



May 3, 2011

Vertex Reports First Quarter 2011 Financial Results and Reviews Milestones for Key Development Programs

-Hepatitis C: FDA decision on NDA for INCIVEK™ (telaprevir) expected this month-

-Cystic Fibrosis: Phase 3 program for VX-770 supports applications for approval in U.S. and E.U., with NDA and MAA planned for second half of 2011-

-Financial: Vertex enters second quarter with more than \$820 million in cash, cash equivalents and marketable securities-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today provided an update on recent progress in its key development programs, discussed upcoming milestones and reported consolidated financial results for the quarter ended March 31, 2011.

"Our progress in recent months with both INCIVEK for people with hepatitis C and VX-770 for people with cystic fibrosis marks a significant step toward Vertex becoming a company capable of discovering, developing and launching innovative new medicines for serious diseases," said Matthew Emmens, Chairman, President and Chief Executive Officer of Vertex.

"We are pleased with the outcome of our recent FDA advisory committee meeting and look forward to a decision on our New Drug Application for INCIVEK later this month. In cystic fibrosis, we recently announced results from our registration program for VX-770, which we expect to form the basis for global regulatory submissions for approval in the second half of the year.

"We have many important milestones still ahead in 2011, and we believe our financial position will continue to support our business as we prepare for the launch of INCIVEK and advance toward becoming a cash flow- and earnings-positive company in 2012," concluded Mr. Emmens.

Recent Clinical Development Progress

Vertex today reviewed recent progress in its clinical development programs and provided the following updates:

Hepatitis C:

Preparing for Launch of INCIVEK™ (telaprevir)

- Vertex today announced that it intends to use the name INCIVEK (in-SEE-veck) as the trade name for telaprevir. If approved, telaprevir will be marketed by Vertex as INCIVEK in the U.S.
- Vertex recently completed its FDA Antiviral Drugs Advisory Committee meeting for INCIVEK (telaprevir). At the conclusion of the meeting, the committee voted unanimously (18-0) to recommend FDA approval of INCIVEK (telaprevir) for people with genotype 1 chronic hepatitis C who were not treated previously and those who were treated previously but not cured with currently available medicines. Vertex expects the FDA to provide its formal decision on the New Drug Application for INCIVEK (telaprevir) by May 23.
- Vertex's collaborator, Janssen-Cilag International NV, is awaiting a formal decision from the European Medicines Agency (EMA) on its Marketing Authorisation Application (MAA) for telaprevir in the EU. The EMA accepted telaprevir for accelerated assessment, which is granted to new medicines of major public health interest. Vertex believes that Tibotec, a division of Janssen-Cilag, may receive a response on the MAA in the second half of 2011.
- Vertex's entire commercial function is in place and prepared for the planned launch of INCIVEK (telaprevir). Approximately 200 field-based employees have been hired to date to support the future use of INCIVEK (telaprevir) across the United States following the planned launch, including a sales team of 115 therapeutic specialists and others who will support the future sale of INCIVEK (telaprevir).

Phase 3b Study of Twice-daily Dosing of INCIVEK (telaprevir)

- Patient enrollment is ongoing in a Phase 3b clinical trial to evaluate twice-daily dosing of INCIVEK (telaprevir; 1,125 mg; BID) compared to three-times-daily dosing of INCIVEK (telaprevir; 750 mg; q8h) in combination with Pegasys[®] (pegylated-interferon alfa-2a) and Copegus[®] (ribavirin) for people with chronic genotype 1 hepatitis C. The study, known as OPTIMIZE, does not include a control arm of pegylated-interferon and ribavirin alone. Sustained viral response (SVR or viral cure) data from OPTIMIZE are expected as early as the second half of 2012, which could support the submission of a supplemental NDA for twice-daily (BID) dosing of INCIVEK (telaprevir) by the end of 2012.

Phase 2 Combination Study of INCIVEK (telaprevir) and VX-222

- Vertex is conducting a Phase 2 clinical trial evaluating multiple 12- and 24-week response-guided regimens of INCIVEK (telaprevir) dosed in combination with Vertex's lead investigational polymerase inhibitor, VX-222, for the treatment of hepatitis C. The study currently includes three treatment arms. Two of the treatment arms are fully enrolled and are evaluating four-drug combinations of INCIVEK (telaprevir; 1,125 mg; BID), VX-222 (400 mg or 100 mg; BID), pegylated-interferon and ribavirin. Vertex expects to complete enrollment in the second quarter of 2011 in a three-drug treatment arm that will evaluate an all-oral, interferon-free regimen of INCIVEK (telaprevir; 1,125 mg), VX-222 (400 mg) and ribavirin dosed twice daily. A final arm may be added to the trial per protocol based on data from other arms of the study. In April, Vertex announced interim results from this study and expects to present additional data from the study in the second half of 2011.

Phase 3 Study of INCIVEK (telaprevir) in People Co-infected with the Hepatitis C Virus (HCV) and Human Immunodeficiency Virus (HIV)

- In February, Vertex reported interim results from a Phase 2 clinical trial of INCIVEK (telaprevir) dosed in combination with pegylated-interferon and ribavirin in people who are infected with genotype 1 chronic hepatitis C and HIV. Based on these results, Vertex plans to initiate in the second half of 2011 a Phase 3 study of INCIVEK (telaprevir) dosed in combination with pegylated-interferon and ribavirin in people co-infected with HCV and HIV. The Phase 3 trial will be designed to generate data that, if positive, could support the submission of a supplemental NDA for this population.

Phase 2 Study of INCIVEK (telaprevir) to Evaluate 3-month Treatment Regimens

- In the third quarter of 2011, Vertex plans to initiate a clinical trial to evaluate a 12-week treatment regimen of INCIVEK (telaprevir) dosed in combination with pegylated-interferon and ribavirin for people who have a specific genetic marker, known as *CC*, near the *IL28B* gene. In April, Vertex announced data from retrospective analyses that evaluated the relationship between variations at the *IL28B* gene and a patient's response to treatment with INCIVEK (telaprevir), pegylated-interferon and ribavirin. These data support the initiation of the study to evaluate 12-week treatment regimens for certain patients.

Phase 2 Post-Transplant Study of INCIVEK (telaprevir)

- Earlier in 2011, Vertex completed a drug-drug interaction study of INCIVEK (telaprevir) dosed with immunosuppressive agents that are commonly used following a liver transplant. In the fourth quarter of 2011, Vertex plans to initiate a Phase 2 study in the U.S. of INCIVEK dosed in combination with pegylated-interferon and ribavirin in people with recurrent hepatitis C following a liver transplant.

Cystic Fibrosis:

VX-770 NDA and MAA Submissions Planned for Second Half of 2011

- The ongoing Phase 3 program for VX-770, Vertex's cystic fibrosis transmembrane conductance regulator protein (CFTR) potentiator, is nearing completion. The Phase 3 STRIVE trial in people with CF aged 12 and older with at least one copy of the G551D mutation is complete, as is the Phase 2 DISCOVER trial, which was primarily a safety study that enrolled people aged 12 and older with two copies of the F508del mutation. Vertex reported 48-week top-line data from STRIVE and 16-week top-line data from DISCOVER in February 2011. In March 2011, Vertex also reported 24-week top-line data from the Phase 3 ENVISION trial in children with CF aged six to 11 with at least one copy of the G551D mutation. 48-week data from ENVISION are expected in mid-2011. Vertex is on track to submit global regulatory applications for approval in the United States, Canada and Europe, including an NDA and MAA in the second half of 2011.
- Vertex expects to present clinical data from the Phase 3 registration program for VX-770 at the 34th European Cystic Fibrosis Conference, which will take place in Hamburg, Germany from June 8 to 11, 2011. The presentations are expected to include results from the Phase 3 STRIVE and Phase 2 DISCOVER trials as well as data related to in vitro studies of VX-770.

Phase 2 Trial Combining Two CFTR Modulators for the Treatment of People with the Most Common Mutation of Cystic

Fibrosis

- Vertex is conducting an exploratory Phase 2a clinical trial to evaluate combination regimens of its lead CFTR modulators - VX-770 and VX-809, a CFTR corrector - in people with the most common mutation of CF, known as F508del. Part One of the trial is ongoing and is evaluating VX-809 (200 mg), or placebo, dosed alone for 14 days and in combination with VX-770 (150 mg or 250 mg), or placebo, for 7 days. Vertex expects to obtain interim data from Part One of the trial in the second quarter of 2011.

Additional CFTR Corrector Enters Clinical Development

- In April 2011, Vertex announced a new collaboration with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) to support development activities for VX-661, Vertex's second corrector to enter clinical development, and the accelerated discovery and development of next-generation correctors. CFFT is the non-profit drug discovery and development affiliate of the Cystic Fibrosis Foundation (CFF). Vertex intends to begin a Phase 2 study of VX-661 by the end of 2011 and expects the study to enroll people with CF who have the F508del mutation.

Epilepsy:

Phase 2b Trial Planned for Second Half of 2011

- In March, Vertex announced results from a Phase 2a trial of VX-765 in treatment-resistant epilepsy. Based on these results, Vertex plans to initiate a larger and longer-duration Phase 2b study of VX-765 in people with treatment-resistant epilepsy as early as the fourth quarter of 2011.

Rheumatoid Arthritis:

Ongoing Phase 2 Study of JAK3 Inhibitor VX-509

- Vertex recently completed enrollment in an ongoing Phase 2 proof-of-concept clinical trial of the JAK3 inhibitor VX-509 in people with moderate to severe rheumatoid arthritis. The trial is evaluating 12 weeks of treatment with VX-509 dosed twice daily compared to placebo. In the third quarter of 2011, Vertex expects to obtain clinical data, including measurements of safety, tolerability and clinical efficacy, as measured by American College of Rheumatology (ACR) and Disease Activity Score (DAS) response criteria.

First Quarter Results

For the quarter ended March 31, 2011, the Company's GAAP net loss was \$176.1 million, or \$0.87 per share, compared to a GAAP net loss for the quarter ended March 31, 2010 of \$165.3 million, or \$0.83 per share.

The non-GAAP loss for the quarter ended March 31, 2011 was \$183.9 million, or \$0.91 per share, compared to \$140.1 million, or \$0.70 per share, for the quarter ended March 31, 2010. The increase in the company's 2011 non-GAAP loss was principally attributable to costs to support the planned launch of INCIVEK (telaprevir), including an increase in sales, general and administrative expenses (SG&A). The non-GAAP loss in each period excludes stock-based compensation expense, restructuring expense and expenses related to the September 2009 financial transactions. The non-GAAP loss for the first quarter of 2011 also excludes revenues from a \$50.0 million milestone payment we received from Janssen upon the acceptance of the MAA for telaprevir in Europe. We used that milestone to redeem a portion of the 2012 secured notes issued in the September 2009 financial transactions, in accordance with the note terms.

Total revenues for the quarter ended March 31, 2011 were \$73.7 million, including the \$50.0 million Janssen milestone payment, compared with \$22.4 million for the first quarter of 2010.

Research and development (R&D) expenses for the quarter ended March 31, 2011 were \$158.6 million, compared to \$143.0 million in R&D expenses for the first quarter of 2010. These expenses reflect the company's continued investment in its research and development pipeline.

Sales, general and administrative (SG&A) expenses for the quarter ended March 31, 2011 were \$71.5 million, compared to \$35.6 million for the first quarter of 2010. This increase reflects increased investment to prepare the company for the planned launch of INCIVEK (telaprevir) in May.

Net interest expense for the quarter ended March 31, 2011 was \$10.6 million, compared to \$3.5 million for the first quarter of 2010. This increase resulted from interest expense relative to the 2015 convertible notes and the 2012 secured notes.

At March 31, 2011, Vertex had \$823.5 million in cash, cash equivalents and marketable securities.

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

Vertex is today reiterating its guidance for 2011 total operating expenses, excluding costs of revenues and stock-based compensation expense, of \$890 to \$930 million, as provided on February 3, 2011.

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 2011 and 2010 loss, excluding stock-based compensation expense, restructuring expense, and revenues and expenses related to certain September 2009 financial transactions. 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

2011 First Quarter Results
Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2011	2010
Revenues:		
Royalty revenues	\$6,061	\$6,407
Collaborative revenues	67,601	16,022
Total revenues	<u>73,662</u>	<u>22,429</u>
Costs and expenses:		
Royalty expenses	2,666	3,367
Research and development expenses (R&D)	158,612	143,012
Sales, general & administrative expenses (SG&A)	71,523	35,552
Restructuring expense	<u>760</u>	<u>780</u>
Total costs and expenses	233,561	182,711
Loss from operations	<u>(159,899)</u>	<u>(160,282)</u>
Net interest expense (Note 1)	(10,599)	(3,500)
Change in fair value of derivative instruments (Note 1)	<u>(5,598)</u>	<u>(1,489)</u>
Net loss	<u><u>\$(176,096)</u></u>	<u><u>\$(165,271)</u></u>
Basic and diluted net loss per common share	\$ (0.87)	\$ (0.83)
Basic and diluted weighted-average number of common shares outstanding	202,329	198,935
Non-GAAP Loss and Loss per Common Share Reconciliation	Three Months Ended March 31,	
	2011	2010
GAAP Net Loss	<u><u>\$(176,096)</u></u>	<u><u>\$(165,271)</u></u>

Pro Forma Adjustments:		
Milestone revenues related to September 2009 financial transactions (Note 1)	\$(50,000)	\$---
Stock-based compensation expense included in R&D	\$18,549	\$14,320
Stock-based compensation expense included in SG&A	<u>9,330</u>	<u>5,013</u>
Total stock-based compensation expense	\$27,879	\$19,333
Expenses related to September 2009 financial transactions (Note 1)	13,532	5,072
Restructuring expense	<u>760</u>	<u>780</u>
Non-GAAP Loss	<u>\$(183,925)</u>	<u>\$(140,086)</u>
Basic and diluted non-GAAP loss per common share	\$(0.91)	\$ (0.70)

Note 1: 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, and the liabilities related to milestone transactions reflected in the Condensed Consolidated Balance Sheets Data, relate to two financial transactions that the company entered into in September 2009 relating to future milestone payments under the company's collaboration agreement with Janssen Pharmaceutica, N.V. In the first quarter of 2011, the company redeemed \$50.0 million in 2012 Notes with the proceeds of a milestone payment the company received from Janssen and the company recognized the \$50.0 million as revenues. During the three months ended March 31, 2011 and 2010, the company recorded interest expense of \$7.9 million and \$3.6 million, respectively, related to its secured notes (due 2012) and an additional aggregate expense of \$5.6 million and \$1.5 million, respectively, related to the changes in estimated fair values of the rights to the \$95.0 million in potential future milestone payments and the derivative embedded in the secured notes (due 2012).

Note 2: 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.

Note 3: On January 1, 2011, the Company began capitalizing its inventory for INCIVEK (telaprevir).

Condensed Consolidated Balance Sheets Data

(in thousands)
(unaudited)

	March 31, 2011	December 31, 2010
Assets		
Cash, cash equivalents and marketable securities	\$823,452	\$1,031,411
Inventories (Note 3)	17,816	---
Other current assets	31,706	25,628
Property and equipment, net	70,877	72,333
Restricted cash	34,111	34,090
Intangible assets (Note 2)	518,700	518,700
Goodwill (Note 2)	26,102	26,102
Other non-current assets	15,723	17,182
Total assets	<u>\$1,538,487</u>	<u>\$1,725,446</u>

Liabilities and Stockholders' Equity

Other liabilities	\$162,121	\$182,142
Accrued restructuring expense	28,814	29,595
Deferred tax liability (Note 2)	160,278	160,278
Deferred revenues	218,609	234,668
Convertible notes (due 2015)	400,000	400,000

Liabilities related to milestone transactions (Note 1)	176,993	214,790
Stockholders' equity	391,672	503,973
Total liabilities and stockholders' equity	<u>\$1,538,487</u>	<u>\$1,725,446</u>
Common shares outstanding	205,458	203,523

About Vertex

Vertex creates new possibilities in medicine. Our team aims to discover, develop and commercialize 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, 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.

INCIVEKTM is a trademark of Vertex Pharmaceuticals Incorporated.

PEGASYS[®] and COPEGUS[®] are registered trademarks of Hoffmann-La Roche.

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 statements regarding (i) the expectation that the FDA will make a decision on INCIVEK by May 23, 2011; (ii) the Phase 3 program for VX-770 supporting applications for approval in U.S., Canada and E.U. and the plan to submit an NDA and an MAA in the second half of 2011; (iii) Vertex becoming a company capable of discovering, developing and launching innovative new medicines for serious diseases; (iv) Vertex's financial position continuing to support its business as it prepares for the launch of INCIVEK; (v) Vertex advancing toward becoming a cash flow and earnings positive company in 2012; (vi) Vertex's belief that Tibotec may receive a response on the MAA in the second half of 2011; (vii) Vertex's commercial function being prepared for the potential commercial launch of INCIVEK; (viii) Vertex's expectations regarding when it will obtain data from ongoing clinical trials, including the 48-week data from ENVISION, SVR data from OPTIMIZE, additional data from the combination study of INCIVEK and VX-222, interim data from the combination trial of VX-770 and VX-809 and data from the VX-509 clinical trial; (ix) the design, initiation and enrollment expectations for ongoing and planned clinical trials, including the ongoing combination trial of INCIVEK and VX-222, the possible Phase 3 study of INCIVEK dosed in combination with pegylated-interferon and ribavirin in people co-infected with HCV and HIV, the additional planned studies of INCIVEK dosed in combination with pegylated-interferon and ribavirin and the trials designed to evaluate VX-661 and VX-765; (x) the potential that ongoing and planned clinical trials of INCIVEK could support supplemental NDAs and the potential timing of such filings; (xi) the expectation that Vertex will present clinical and *in vitro* data regarding VX-770 in June; and (xii) the information provided in the paragraph following the statement "This section contains forward-looking guidance about the financial outlook for Vertex Pharmaceuticals." 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 Vertex or Janssen-Cilag could experience unforeseen delays in obtaining approval to market telaprevir, that the outcomes for each of Vertex's ongoing and planned clinical trials and studies may not be favorable, that regulatory authorities may require supplemental clinical trials in order to support the approval of INCIVEK and/or VX-770, that the company may not be able to successfully develop INCIVEK, VX-770, VX-222, VX-809, VX-661, VX-765 or VX-509, that the company's expectations regarding its 2011 operating expenses and/or its expectation that it will advance toward becoming a cash flow and earnings positive company in 2012 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 and Webcast

Vertex will host a conference call and webcast today, Tuesday, May 3, 2011 at 5:00 p.m. ET to review financial results and recent developments. This call and webcast will be broadcast via the Internet at www.vrtx.com. It is suggested that webcast participants go to the web site at least 10 minutes in advance of the call to ensure that they can access the slides. The link to the webcast is available on the Events and Presentations button on the home page.

To listen to the call on the telephone, dial 866-501-1537 (U.S. and Canada) 720-545-0001 (International). Vertex is also providing a podcast MP3 file available for download on the Vertex website at www.vrtx.com. The conference ID number is

58377696.

The call will be available for replay via telephone commencing May 3, 2011 at 8:00 p.m. ET running through 5:00 p.m. ET on May 10, 2011. The replay phone number for the U.S. and Canada is 800-642-1687. The international replay number is 706-645-9291. The conference ID number is 58377696. Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. ET on May 17, 2011.

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

(VRTX-GEN)

Vertex Pharmaceuticals Incorporated

Investors

Michael Partridge, 617-444-6108

Lora Pike, 617-444-6755

Matthew Osborne, 617-444-6057

or

Media

Zachry Barber, 617-444-6470

Source: Vertex Pharmaceuticals Incorporated

News Provided by Acquire Media