertex had \$1.24 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.

Convertible Debt: As of March 31, 2013, Vertex had \$400.0 million in convertible debt due in October 2015. The conversion price of the debt is \$48.83 per share and is callable on or after October 1, 2013. Vertex holds a provisional redemption option that allows the debt to be called prior to October 1, 2013 if the closing price of Vertex shares is above \$63.48 per share for 20 of 30 consecutive trading days.

2013 Financial Guidance

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

Vertex today updated its financial guidance for full-year 2013 KALYDECO net revenues. The company now expects full-year 2013 KALYDECO net revenues to be in the range of \$300 million to \$340 million. The prior range, provided on January 29, 2013, was for full-year 2013 KALYDECO net revenues to be in the range of \$280 million to \$320 million.

The company today reiterated its financial guidance for total 2013 revenues to be in the range of \$1.10 billion to \$1.25 billion. The company also reiterated its guidance for total 2013 non-GAAP operating expenses, excluding cost of revenues, stock-based compensation expense, intangible asset impairment charges and Alios expenses related to the accounting for the collaboration with Vertex, of \$1.09 billion to \$1.15 billion, including full-year 2013 R&D expenses of \$750 million to \$790 million and full-year 2013 SG&A expenses of \$340 million to \$360 million.

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 first quarter 2013 and 2012 non-GAAP net income excluding stock-based compensation expense, restructuring expense, intangible asset impairment charges, net of tax, 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
First Quarter Results
Condensed Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended	
	March 31,	
	2013	2012
Revenues:		
Product revenues, net	\$ 267,381	\$ 375,375
Royalty revenues	43,573	38,981
Collaborative revenues	17,414	24,381

Total revenues	328,368	438,737
Costs and expenses:		
Cost of product revenues	30,955	25,918
Royalty expenses	11,788	13,293
Research and development expenses (R&D)	218,095	196,371
Sales, general and administrative expenses (SG&A)	92,879	111,146
Restructuring expense	39	360
Intangible asset impairment charge (Note 1)	412,900	—
Total costs and expenses	766,656	347,088
Income (loss) from operations	(438,288)	91,649
Other income (expense), net	(4,652)	(3,741)
Income (loss) before provision for (benefit from) income taxes	(442,940)	87,908
Provision for (benefit from) income taxes (Note 1)	(130,313)	32
Net income (loss)	(312,627)	87,876
Net loss attributable to noncontrolling interest (Note 2)	4,611	3,714
Net income (loss) attributable to Vertex	\$ (308,016)	\$ 91,590

Net income (loss) per share attributable to Vertex common shareholders:

Basic	\$ (1.43)	\$ 0.44
Diluted	\$ (1.43)	\$ 0.43

Shares used in per share calculations:

Basic	215,421	208,018
Diluted	215,421	219,264

Consolidated Revenues
(in millions)
(unaudited)

Three Months Ended

	March 31, 2013	December 31, 2012	September 30, 2012	June 30, 2012	March 31, 2012
Product revenues					
INCIVEK revenues, net	\$ 205.6	\$ 222.8	\$ 254.3	\$ 327.7	\$ 356.9
KALYDECO revenues, net	61.8	58.5	49.2	45.5	18.4
Total product revenues, net	267.4	281.3	303.5	373.3	375.4
Royalty revenues					
Royalty revenues from INCIVO	39.0	36.8	20.0	28.0	32.9
Other royalty revenues	4.6	6.7	5.6	5.5	6.0
Total royalty revenues	43.6	43.5	25.6	33.5	39.0
Collaborative revenues	17.4	9.2	6.9	11.6	24.4
Total revenues	\$ 328.4	\$ 334.0	\$ 336.0	\$ 418.3	\$ 438.7

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

Three Months Ended March 31,
2013

GAAP	Adjustments					Non-GAAP
	Alios Transaction	Stock-based Compensation Expense	Intangible Asset Impairment Charge, Net of Tax	Restructuring Expense		

Income (loss) from operations	\$ (438,288)	\$ 5,289	\$ 31,152	\$ 412,900	\$ 39	\$ 11,092
Other income (expense), net	(4,652)	8	—	—	—	(4,644)
Income (loss) before provision for (benefit from) income taxes	(442,940)	5,297	31,152	412,900	39	6,448
Provision for (benefit from) income taxes	(130,313)	3,426	—	127,586	—	699
Net income (loss)	(312,627)	1,871	31,152	285,314	39	5,749
Net loss (income) attributable to noncontrolling interest (Alios)	4,611	(4,611)	—	—	—	—
Net income (loss) attributable to Vertex	<u>\$ (308,016)</u>	<u>\$ (2,740)</u>	<u>\$ 31,152</u>	<u>\$ 285,314</u>	<u>\$ 39</u>	<u>\$ 5,749</u>
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 3)	\$ (1.43)					\$ 0.03

Three Months Ended March 31, 2012

	GAAP	Adjustments				Non-GAAP
		Alios Transaction	Stock-based Compensation Expense	Intangible Asset Impairment Charge, Net of Tax	Restructuring Expense	
Income (loss) from operations	\$91,649	\$ 5,086	\$ 27,627	\$ —	\$ 360	\$124,722
Other income (expense), net	(3,741)	(62)	—	—	—	(3,803)
Income (loss) before provision for (benefit from) income taxes	87,908	5,024	27,627	—	360	120,919
Provision for (benefit from) income taxes	32	2,280	—	—	—	2,312
Net income (loss)	87,876	2,744	27,627	—	360	118,607
Net loss (income) attributable to noncontrolling interest (Alios)	3,714	(3,714)	—	—	—	—
Net income (loss) attributable to Vertex	<u>\$91,590</u>	<u>\$ (970)</u>	<u>\$ 27,627</u>	<u>\$ —</u>	<u>\$ 360</u>	<u>\$118,607</u>
Net income (loss) per diluted share attributable to Vertex common shareholders (Note 3)	\$ 0.43					\$ 0.55

Three Months Ended March 31,

	2013	2012
GAAP operating costs and expenses	\$ 766,656	\$ 347,088
Adjustments:		
Cost of product revenues	(30,955)	(25,918)
Royalty expenses	(11,788)	(13,293)
Stock-based compensation expense	(31,152)	(27,627)
Alios transaction	(5,289)	(5,086)
Intangible asset impairment charge	(412,900)	—
Restructuring expense	(39)	(360)
Non-GAAP operating costs and expenses	<u>\$ 274,533</u>	<u>\$ 274,804</u>
GAAP research and development expenses	\$ 218,095	\$ 196,371
Adjustments:		
Stock-based compensation expense	(19,273)	(17,161)
Alios transaction	(4,048)	(3,960)
Non-GAAP research and development expenses	<u>\$ 194,774</u>	<u>\$ 175,250</u>
GAAP sales, general, and administrative expenses	\$ 92,879	\$ 111,146
Adjustments:		
Stock-based compensation expense	(11,879)	(10,466)
Alios transaction	(1,241)	(1,126)

Non-GAAP sales, general, and administrative expenses \$ 79,759 \$ 99,554

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

	<u>March 31, 2013</u>	<u>December 31, 2012</u>
Assets		
Cash, cash equivalents and marketable securities	\$ 1,239,354	\$ 1,321,215
Restricted cash and cash equivalents (Alios) (Note 2)	63,008	69,983
Accounts receivable, net	194,054	143,250
Inventories	21,532	30,464
Other current assets	47,835	24,673
Restricted cash	31,934	31,934
Property and equipment, net	504,232	433,609
Intangible assets (Note 1)	250,600	663,500
Goodwill	30,992	30,992
Other non-current assets	8,693	9,668
Total assets	<u>\$ 2,392,234</u>	<u>\$ 2,759,288</u>
Liabilities and Shareholders' Equity		
Other liabilities	\$ 398,628	\$ 429,372
Accrued restructuring expense	22,459	23,328
Deferred tax liability (Note 1)	151,664	280,367
Deferred revenues	125,830	123,808
Construction financing lease obligation	316,821	268,031
Convertible notes (due 2015)	400,000	400,000
Noncontrolling interest (Alios) (Note 2)	230,717	235,202
Shareholders' equity (Vertex)	746,115	999,180
Total liabilities and shareholders' equity	<u>\$ 2,392,234</u>	<u>\$ 2,759,288</u>
Common shares outstanding	218,652	217,287

Note 1: As of March 31, 2013, the intangible assets and deferred tax liability reflected in the Condensed Consolidated Balance Sheets Data relate to the company's collaboration agreement with Alios BioPharma, Inc.

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 2: The company has consolidated the financial statements of its collaborator Alios as of March 31, 2013, December 31, 2012, and for the three months ended March 31, 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 3: Shares used in non-GAAP net income per diluted share attributable to Vertex common shareholders were 218,317,000 and 219,264,000 for the three months ended March 31, 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 CF patients with two 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.

High 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. Moderate transaminase elevations are common in subjects with CF. Overall, the incidence and clinical features of transaminase elevations in clinical trials was similar between subjects in the ivacaftor and placebo treatment groups. In the subset of patients with a medical history of elevated transaminases, increased ALT or AST have been reported more frequently in patients receiving ivacaftor compared to placebo.

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 concomitantly used 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 full product labeling for each country where ivacaftor is approved. Patients should tell their healthcare providers about any side effect that bothers them or doesn't 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>, and the KALYDECO Canadian Product Monograph at www.vrtx.ca.

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 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 non-prescription 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 creates new possibilities in medicine. Our team discovers, develops and commercializes innovative therapies 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 hepatitis C, cystic fibrosis, rheumatoid arthritis and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, Mass., we now have ongoing worldwide research programs and sites in the U.S., U.K. and Canada. Today, Vertex has more than 2,000 employees around the world, and for three years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences.

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 section captioned "2013 Financial Guidance" and statements regarding (i) Vertex's strategies in cystic fibrosis, HCV and autoimmune diseases; (ii) the potential timing of clinical data from ongoing clinical trials; (iii) the percentage of patients that Vertex may be able to treat with ivacaftor monotherapy; (iv) ongoing and planned studies involving VX-135; and (v) potential collaborative opportunities that could providing funding and capabilities to broaden and accelerate global development of VX-509. 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 total revenues and/or 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 not be favorable, that the initiation of planned studies may be delayed or prevented, 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

Vertex will host a conference call and webcast today, April 30, 2013 at 5:00 p.m. ET to review financial results and recent developments. 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.

To listen to the live call on the telephone, dial 1-866-501-1537 (United States and Canada) or 1-720-545-0001 (International). To ensure a timely connection, it is recommended that users register at least 15 minutes prior to the scheduled webcast.

The conference ID number for the live call and replay is 30435497.

The call will be available for replay via telephone commencing April 30, 2013 at 8:00 p.m. ET running through 5:00 p.m. ET on May 7, 2013. The replay phone number for the United States and Canada is 1-855-859-2056. The international replay number is 1-404-537-3406.

Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. ET on May 7, 2013. Vertex is also providing a podcast MP3 file available for download on the Vertex website at www.vrtx.com.

(VRTX-GEN)

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News Provided by Acquire Media