



July 28, 2011

Vertex Reports Second Quarter 2011 Financial Results and Provides Updates on Launch of INCIVEK™ (telaprevir) and Development Programs

-Quarter highlighted by approval and launch of INCIVEK™ for hepatitis C and completion of VX-770 Phase 3 program in cystic fibrosis-

-Continued progress in advancing new combinations of medicines for the future treatment of hepatitis C and cystic fibrosis-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended June 30, 2011 and provided an update on the launch of INCIVEK™ (telaprevir) tablets and its development programs.

The company reported approximately \$75 million in net product revenues for INCIVEK in the second quarter. Vertex ended the quarter with a cash position of approximately \$593 million and expects to become a cash flow and earnings positive company for 2012. The company also today provided several updates to its development-stage programs in hepatitis C, cystic fibrosis (CF), rheumatoid arthritis and influenza. The company noted that it plans to submit its New Drug Application (NDA) in the United States and Marketing Authorization Application (MAA) in the European Union for VX-770 in October 2011 for people with CF who have a specific mutation known as G551D. Vertex is also preparing to initiate multiple additional studies in CF, including two studies to evaluate combinations of Vertex's investigational CF medicines planned for later this year and three additional studies of VX-770 planned to begin in the first half of 2012.

"The second quarter of 2011 was marked by several defining events for our company, including the approval and launch of INCIVEK for the treatment of hepatitis C and the completion of the Phase 3 program for VX-770 in cystic fibrosis," said Matthew Emmens, Chairman, President and Chief Executive Officer of Vertex.

"Upon approval in May, Vertex was prepared to immediately make INCIVEK available to people with hepatitis C, and the first person was able to begin treatment just three days after approval. We are very pleased with the launch of this important new medicine."

Recent Clinical Development Progress

Hepatitis C:

Positive Opinion Issued by European Committee for Medicinal Products for Human Use (CHMP) for Telaprevir

- Vertex's collaborator, Tibotec Virco-Virology BVBA, one of the Janssen Pharmaceutical companies of Johnson & Johnson, announced last week that the CHMP adopted a positive opinion to recommend the approval of telaprevir in Europe. The positive opinion will be considered by the European Commission, which has authority to approve new medicines for use throughout the European Union. Vertex expects that Tibotec will receive a response from the European Commission on their application for approval in the third quarter of 2011. Following marketing authorization approvals, telaprevir will be marketed in the European Union and certain other global territories under the brand name INCIVO™ by the Janssen companies.

Enrollment Complete in Phase 3b Study of Twice-daily Dosing of INCIVEK

- Patient enrollment is complete in a Phase 3b clinical trial to evaluate twice-daily dosing of INCIVEK (1,125 mg; BID) compared to three-times-daily dosing of INCIVEK (750 mg; q8h) in combination with Pegasys® (pegylated-interferon alfa-2a) and Copegus® (ribavirin) for people with chronic genotype 1 hepatitis C. 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 dosing of INCIVEK by the end of 2012.

Interim Results from Phase 2 Combination Study of INCIVEK and VX-222

- In a separate press release issued earlier this week, Vertex announced results from an interim analysis of the two, four-drug (quad) arms of an ongoing Phase 2 clinical trial evaluating multiple 12- and 24-week response-guided regimens of Vertex's lead investigational hepatitis C virus polymerase inhibitor, VX-222, dosed in combination with INCIVEK. The study currently includes four treatment arms. Two of the arms are fully enrolled and are evaluating four-drug combinations of VX-222 (400 mg or 100 mg; BID), INCIVEK (1,125 mg; BID), pegylated-interferon and ribavirin. The third and fourth arms are three-drug treatment arms that are evaluating a twice-daily, all-oral, interferon-free regimen of INCIVEK (1,125 mg), VX-222 (400 mg) and ribavirin in people with genotype 1a or 1b chronic hepatitis C. Vertex expects to complete enrollment in the all-oral arms in the third quarter of 2011.
- Data from this study will be submitted for presentation at a medical meeting later this year.

Phase 2 Study of INCIVEK Combination Treatment Evaluating 12-week Regimens on Track to Begin in the Third Quarter

- Vertex plans to begin a Phase 2 trial in the third quarter that will evaluate a treatment regimen of INCIVEK dosed in combination with pegylated-interferon and ribavirin for people who have a specific genetic marker, known as CC, near the *IL28B* gene. All people in the study will receive 12 total weeks of treatment. The trial is expected to include approximately 400 people with genotype 1 chronic hepatitis C who have not previously been treated. Data from this trial are expected in 2012.

Additional information on INCIVEK, including important safety information, appears at the end of this release.

Cystic Fibrosis (CF):

Submission of VX-770 NDA and MAA on Track for October 2011

- The Phase 3 program for VX-770, Vertex's cystic fibrosis transmembrane conductance regulator protein (CFTR) potentiator, is now complete. In October, Vertex plans to submit both its VX-770 NDA to the U.S. Food and Drug Administration (FDA) and its VX-770 MAA to the European Medicines Agency (EMA). Additional regulatory submissions are planned for Canada and other countries following the submissions of the NDA and MAA. Vertex is seeking approval of VX-770 in people six years of age and older who have at least one copy of the G551D mutation in the *CFTR* gene.

Additional Studies of VX-770 Planned for 2012

- *Pediatric Study:* The Phase 3 program for VX-770 was focused on people ages 6 and older with the G551D mutation. In the first half of 2012, Vertex plans to begin the first study of VX-770 in children younger than 6 years of age. The study is expected to enroll children ages 2 through 5 and will evaluate the safety, tolerability, and effect on sweat chloride and other measures of clinical efficacy using a pediatric formulation of VX-770.
- *Studies of VX-770 in People with other CFTR Mutations:* The G551D mutation is the most common gating mutation, present in approximately 4 percent of all people with CF. In the first half of 2012, Vertex plans to begin two clinical studies of VX-770 in people with CF who have other *CFTR* mutations where, based on *in vitro* data, VX-770 may help improve the function of CFTR proteins at the cell surface. These additional studies are expected to enroll people with CF who have certain other gating mutations that result in the CFTR protein functioning abnormally at the cell surface, as well as people with other mutations that result in some residual CFTR function. A goal of the trials will be to generate data to support the evaluation of the use of sweat chloride measurements as a marker for clinical benefit in people with CF.
- Additional information on these studies will be provided upon completion of discussions with regulatory agencies in the U.S. and E.U.

Phase 2 Trials Combining Two CFTR Modulators for the Treatment of People with the Most Common Mutation of CF

- Vertex is conducting an exploratory Phase 2 clinical trial to evaluate combination regimens of VX-770 and VX-809, a CFTR corrector, in people with the most common mutation in CF, known as F508del. Vertex recently completed the first part of the trial and is on track to initiate the second part of the trial in September 2011. Part Two of this trial will include dosing of VX-809 alone for at least four weeks followed by dosing of VX-770 and VX-809 in combination for at least four weeks. Similar to Part One, the primary goals of the second part of the trial will be to evaluate the safety and tolerability and the effect of the combination of VX-770 and VX-809 on CFTR function as measured by sweat chloride. Lung function will be measured as a secondary endpoint. The trial is expected to evaluate higher doses of VX-809 than the 200 mg dose studied in the first part of the trial and to enroll people with CF who have one copy or two copies of the F508del mutation.
- Vertex also plans to initiate a Phase 2a clinical trial to evaluate combination regimens of VX-770 and VX-661, another CFTR corrector, by the end of 2011. This trial will evaluate people with two copies of the F508del mutation.

Rheumatoid Arthritis:

Data from Phase 2 Study of JAK3 Inhibitor VX-509 Expected in September

- Vertex is conducting a Phase 2 proof-of-concept clinical trial of the JAK3 inhibitor VX-509 in people with moderate to severe rheumatoid arthritis. All patients in the study have completed dosing of VX-509, and analyses of all data from the trial are ongoing. Vertex expects to announce clinical results from this trial in September, including safety and tolerability data and measurements of clinical efficacy using American College of Rheumatology (ACR) and Disease Activity Score (DAS) response criteria.

Influenza:

VX-787 to Enter Phase 1 Clinical Development in September

- Vertex recently completed a successful pre-Investigational New Drug (pre-IND) meeting with the FDA for VX-787, a medicine in development designed to treat influenza A, including recent H1 (pandemic) and H5 (avian) influenza strains. VX-787 is the first of a new class of molecules that aims to treat influenza in a way that is distinct from neuraminidase inhibitors, the current standard of care for the treatment of influenza, and from other previous approaches to the treatment of influenza. Vertex plans to begin Phase 1 development of VX-787 in healthy volunteers in September. Pending results from Phase 1 studies, VX-787 could begin to be evaluated in influenza in the first half of 2012.

Second Quarter Results

INCIVEK Revenues: For the quarter ended June 30, 2011, Vertex reported \$74.5 million in net revenues of INCIVEK. Revenue was recorded on an ex-factory basis.

Total Revenues: Total revenues for the quarter ended June 30, 2011 were \$114.4 million, compared with \$31.6 million in total revenues for the second quarter of 2010. The increase in total revenues is a result of net INCIVEK revenues in the second quarter of 2011 following approval on May 23, 2011.

Cost of Product Revenues: Cost of product revenues for the quarter ended June 30, 2011 was \$5.4 million, which principally reflects royalty expenses owed to third parties on the sale of INCIVEK. The majority of the costs related to the manufacture of INCIVEK sold in the second quarter was expensed in prior quarters.

Research and Development (R&D) Expense: R&D expense for the quarter ended June 30, 2011 was \$172.8, including \$20.5 million in stock-based compensation expense, compared to \$155.1 million, including \$17.7 million in stock-based compensation expense, for the second quarter of 2010. This expense reflects the company's continued investment in its research and development pipeline.

Sales, General and Administrative (SG&A) Expense: SG&A expense for the quarter ended June 30, 2011 was \$97.5 million, including \$11.4 million in stock-based compensation expense, compared to \$40.9 million, including \$6.7 million in stock-based compensation expense, for the second quarter of 2010. This increase reflects the expansion of our commercial organization to support both INCIVEK and VX-770 and costs related to the commercial launch of INCIVEK.

GAAP and Non-GAAP Loss Attributable to Vertex: For the quarter ended June 30, 2011, the Company's GAAP net loss attributable to Vertex was \$174.1 million, or \$0.85 per share, compared to a GAAP net loss attributable to Vertex for the quarter ended June 30, 2010 of \$200.0 million, or \$1.00 per share.

The non-GAAP loss attributable to Vertex for the quarter ended June 30, 2011 was \$136.4 million, or \$0.67 per share, compared to \$142.5 million, or \$0.71 per share, for the quarter ended June 30, 2010. The decrease in the second quarter 2011 non-GAAP loss attributable to Vertex resulted principally from increased revenues partially offset by increased operating costs and expenses. The non-GAAP loss attributable to Vertex in each period excludes stock-based compensation expense, restructuring expense and expenses related to certain September 2009 financial transactions.

Cash Position: At June 30, 2011, Vertex had \$593.5 million in cash, cash equivalents and marketable securities.

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

Vertex is today revising its guidance for 2011 total operating expenses. Vertex now expects 2011 total operating expenses, excluding cost of revenues and stock-based compensation expense, of \$960 to \$980 million. Vertex's previous guidance for 2011 total operating expenses, excluding cost of revenues and stock-based compensation expense, was \$890 to \$930 million. The revised guidance reflects increased investment to support the expansion of Vertex's clinical development program in cystic

fibrosis, including an increased investment in the company's European infrastructure to support the planned global launch of VX-770, as well as activities related to the Alios transaction.

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 second quarter 2011 and 2010 loss excluding stock-based compensation expense, restructuring expense, and any 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 Second Quarter and Six Month Results
Consolidated Statements of Operations Data
(in thousands, except per share amounts)
(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2011	2010	2011	2010
Revenues:				
Product revenues, net	\$74,535	\$ ---	\$74,535	\$ ---
Royalty revenues	10,010	7,262	16,071	13,669
Collaborative revenues	29,879	24,360	97,480	40,382
Total revenues	114,424	31,622	188,086	54,051
Costs and expenses:				
Cost of product revenues	5,404	---	5,404	---
Royalty expenses	3,902	3,086	6,568	6,453
Research and development expenses (R&D)	172,796	155,082	331,408	298,094
Sales, general & administrative expenses (SG&A)	97,471	40,915	168,994	76,467
Restructuring expense	741	2,112	1,501	2,892
Total costs and expenses	280,314	201,195	513,875	383,906
Loss from operations	(165,890)	(169,573)	(325,789)	(329,855)
Net interest expense (Note 2)	(6,760)	(3,199)	(17,359)	(6,699)
Change in fair value of derivative instruments (Note 2)	(2,220)	(27,234)	(7,818)	(28,723)
Net loss	\$(174,870)	\$(200,006)	\$(350,966)	\$(365,277)
Adjusted for: Net loss attributable to noncontrolling interest (Note 1)	(801)	---	(801)	---
Net loss attributable to Vertex	\$(174,069)	\$(200,006)	\$(350,165)	\$(365,277)
Basic and diluted net loss per common share attributable to Vertex common shareholders	\$(0.85)	\$(1.00)	\$(1.72)	\$(1.83)
Basic and diluted weighted-average number of common shares outstanding	204,413	200,397	203,377	199,670

Non-GAAP Loss and Loss per Common Share Attributable to Vertex Reconciliation	Three Months Ended June 30,		Six Months Ended June 30,	
	2011	2010	2011	2010
GAAP Net Loss Attributable to Vertex	\$(174,069)	\$(200,006)	\$(350,165)	\$(365,277)
Pro Forma Adjustments:				
Milestone revenues related to September 2009 financial transactions (Note 2)	\$---	\$---	\$(50,000)	\$---
Stock-based compensation expense included in R&D	\$20,453	\$17,735	\$39,002	\$ 32,055
Stock-based compensation expense included in SG&A	11,426	6,714	20,756	11,727
Total stock-based compensation expense	\$31,879	\$24,449	\$59,758	\$43,782
Expenses related to September 2009 financial transactions (Note 2)				
Restructuring expense	5,083	30,936	18,615	36,008
	741	2,112	1,501	2,892
Non-GAAP Loss Attributable to Vertex	<u>\$(136,366)</u>	<u>\$(142,509)</u>	<u>\$(320,291)</u>	<u>\$(282,595)</u>
Basic and diluted non-GAAP loss per common share attributable to Vertex common shareholders	\$(0.67)	\$(0.71)	\$(1.57)	\$(1.42)

Note 1: The Company has consolidated the financial statements of its collaborator Alios BioPharma, Inc., as of June 13, 2011 and for the period from June 13, 2011 through June 30, 2011. 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. Alios' operations are not material to the company's statements of operations for the periods presented.

Note 2: A portion of the collaborative revenues for the first half of 2011, 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.

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 2011. The company recorded \$250.6 million of in-process research and development as an intangible asset and \$7.4 million of goodwill related to the Alios collaboration in the second quarter of 2011.

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

	June 30, 2011	December 31, 2010
Assets		
Cash, cash equivalents and marketable securities	\$593,491	\$1,031,411
Restricted cash and cash equivalents (Alios) (Note 1)	63,098	---
Accounts receivable, net	96,016	12,529

Inventories	52,622	---
Other current assets	21,897	13,099
Property and equipment, net	84,203	72,333
Restricted cash	34,114	34,090
Intangible assets (Note 3)	769,300	518,700
Goodwill (Note 3)	33,501	26,102
Other non-current assets	14,858	17,182
Total assets	<u>\$1,763,100</u>	<u>\$1,725,446</u>

Liabilities and Shareholders' Equity

Other liabilities	\$229,206	\$182,142
Accrued restructuring expense	28,205	29,595
Deferred tax liability (Note 3)	160,372	160,278
Deferred revenues	199,054	234,668
Convertible notes (due 2015)	400,000	400,000
Liabilities related to milestone transactions (Note 2)	181,795	214,790
Noncontrolling interest (Alios) (Note 1)	256,919	---
Shareholders' equity (Vertex)	307,549	503,973
Total liabilities and shareholders' equity	<u>\$1,763,100</u>	<u>\$1,725,446</u>
Common shares outstanding	207,473	203,523

Indication and Important Safety Information

INCIVEK™ (telaprevir) tablets 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, you should not take INCIVEK combination treatment if you are pregnant or may become pregnant, or if you are a man with a sexual partner who is pregnant.

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 you cannot take with INCIVEK combination treatment. Tell your healthcare provider about all the medicines you take, including prescription and non-prescription medicines, vitamins and herbal supplements.

INCIVEK can cause serious side effects including rash and anemia. 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. Tell your healthcare provider about any side effect that bothers you or doesn't go away.

Please see full Prescribing Information for INCIVEK, including the Medication Guide, 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, 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.

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

INCIVEK™ 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) continued progress in advancing new combinations of medicines for the future treatment of hepatitis C and cystic fibrosis, (ii) the expectation that Vertex will become a cash flow and earnings positive company for 2012, (iii) the plan to submit an NDA in the United States and an MAA in the European Union for VX-770 in October 2011 and to make additional submissions in Canada and other countries in periods thereafter, (iv) the multiple additional studies in CF that the company is preparing to initiate, (v) the expectation that Tibotec will receive a response to its MAA submission for telaprevir from the European Commission in the third quarter of 2011, (vi) the expected timing of SVR data from OPTIMIZE and the possibility that this data could support the submission of a supplemental NDA for twice-daily dosing of INCIVEK by the end of 2012, (vii) the design and timing of initiation, completion of enrollment and receipt of data from planned and ongoing studies of INCIVEK, VX-770, VX-509, VX-787, all-oral combinations of VX-222 and INCIVEK, combinations of VX-770 and VX-809 and combinations of VX-770 and VX-661 and (viii) 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 Tibotec 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 VX-770, that the company may not be able to successfully develop INCIVEK, VX-770, VX-222, VX-809, VX-661, VX-765, VX-509 or VX-787, that the company's expectations regarding its 2011 operating expenses and/or its expectation that it will become 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 Information

Vertex will host a conference call and webcast today, July 28, 2011 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 1-866-501-1537 (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 80694276.

The call will be available for replay via telephone commencing July 28, 2011 at 8:00 p.m. ET running through 5:00 p.m. ET on August 5, 2011. The replay phone number for the United States and Canada is 1-800-642-1687. The international replay number is 1-706-645-9291.

Following the live webcast, an archived version will be available on Vertex's website until 5:00 p.m. ET on August 11, 2011. 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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