



May 1, 2014

Vertex Reports First Quarter 2014 Financial Results and Provides Updates on Key Business Priorities

-Net product revenues of \$100 million for KALYDECO in cystic fibrosis-

-Cash, cash equivalents and marketable securities of \$1.32 billion on March 31, 2014-

-Six-month dosing period complete for Phase 3 TRAFFIC and TRANSPORT studies of lumacaftor in combination with ivacaftor for people with CF homozygous for the F508del mutation-

BOSTON--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended March 31, 2014 and provided updates on key business priorities, including ongoing studies in cystic fibrosis (CF).

Vertex reported total first quarter 2014 revenues of \$118 million, including revenues of \$100 million from KALYDECO® (ivacaftor). The GAAP net loss for the first quarter of 2014 was \$(232) million, or \$(1.00) per share, including net charges of \$81 million. Non-GAAP net loss for the first quarter of 2014 was \$(151) million, or \$(0.65) per share. As of March 31, 2014, Vertex had \$1.32 billion in cash, cash equivalents and marketable securities.

Vertex also provided updated financial guidance for 2014 non-GAAP revenues and non-GAAP operating expenses and maintained its guidance for 2014 KALYDECO revenues as provided on January 29, 2014. The updated guidance reflects the removal of revenues and costs following Vertex's decision to end further investment in hepatitis C research and development activities.

"In 2014, we are focused on three critical objectives to support the long-term growth of our business - advancing our clinical development program in cystic fibrosis, prudently managing our financial profile, and growing our clinical pipeline to create future medicines," commented Jeffrey Leiden, M.D., Ph.D., Chairman, President and Chief Executive Officer of Vertex. "We recognize the unique opportunity we have to develop multiple new medicines for people with CF, and with our decision to end further investment in hepatitis C, we have increased our focus on CF and are advancing key clinical studies as rapidly as possible. With a cash position of more than \$1.3 billion and the potential for further growth in revenues from KALYDECO, our financial position remains strong, supporting our expanded investment in CF as well as our continued investment in research programs to create future medicines for other serious and rare diseases."

First Quarter 2014 Non-GAAP Financial Results

The first quarter 2014 non-GAAP financial results reflect the removal of hepatitis C costs and revenues and primarily exclude stock-based compensation expense and transition costs related to the relocation of our corporate headquarters. The first quarter 2013 non-GAAP financial results primarily exclude expenses related to Alios (HCV), stock-based compensation expense and an asset impairment charge.

Total Non-GAAP Revenues: Total non-GAAP revenues, which exclude hepatitis C revenues and royalties, for the first quarter of 2014 were \$108.2 million, including \$99.5 million in net product revenues from KALYDECO and \$8.7 million from royalties and collaborative revenues.

• Net Product Revenues from KALYDECO

Vertex's first quarter 2014 net product revenues from KALYDECO were \$99.5 million compared to \$61.8 million for the first quarter of 2013. The increased revenues, compared to the first quarter of 2013, resulted primarily from increased revenues from KALYDECO in eligible patients in Europe, in addition to those previously being treated in the U.S. In 2014, further growth and achievement of the company's total 2014 net product revenue guidance for KALYDECO is dependent on completion of reimbursement discussions in Australia and Canada for eligible patients with the G551D mutation and on the potential further expansion of the KALYDECO label globally.

Three Months Ended March 31, 2014

(in millions)

GAAP Revenues HCV related Revenues Non-GAAP Revenues

Product revenues

KALYDECO revenues, net	\$ 99.5	\$ —	\$ 99.5
INCIVEK revenues, net	3.9	(3.9)	—
Total product revenues, net	103.5	(3.9)	99.5
Royalty revenues	10.7	(4.9)	5.8
Collaborative revenues	4.3	(1.4)	2.9
Total revenues	<u>\$ 118.5</u>	<u>(10.2)</u>	<u>\$ 108.2</u>

Non-GAAP Operating Expenses: Total non-GAAP operating expenses for the first quarter of 2014 were \$233.9 million. Non-GAAP operating expenses include:

- **Research and Development (R&D) Expenses:** Non-GAAP R&D expenses were \$181.5 million for the first quarter of 2014, compared to \$194.8 million in non-GAAP R&D expenses for the first quarter of 2013. This reduction was the result of a narrowed clinical focus and prioritization of the business toward medicines for CF and other expense management activities.
- **Sales, General and Administrative (SG&A) Expenses:** Non-GAAP SG&A expenses were \$52.4 million for the first quarter of 2014, compared to \$79.8 million in non-GAAP SG&A expenses for the first quarter of 2013. This reduction was the result of a narrowed commercial focus toward CF medicines and other expense management activities.

Non-GAAP Net Income (Loss) Attributable to Vertex: Vertex's first quarter 2014 non-GAAP net loss was \$(151.4) million, or \$(0.65) per diluted share, compared to non-GAAP net income of \$5.7 million, or \$0.03 per diluted share, for the first quarter of 2013. The non-GAAP net loss for the first quarter of 2014 was primarily the result of a reduction in INCIVEK net product revenues and in royalties from INCIVO.

Cash Position: As of March 31, 2014, Vertex had \$1.32 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.

2014 Financial Guidance

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

- **Total Revenues:** Vertex today revised its estimate for total 2014 non-GAAP net revenues. The company now expects total non-GAAP revenues of \$520 to \$550 million, which excludes revenues related to hepatitis C (INCIVEK and INCIVO royalties).
 - **KALYDECO Net Revenues:** Vertex today maintained its estimate for total 2014 KALYDECO net revenues of \$470 to \$500 million. Achieving this guidance depends on anticipated revenues from Australia and Canada following the potential completion of reimbursement discussions, from Europe following the potential approval of KALYDECO for use in additional gating mutations and in the U.S. from the use of KALYDECO in eight additional mutations for which it was approved for use in February 2014.
- **Non-GAAP Operating Expenses:** Vertex today revised its estimate for its 2014 non-GAAP operating expenses. The company now expects 2014 non-GAAP operating expenses to be in the range of \$890 to \$930 million. The anticipated decrease in total non-GAAP operating expenses is primarily attributable to expected savings from the company's decision to end further investment in hepatitis C.

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 the first quarter of 2014 revenues and expenses related to hepatitis C consistent with the company's decision to end further investment in hepatitis C, stock-based compensation expense, transition costs related to the relocation of our corporate headquarters and other adjustments and (ii) in the first quarter of 2013 expenses related to Alios (HCV), stock-based compensation expense, the impairment of VX-222 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 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 information.

First Quarter 2014 GAAP Financial Results

Total Revenues: Total revenues for the first quarter of 2014 were \$118.5 million compared with \$328.4 million in total revenues for the first quarter of 2013. First quarter 2014 revenues are comprised primarily of \$99.5 million in KALYDECO net revenues, compared to \$61.8 million for the first quarter of 2013, and also include an aggregate of \$18.9 million in net product revenues from INCIVEK, royalty revenues and collaborative revenues. INCIVEK net revenues were \$3.9 million for the first quarter of 2014, compared to \$205.6 million for the first quarter of 2013.

Operating Costs and Expenses: Total operating costs and expenses for the first quarter of 2014 were \$334.8 million, including

certain charges of \$100.9 million, compared to \$766.7 million for the first quarter of 2013, including certain charges of \$492.1 million. GAAP operating costs and expenses include:

- **R&D Expenses:** R&D expenses were \$239.0 million for the first quarter of 2014, including \$57.4 million of certain charges, compared to \$218.1 million for the first quarter of 2013, including \$23.3 million of certain charges.
- **Sales, General and Administrative (SG&A) Expenses:** SG&A expenses were \$74.2 million for the first quarter of 2014, including \$21.8 million of certain charges, compared to \$92.9 million for the first quarter of 2013, including \$13.1 million of certain charges.

Net Loss Attributable to Vertex: Vertex's first quarter 2014 net loss was \$(232.5) million, or \$(1.00) per share, and includes net charges of \$81.1 million, comprised primarily of hepatitis C revenues and expenses, stock-based compensation expense and transition costs related to the relocation of our corporate headquarters. Vertex's GAAP net loss for the first quarter of 2013 was \$(308.0) million, or \$(1.43) per share, including \$313.8 million in certain charges, 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).

Research and Development Updates

Vertex today provided the following research and development updates:

Cystic Fibrosis (CF)

Global Availability of KALYDECO (ivacaftor)

- KALYDECO is currently available to all eligible patients in the United States, England, Scotland, Northern Ireland, Wales, the Republic of Ireland, France, Germany, the Netherlands, Austria, Denmark, Sweden, Norway, Greece and Italy. KALYDECO is also approved in Australia and Canada, and Vertex is in active discussions with relevant agencies in these countries to expand access to KALYDECO for eligible patients through public reimbursement. There are approximately 300 people age 6 years and older who have the G551D mutation in Australia and Canada.
- In February, the U.S. Food and Drug Administration (FDA) approved a supplemental New Drug Application (sNDA) for KALYDECO (ivacaftor) for people with cystic fibrosis (CF) ages 6 and older who have one of eight additional mutations in the *cystic fibrosis transmembrane conductance regulator (CFTR)* gene. With the approval of the sNDA, KALYDECO is now approved for use in people with CF with the following nine mutations: G551D, G178R, S549N, S549R, G551S, G1244E, S1251N, S1255P and G1349D. In the United States, approximately 150 people ages 6 and older have one of the additional eight mutations for which KALYDECO is now approved.

Additional Activities and Clinical Studies Aimed at Increasing the Number of People Eligible for Ivacaftor

- **Gating Mutations in Europe:** Vertex submitted a Marketing Authorization Application (MAA) variation in Europe for approval of ivacaftor in people with CF ages 6 years and older who have a non-G551D gating mutation. Outside the U.S., approximately 250 people ages 6 years and older have these non-G551D gating mutations, and the large majority of these patients are in Europe.
- **R117H Mutation:** In December 2013, Vertex announced data from a Phase 3 study of people with CF ages 6 years and older with one copy of the R117H mutation. The study did not meet its primary endpoint of the absolute change from baseline in FEV₁ (percent predicted forced expiratory volume in one second), however a pre-specified subset analysis in patients 18 years of age and older showed statistically significant improvements in lung function and other key secondary endpoints. Based on these data, the company plans to submit an sNDA in the U.S. and MAA variation in Europe for people ages 18 and older who have the R117H mutation. The sNDA submission is planned for mid-2014, followed by the MAA variation submission in the second half of 2014. In North America, Europe and Australia, approximately 700 people with CF ages 18 and older have at least one copy of an R117H mutation.
- **Children Ages 2 to 5 with Gating Mutations:** A Phase 3 study of ivacaftor in children with CF ages 2 to 5 with a gating mutation is fully enrolled, and all patients have completed the 24-week dosing period. The primary endpoint of this study is safety, and secondary endpoints include pharmacokinetics, change in sweat chloride and change in weight. Data from this study are expected in the third quarter of 2014 to support a potential NDA submission and MAA variation in the second half of 2014. In North America, Europe and Australia, approximately 300 children ages 2 to 5 have the gating mutations evaluated in this study.
- **Residual Function Study:** Enrollment is complete in a Phase 2 proof-of-concept study evaluating ivacaftor in people with CF who have clinical evidence of residual CFTR function. Data from this study are expected in the second quarter of 2014. In North America, Europe and Australia, more than 3,000 people ages 6 and older have non-R117H mutations that result in residual function.

Phase 3 Program of Lumacaftor in Combination with Ivacaftor in People with CF Who Have Two Copies of the F508del Mutation

- All patients have now completed the six-month dosing period in the global Phase 3 TRAFFIC and TRANSPORT studies evaluating lumacaftor (VX-809) in combination with ivacaftor in people with CF ages 12 and older who have two copies (homozygous) of the F508del mutation. Vertex expects 24-week data from these studies to be available in mid-2014 to support the potential submission of an NDA and MAA for the combination therapy in people homozygous for the F508del mutation in the second half of 2014.

VX-661 in Combination with Ivacaftor

- Vertex recently began dosing in a 12-week Phase 2 study of VX-661 in combination with ivacaftor in people with CF who have two copies of the F508del mutation. The study is designed to evaluate safety, efficacy and pharmacokinetics to characterize VX-661 for further clinical development.
- In a separate press release issued today, Vertex announced that treatment with the combination of VX-661 and KALYDECO in a 28-day Phase 2 study showed statistically significant improvements in lung function (FEV₁), as well as decreases in sweat chloride, in people (n=18) with the F508del mutation and G551D mutation who were already taking KALYDECO. VX-661 was generally well-tolerated when dosed in combination with KALYDECO, and all 18 patients completed the 28-day treatment period.
- Vertex also announced today that the U.S. FDA has granted VX-661 Orphan Drug Designation. The FDA grants Orphan Drug Designation to medicines intended to treat less than 200,000 people in the U.S.

Hepatitis C

- Vertex today announced that it has amended the terms of its agreement with Alios BioPharma regarding the development and commercialization of the nucleotide analogue hepatitis C virus (HCV) polymerase inhibitor VX-135. Based on the revised agreement and the rapid changes in the hepatitis C treatment landscape following the introduction of new oral therapies, Vertex plans to out-license VX-135 and to end further investment into research and development efforts in hepatitis C.

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

	Three Months Ended	
	March 31,	
	2014	2013
Revenues:		
Product revenues, net	\$ 103,461	\$ 267,381
Royalty revenues	10,733	43,573
Collaborative revenues	4,257	17,414
Total revenues	<u>118,451</u>	<u>328,368</u>
Costs and expenses:		
Cost of product revenues	8,572	30,955
Royalty expenses	6,904	11,788
Research and development expenses (R&D)	238,963	218,095
Sales, general and administrative expenses (SG&A)	74,212	92,879
Restructuring expenses	6,188	39
Intangible asset impairment charge (Note 1)	—	412,900
Total costs and expenses	<u>334,839</u>	<u>766,656</u>
Loss from operations	<u>(216,388)</u>	<u>(438,288)</u>
Interest expense, net	(15,717)	(3,465)
Other income (expense), net	451	(1,187)
Loss before provision for (benefit from) income taxes	<u>(231,654)</u>	<u>(442,940)</u>
Provision for (benefit from) income taxes (Note 1)	803	(130,313)
Net loss	<u>(232,457)</u>	<u>(312,627)</u>
Net loss attributable to noncontrolling interest (Note 2)	—	4,611
Net loss attributable to Vertex	<u>\$ (232,457)</u>	<u>\$ (308,016)</u>
Net loss per share attributable to Vertex common shareholders:		
Basic	\$ (1.00)	\$ (1.43)
Diluted	\$ (1.00)	\$ (1.43)

Shares used in per share calculations:

Basic	232,887	215,421
Diluted	232,887	215,421

Consolidated Revenues

(in millions)

(unaudited)

Three Months Ended

	March 31, 2014	December 31, 2013	September 30, 2013	June 30, 2013	March 31, 2013
Product revenues					
KALYDECO revenues, net	\$ 99.5	\$ 109.5	\$ 101.1	\$ 99.0	\$ 61.8
INCIVEK revenues, net	3.9	19.3	85.6	155.8	205.6
Total product revenues, net	103.5	128.8	186.7	254.8	267.4
Royalty revenues	10.7	36.9	27.0	49.1	43.6
Collaborative revenues	4.3	185.4	8.0	6.8	17.4
Total revenues	\$ 118.5	\$ 351.2	\$ 221.7	\$ 310.8	\$ 328.4

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

(in thousands, except per share amounts)

(unaudited)

Three Months Ended March 31, 2014

	GAAP	Adjustments				Non-GAAP
		Stock-based Compensation Expense (Note 3)	Corporate Headquarters Relocation	HCV Related Costs (Note 4)	Other Adjustments (Note 5)	
Income (loss) from operations	\$(216,388)	\$ 46,580	\$ 19,611	\$ 11,216	\$ 3,656	\$(135,325)
Other income (expense), net	(15,266)	—	—	—	—	(15,266)
Income (loss) before provision for (benefit from) income taxes	(231,654)	46,580	19,611	11,216	3,656	(150,591)
Provision for income taxes	803	—	—	—	—	803
Net income (loss)	<u>\$(232,457)</u>	<u>\$ 46,580</u>	<u>\$ 19,611</u>	<u>\$ 11,216</u>	<u>\$ 3,656</u>	<u>\$(151,394)</u>
Net loss per diluted share attributable to Vertex common shareholders (Note 6)	\$ (1.00)					\$ (0.65)

Three Months Ended March 31, 2013

	GAAP	Adjustments				Non- GAAP
		Stock-based Compensation Expense (Note 3)	Alios Transaction (Note 2)	Intangible Asset Charge (VX-222) (Note 1)	Other Adjustments (Note 5)	
Income (loss) from operations	\$(438,288)	\$ 31,152	\$ 5,289	\$ 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)	31,152	5,297	412,900	39	6,448
Provision for (benefit from) income taxes	(130,313)	—	3,426	127,586	—	699
Net income (loss)	(312,627)	31,152	1,871	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>\$ 31,152</u>	<u>\$ (2,740)</u>	<u>\$ 285,314</u>	<u>\$ 39</u>	<u>\$ 5,749</u>
Net income (loss) per diluted share attributable to Vertex common						

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

(in thousands)

(unaudited)

	Three Months Ended March 31,	
	2014	2013
GAAP total costs and expenses	\$ 334,839	\$ 766,656
Adjustments:		
Cost of product revenues and royalty expenses	(15,476)	(42,743)
Stock-based compensation expense (Note 3)	(46,580)	(31,152)
Corporate headquarters relocation	(19,611)	—
HCV related costs (Note 4)	(15,570)	(412,900)
Alios transaction (Note 2)	—	(5,289)
Other adjustments (Note 5)	(3,656)	(39)
Non-GAAP operating costs and expenses	\$ 233,946	\$ 274,533
GAAP research and development expenses	\$ 238,963	\$ 218,095
Adjustments:		
Stock-based compensation expense (Note 3)	(32,900)	(19,273)
Corporate headquarters relocation	(12,201)	—
HCV related costs (Note 4)	(9,002)	—
Alios transaction (Note 2)	—	(4,048)
Other adjustments (Note 5)	(3,325)	—
Non-GAAP research and development expenses	\$ 181,535	\$ 194,774
GAAP sales, general and administrative expenses	\$ 74,212	\$ 92,879
Adjustments:		
Stock-based compensation expense (Note 3)	(13,680)	(11,879)
Corporate headquarters relocation	(2,200)	—
HCV related costs (Note 4)	(5,921)	—
Alios transaction (Note 2)	—	(1,241)
Non-GAAP sales, general and administrative expenses	\$ 52,411	\$ 79,759

Condensed Consolidated Balance Sheets Data

(in thousands)

(unaudited)

	March 31, 2014	December 31, 2013
Assets		
Cash, cash equivalents and marketable securities	\$ 1,324,200	\$ 1,465,076
Accounts receivable, net	59,858	85,517
Inventories	11,261	14,147
Other current assets	35,011	23,836
Restricted cash	130	130
Property and equipment, net	726,204	696,911
Goodwill	30,992	30,992
Other non-current assets	9,452	2,432
Total assets	\$ 2,197,108	\$ 2,319,041
Liabilities and Shareholders' Equity		
Other liabilities	\$ 396,278	\$ 422,377
Accrued restructuring expense	23,867	28,353
Deferred revenues	72,725	70,969
Construction financing lease obligation	473,360	440,937
Shareholders' equity	1,230,878	1,356,405

Total liabilities and shareholders' equity	\$ 2,197,108	\$ 2,319,041
Common shares outstanding	236,201	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 first quarter of 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 consolidated the financial statements of its collaborator Alios for the three months ended March 31, 2013. The company determined that it would no longer consolidate Alios as of December 31, 2013. Each reporting period that Vertex consolidated Alios, the company estimated 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 resulted 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: Stock compensation expense in the three months ended March 31, 2014 includes the impact of the company's full career provision, which was effective for equity grants issued in February 2014, and results in partial or full acceleration of stock compensation expense for qualified grants.

Note 4: In the three months ended March 31, 2014, "HCV Related Costs" primarily consists of (i) \$3.9 million in net product revenues related to INCIVEK, (ii) \$4.9 million in royalty revenues related to INCIVO and a corresponding amount of royalty expenses, (iii) net charges of \$7.6 million related to post-restructuring HCV collaborative revenues and development costs, and (iv) \$5.9 million related to the 2014 pharma fee and commercial costs related to INCIVEK.

Note 5: In the three months ended March 31, 2014, "Other Adjustments" primarily consists of \$3.3 million of development costs associated with VX-509. "Other Adjustments" in the three months ended March 31, 2013 were not material.

Note 6: Shares used in non-GAAP net income (loss) per diluted share attributable to Vertex common shareholders were 232,887,000 and 218,317,000 for the three months ended March 31, 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 only, 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.

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 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 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 plan to submit an sNDA in the U.S. and an MAA variation in Europe for people with CF ages 18 and older who have the R117H mutation; (ii) expectations regarding the timing of data from ongoing studies of ivacaftor and ivacaftor in combination with lumacaftor, and the timing of potential regulatory submissions based on the data from these studies; and (iii) Vertex's plans to out-license VX-135. 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 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 may 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 until June 30, 2014.

(VRTX-GEN)

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News Provided by Acquire Media