



April 26, 2012

Vertex Reports First Quarter 2012 Financial Results and Provides Update on Launch of KALYDECOTM

-Approximately 600 people with cystic fibrosis have started treatment with KALYDECO since approval on January 31-

-Multiple ongoing clinical trials to generate data beginning in second quarter, including study of KALYDECO combined with VX-809 in cystic fibrosis and first data for Alios nucleotides in hepatitis C-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended March 31, 2012.

Vertex reported total first quarter 2012 revenues of approximately \$439 million, including net product revenues of approximately \$357 million from INCIVEK[®] (telaprevir) and approximately \$18 million from KALYDECO[™] (ivacaftor). Royalty revenues related to the sale of INCIVO[®] in Europe by our collaborator were approximately \$33 million. The company reported GAAP net income of approximately \$92 million and non-GAAP net income of approximately \$119 million, or \$0.43 and \$0.55 per diluted share, respectively. Additionally, Vertex today announced that it expects to obtain data from multiple ongoing clinical trials beginning in the second quarter, including data from a Phase 2 study evaluating KALYDECO in combination with VX-809 in people with the most common type of cystic fibrosis (CF) and the first viral kinetic data of the nucleotide hepatitis C virus (HCV) polymerase inhibitors ALS-2220 and ALS-2158.

"Since January, approximately 600 people with cystic fibrosis have started treatment with KALYDECO in the U.S., underscoring the importance of this new medicine to the CF community and further demonstrating our ability to develop and launch transformative new medicines," said Jeffrey Leiden, M.D., Ph.D., President and Chief Executive Officer of Vertex. "With INCIVEK, we continue to treat thousands of people with hepatitis C, and we are highly encouraged by the early success seen with this medicine outside the U.S., where our collaborator Janssen has attained a market-leading position in Europe and availability of INCIVO in more than 15 countries around the world.

"The success of our first two approved medicines provides for continued investment in our pipeline, where multiple ongoing clinical studies will begin to generate important new data beginning this quarter. We're also preparing to initiate Phase 2b studies of all-oral, short-duration treatment regimens for hepatitis C and of our JAK3 inhibitor VX-509 for rheumatoid arthritis, as well as pivotal studies of KALYDECO in people with types of cystic fibrosis not studied in earlier trials," concluded Dr. Leiden.

Cystic Fibrosis

- **KALYDECO Launch:** Vertex today announced that approximately 600 people have started treatment with KALYDECO following the FDA approval on January 31. KALYDECO is approved in the U.S. for people with cystic fibrosis ages 6 and older who have at least one copy of the G551D mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.
- **European Marketing Authorization Application:** In December, the European Medicines Agency (EMA) validated Vertex's marketing authorization application (MAA) for KALYDECO. The acceptance of the MAA marked the start of the regulatory review process by the Committee for Medicinal Products for Human Use (CHMP). Vertex expects to obtain approval of KALYDECO in Europe in the third quarter of 2012.
- **Studies of KALYDECO in other *CFTR* Mutations:** In 2012, Vertex plans to begin three additional pivotal studies of KALYDECO that will enroll people with CF who have certain *CFTR* mutations that were not evaluated in the previous Phase 3 studies, as well as children with CF as young as two years of age. The first two of these studies are expected to begin in the middle of the year and will include a study of people with CF who have at least one copy of the R117H *CFTR* mutation, which is present in approximately three percent of people with CF in the U.S., and a study in people with CF who have other *CFTR* gating mutations where *CFTR* proteins are present at the cell surface but do not function properly. G551D is the most common gating mutation, present in approximately four percent of people with CF. The remaining gating mutations to be evaluated in the second study account for an additional approximately one percent of people with CF in the U.S. Pending final feedback from regulatory agencies, a third study is planned for later this year in children with CF as young as two years of age who have gating mutations.

- **Combination Study in Most Common Type of CF:** Vertex is also conducting a Phase 2 study of the CFTR corrector VX-809 dosed in combination with KALYDECO in people with the most common *CFTR* mutation, known as F508del. Final data from this study are expected in mid-2012. The study enrolled people with two copies of the F508del mutation (homozygotes) and also included the first evaluation of KALYDECO combined with VX-809 in people with one copy of the F508del mutation and one copy of certain non-gating mutations (heterozygotes). A Phase 2 study of VX-661, a second CFTR corrector, dosed in combination with KALYDECO is also ongoing, with data expected in the second half of 2012.

Hepatitis C

- **Global Availability of Telaprevir:** Telaprevir (INCIVEK, INCIVO, TELAVIC) is now available in countries in North America, Europe, South America and the Far East. Vertex's collaborator, Janssen, is marketing telaprevir in more than 15 countries in Europe and other regions as INCIVO. Vertex's collaborator Mitsubishi Tanabe Pharma markets this medicine in Japan as TELAVIC®.
- **Viral Kinetic Studies of Two Alios Nucleotides:** Seven-day viral kinetic studies of the nucleotide analogues ALS-2200 and ALS-2158 are ongoing in people with hepatitis C. The first data from these studies are expected in the second quarter of 2012. If successful, Vertex plans to begin Phase 2 studies in the second half of 2012 to evaluate combination regimens of ALS-2200 or ALS-2158 with INCIVEK or VX-222 with or without ribavirin, as well as other potential interferon-free combination regimens.
- **Phase 2b Study of 12-week All-Oral Regimen of INCIVEK, VX-222 and Ribavirin:** In the second quarter of 2012, Vertex plans to begin an approximately 100-person Phase 2b study of an all-oral treatment regimen of INCIVEK, VX-222 and ribavirin in people with genotype 1 hepatitis C. The study will evaluate treatment regimens as short as 12 weeks. If successful, data from this study will be used to design a Phase 3 program with the goal of submitting a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for Vertex's first interferon-free regimen for genotype 1 (1a and 1b) patients as early as the end of 2014, pending regulatory discussions.

Rheumatoid Arthritis

- **Initiation of Phase 2b Study:** In May, Vertex plans to initiate a global Phase 2b study of the JAK3 inhibitor VX-509 in people with moderate to severe rheumatoid arthritis (RA) that will evaluate once and twice-daily dosing in combination with methotrexate. The six-month study is expected to enroll approximately 350 people.

Flu

- **Ongoing Phase 2 Study of VX-787:** Vertex recently initiated a Phase 2, randomized, double-blind, placebo-controlled study of VX-787 that is expected to enroll approximately 140 healthy volunteers who will be infected with live influenza virus as part of this study. The primary efficacy endpoint of the study is the reduction in viral shedding. Data from the study are expected in the second half of 2012. VX-787 is an investigational medicine that is designed to treat influenza A, including recent H1 (pandemic) and H5 (avian) influenza strains.

First Quarter 2012 Financial Results

"With the launch of KALYDECO earlier this year and the global availability of INCIVEK and INCIVO, Vertex has begun to generate significant revenues from approved medicines worldwide," said Ian Smith, Executive Vice President and Chief Financial Officer for Vertex. "Our total first quarter revenues of approximately \$439 million support reinvestment in our pipeline, which may lead to future medicines for people with diseases such as hepatitis C, cystic fibrosis, influenza and rheumatoid arthritis, while generating significant earnings, operating margins and cashflow for our business."

Total Revenues: Total revenues for the first quarter of 2012 were \$438.7 million, compared with \$73.7 million in total revenues for the first quarter of 2011. Key components of total revenues for the first quarter 2012 were:

- **Net Product Revenues from INCIVEK:** Net product revenues from INCIVEK for the first quarter of 2012 were \$356.9 million.
- **Net Product Revenues from KALYDECO:** Net product revenues from KALYDECO, which was approved on January 31, 2012, were \$18.4 million in the first quarter of 2012.
- **Royalty Revenues:** Vertex recognized \$39.0 million in royalty revenues in the first quarter of 2012, including \$32.9 million in INCIVO royalty revenues from our collaborator Johnson and Johnson.
- **Collaborative Revenues:** Vertex recognized \$24.4 million in collaborative revenues in the first quarter of 2012.

Research and Development (R&D) Expenses: R&D expenses for the first quarter of 2012 were \$196.4 million, including \$17.2 million of Vertex stock-based compensation expense and \$4.0 million in Alios expenses related to the accounting for the

collaboration with Vertex, compared to \$158.6 million for the first quarter of 2011, including \$18.5 million of stock-based compensation expense. The increase in Vertex's R&D investment is principally due to development activities related to ongoing and planned clinical trials in influenza, RA, hepatitis C and CF.

Sales, general and administrative (SG&A) expenses: SG&A expenses for the first quarter of 2012 were \$111.1 million, including \$10.5 million of Vertex stock-based compensation expense and \$1.1 million in Alios expenses related to the accounting for the collaboration with Vertex, compared to \$71.5 million for the first quarter of 2011, including \$9.3 million of stock-based compensation expense. This increase reflects the expansion of the company's commercial organization and costs related to the commercial launch of KALYDECO in the U.S. and launch preparation activities for KALYDECO in Europe.

GAAP Net Income (Loss) Attributable to Vertex: Vertex's GAAP net income for the first quarter of 2012 was \$91.6 million, or \$0.43 per diluted share. The GAAP net loss for the first quarter of 2011 was (\$176.1) million, or (\$0.87) per diluted share.

Non-GAAP Net Income (Loss) Attributable to Vertex: Vertex's non-GAAP net income for the first quarter of 2012, was \$118.6 million, or \$0.55 per diluted share. The non-GAAP net loss for the first quarter of 2011 was (\$183.9) million, or (\$0.91) per diluted share.

2012 Financial Guidance

Vertex today reiterated its financial guidance, as provided on February 2, 2012.

Non-GAAP Financial Measures

In this press release, Vertex's financial results and financial guidance are provided both 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 2012 net income and first quarter 2011 net loss excluding stock-based compensation expense, restructuring expense, any revenues and expenses related to certain September 2009 financial transactions, and any items related to 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 other non-GAAP financial results to GAAP financial results is included in the attached financial statements.

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

	Three Months Ended March 31,	
	2012	2011
Revenues:		
Product revenues, net	\$375,375	\$---
Royalty revenues	38,981	6,061
Collaborative revenues	24,381	67,601
Total revenues	<u>438,737</u>	<u>73,662</u>
Costs and expenses:		
Cost of product revenues	25,918	---
Royalty expenses	13,293	2,666
Research and development expenses (R&D)	196,371	158,612
Sales, general & administrative expenses (SG&A)	111,146	71,523
Restructuring expense	360	760
Total costs and expenses	<u>347,088</u>	<u>233,561</u>
Income (loss) from operations	<u>91,649</u>	<u>(159,899)</u>

Net interest expense (Note 2)	(3,741)	(10,599)
Change in fair value of derivative instruments (Note 2)	---	(5,598)
Income (loss) before provision for income taxes	87,908	(176,096)
Provision for income taxes	32	---
Net income (loss)	87,876	(176,096)
Net loss attributable to noncontrolling interest (Note 1)	(3,714)	---
Net income (loss) attributable to Vertex	<u>\$91,590</u>	<u>\$(176,096)</u>

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

Basic	\$0.44	\$(0.87)
Diluted	\$0.43	\$(0.87)

Shares used in per share calculations:

Basic	208,018	202,329
Diluted	219,264	202,329

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

Three Months Ended March 31, 2012

	GAAP	Adjustments				Non-GAAP
		Alios Transaction	Stock-based Compensation Expense	September 2009 Financial Transactions	Restructuring Expense	
Revenues	\$438,737	\$ —	\$ —	\$ —	\$ —	\$438,737
Operating costs and expenses	347,088	(5,086)	(27,627)	—	(360)	314,015
Income from operations	91,649	5,086	27,627	—	360	124,722
Other income and expenses	(3,741)	(62)	—	—	—	(3,803)
Income before provision for income taxes	87,908	5,024	27,627	—	360	120,919
Provision for income taxes	32	2,280	—	—	—	2,312
Net income	87,876	2,744	27,627	—	360	118,607
Net loss attributable to noncontrolling interest (Alios)	(3,714)	3,714	—	—	—	—
Net income 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 per share attributable to Vertex common shareholders:						
Basic	\$ 0.44					\$ 0.56
Diluted	\$ 0.43					\$ 0.55
Shares used in per share calculations:						
Basic	208,018					208,018
Diluted	219,264					219,264

Three Months Ended March 31, 2011

	GAAP	Adjustments				Non-GAAP
		Alios Transaction	Stock-based Compensation Expense	September 2009 Financial Transactions	Restructuring Expense	
Revenues	\$ 73,662	\$ —	\$ —	\$ (50,000)	\$ —	\$ 23,662
Operating costs and expenses	233,561	—	(27,879)	—	(760)	204,922
Loss from operations	(159,899)	—	27,879	(50,000)	760	(181,260)
Other income and expenses	(16,197)	—	—	13,532	—	(2,665)
Net loss attributable to Vertex	<u>\$(176,096)</u>	<u>\$ —</u>	<u>\$ 27,879</u>	<u>\$ (36,468)</u>	<u>\$ 760</u>	<u>\$(183,925)</u>
Net loss per share attributable to Vertex common shareholders:						
Basic	\$ (0.87)					\$ (0.91)

Diluted	\$ (0.87)	\$ (0.91)
Shares used in per share calculations:		
Basic	202,329	202,329
Diluted	202,329	202,329

Condensed Consolidated Balance Sheets Data

(in thousands)
(unaudited)

	March 31, 2012	December 31, 2011
Assets		
Cash, cash equivalents and marketable securities	\$980,867	\$968,922
Restricted cash and cash equivalents (Alios) (Note 1)	58,017	51,878
Accounts receivable, net	232,228	183,135
Inventories	129,595	112,430
Other current assets	35,407	14,889
Property and equipment, net	170,331	133,176
Restricted cash	34,090	34,090
Intangible assets (Note 3)	663,500	663,500
Goodwill (Note 3)	30,992	30,992
Other non-current assets	10,816	11,268
Total assets	<u>\$2,345,843</u>	<u>\$2,204,280</u>
Liabilities and Shareholders' Equity		
Other liabilities	\$426,687	\$405,616
Accrued restructuring expense	25,473	26,313
Deferred tax liability (Note 3)	241,426	243,707
Deferred revenues	146,680	163,132
Convertible notes (due 2015)	400,000	400,000
Noncontrolling interest (Alios) (Note 1)	175,079	178,669
Shareholders' equity (Vertex)	930,498	786,843
Total liabilities and shareholders' equity	<u>\$2,345,843</u>	<u>\$2,204,280</u>
Common shares outstanding	210,863	209,304

Note 1: The company has consolidated the financial statements of its collaborator Alios BioPharma, Inc., as of March 31, 2012 and December 31, 2011, and for the three months ended March 31, 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. Increases (decreases) in the fair value of contingent milestone and royalty payments result in gains (losses) attributable to the noncontrolling interest (Alios), which decrease (increase) net income attributable to Vertex on the Consolidated Statements of Operations Data.

Note 2: In the first quarter of 2011, a portion of the collaborative revenues, the change in fair value of derivative instruments and a portion of the net interest expense reflected in the Consolidated Statements of Operations Data relate to two financial transactions that the company entered into in September 2009 relating to milestone payments under the company's collaboration agreement with Janssen Pharmaceutica, N.V. In the first quarter of 2011, the company earned \$50.0 million in milestone payments from its collaborator, Janssen, which are reflected in total collaborative revenues in the Consolidated Statements of Operations Data.

Note 3: The intangible assets, the goodwill and the deferred tax liability reflected in the Condensed Consolidated Balance Sheets Data relate to the company's acquisition of ViroChem Pharma Inc. in 2009 and the company's collaboration agreement with Alios in June 2011.

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, epilepsy and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, MA, 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 *Science* magazine named Vertex number one on its 2011 list of Top Employers in the life sciences.

Vertex's press releases are available at www.vrtx.com.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO

KALYDECO™ is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients ages 6 years and older who have a certain mutation in their *CFTR* gene called the G551D mutation.

KALYDECO 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.

It is not known if KALYDECO is safe and effective in children under 6 years of age.

KALYDECO should not be used with certain medicines, including the antibiotics rifampin and rifabutin; seizure medications (phenobarbital, carbamazepine, or phenytoin); and the herbal supplement St. John's Wort.

KALYDECO can cause serious side effects. High liver enzymes in the blood have occurred in patients taking KALYDECO. Regular assessment is recommended.

The most common side effects associated with KALYDECO include headache; upper respiratory tract infection (common cold) including sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; nausea; and dizziness.

These are not all the possible side effects of KALYDECO. Patients should tell their healthcare providers about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information for KALYDECO at www.KALYDECO.com.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR INCIVEK

Indication

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 should always be taken in combination with peginterferon alfa and ribavirin. 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. Patients must use two forms of effective birth control during treatment and for the 6 months after treatment with these medicines. Hormonal forms of birth control, including birth control pills, vaginal rings, implants or injections, 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.

INCIVEK can cause serious side effects including skin reactions, rash and anemia that can be severe. The most common side effects of INCIVEK include itching, nausea, diarrhea, vomiting, anal or rectal problems, 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 providers about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information for INCIVEK including the Medication Guide, available at www.INCIVEK.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 Dr. Leiden's statements in the third and fourth paragraphs of this press release, Mr. Smith's statements in the paragraph following the caption "First Quarter 2012 Financial Results," the information provided under the caption "2012 Financial Guidance" and statements regarding (i) multiple ongoing clinical trials generating data beginning in the second quarter of 2012; (ii) the expected timing of data from (A) studies of VX-809 and VX-661, in each case dosed in combination with KALYDECO, (B) studies of ALS-2200 and ALS-2158 and (C) studies of VX-787; (iii) the plans to conduct additional studies of KALYDECO, a Phase 2b study of VX-222, INCIVEK and ribavirin, Phase 2 studies of ALS-2200 or ALS-2158 and a global Phase 2b study of VX-509; and (iv) the expected timing of an approval of KALYDECO in Europe. 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 outcomes for each of Vertex's ongoing and planned clinical trials and studies may not be favorable, that the company's 2012 financial guidance may be incorrect (including because one or more of the company's assumptions underlying its revenue or expense expectations may not be realized) 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 Information

Vertex will host a conference call and webcast today, April 26, 2012 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 'Events & Presentations' page of Vertex's website at www.vrtx.com.

To listen to the live call on the telephone, dial 1-877-250-8889 (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 63676268.

The call will be available for replay via telephone commencing April 26, 2012 at 8:00 p.m. ET running through 5:00 p.m. ET on May 10, 2012. 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 3, 2012. 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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