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Vertex Reports Third Quarter 2012 Financial Results and Recent Progress in Development Programs

-Third quarter 2012 total revenues of \$336 million, including third quarter 2012 net product revenues of approximately \$254 million for INCIVEK in hepatitis C and \$49 million for KALYDECO in cystic fibrosis-

-Cystic Fibrosis: Three ongoing Phase 3 label expansion studies for ivacaftor monotherapy; pivotal program for VX-809 and ivacaftor combination expected to begin in early 2013-

-Hepatitis C: Planned start of first all-oral Phase 2 study of VX-135 by the end of 2012; agreements with GlaxoSmithKline and Janssen Pharmaceuticals announced today provide opportunity to study VX-135 in additional all-oral regimens in Phase 2 studies-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today reported consolidated financial results for the quarter ended September 30, 2012.

Vertex reported total revenues of approximately \$336 million for the third quarter of 2012, including net product revenues of approximately \$254 million from INCIVEK[®] (telaprevir) and approximately \$49 million from KALYDECO[™] (ivacaftor). Royalty revenues related to the sale of INCIVO[®] in Europe by our collaborator were approximately \$20 million for the third quarter of 2012, and the company reported \$1.3 billion in cash, cash equivalents and marketable securities as of September 30, 2012. In the third quarter of 2012, the company reported a GAAP net loss of approximately \$(58) million, or \$(0.27) per share, and non-GAAP net income of approximately \$28 million, or \$0.13 per diluted share. Vertex today also provided updates on a number of ongoing and planned trials in cystic fibrosis, hepatitis C, rheumatoid arthritis and influenza. In separate press releases issued earlier today, Vertex announced that it has entered into two non-exclusive agreements to conduct Phase 2 proof-of-concept studies of its nucleotide analogue hepatitis C virus (HCV) polymerase inhibitor VX-135 in combination with simeprevir (TMC435), a protease inhibitor being jointly developed by Janssen R&D Ireland and Medivir AB, and with GSK2336805, an NS5A inhibitor in development by GlaxoSmithKline (GSK), for the treatment of hepatitis C.

"In the third quarter, we made significant progress across our broad pipeline of potential medicines," said Jeffrey Leiden, M.D., Ph.D., Chair, President and Chief Executive Officer of Vertex. "In hepatitis C, we are advancing rapidly with our plans to evaluate multiple all-oral regimens of VX-135, both with medicines in our own pipeline and, as we announced earlier today, in collaboration with other companies. We are also advancing toward our goal to help more people with cystic fibrosis with the recent initiation of multiple label-expansion studies for ivacaftor and the planned start of pivotal development early next year for a combination of VX-809 and ivacaftor.

"Importantly, we are advancing our business while keeping a focus on financial discipline and prioritization to allow us to continue investing in key research and development programs that may produce transformative medicines for patients in the years to come," concluded Dr. Leiden.

Cystic Fibrosis (CF)

- **Ongoing global launch of KALYDECO:** KALYDECO (ivacaftor) is approved in the U.S. and in all 27 EU countries 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. Currently, Vertex is working with individual countries in Europe to make KALYDECO available to eligible patients as soon as possible. Vertex has submitted marketing applications for KALYDECO in Canada and Australia that are currently under review with the respective regulatory agencies.
- **Efforts to help more CF patients with ivacaftor monotherapy:** There are three Phase 3 label expansion trials and one Phase 2 proof-of-concept study underway for ivacaftor monotherapy:
 - A Phase 3 study of ivacaftor is ongoing in people with CF ages 6 and older who have at least one copy of the R117H mutation. Approximately 3 percent of people with CF in the U.S. have at least one R117H mutation.
 - A Phase 3 study of ivacaftor is ongoing in people with CF ages 6 and older who have at least one non-G551D *CFTR* gating mutation. Approximately 1 percent of people with CF in the U.S. have at least one non-G551D gating mutation.
 - A Phase 3 study of ivacaftor was recently initiated in children with CF ages 2 to 5 who have a gating mutation, with enrollment targeted to begin by the end of 2012.

- A Phase 2 proof-of-concept study is underway evaluating ivacaftor in people with clinical evidence of residual CFTR function. This is the first study to evaluate the efficacy of ivacaftor based on a person's clinical symptoms and characteristics, or phenotype, rather than solely on their *CFTR* mutation, or genotype. Between 5 and 10 percent of people with CF in the U.S. may have residual CFTR function.
- **Combination therapy for people with two copies of the F508del mutation:** Based on data from the second part of an ongoing Phase 2 study and pending discussions with regulatory agencies, Vertex is preparing to start a pivotal program in early 2013 evaluating combination therapy with VX-809 and ivacaftor in people ages 12 and older with two copies of the F508del mutation, the most common form of cystic fibrosis. A third part (Cohort 3) of the Phase 2 study is evaluating the pharmacokinetics, safety and tolerability of a twice-daily (q12h) combination of VX-809 (400mg) and ivacaftor (250mg). Cohort 3 is fully enrolled, and Vertex expects to use data from this part of the study to support the pivotal program for VX-809 and ivacaftor. Additional data are expected in the first half of 2013 from a Phase 2 study of combination therapy with VX-661, another CFTR corrector, and ivacaftor.
- **Research to identify additional CF treatment regimens:** Vertex has an active and ongoing research program that has identified next-generation correctors. This research is being conducted as part of the company's collaboration with Cystic Fibrosis Foundation Therapeutics, Inc. and is focused on the accelerated discovery and development of correctors that could play a role in a variety of future combination treatments, including a dual corrector approach and other combinations with ivacaftor.

Hepatitis C

- **Development-stage agreements:** Vertex announced earlier today that it has entered into two separate, non-exclusive agreements with GSK and Janssen Pharmaceuticals, Inc. to evaluate multiple all-oral treatment regimens for people with genotype 1 hepatitis C. The collaboration with GSK will evaluate Vertex's nucleotide analogue HCV polymerase inhibitor VX-135 in combination with GSK's NS5A inhibitor GSK2336805, and the collaboration with Janssen will evaluate VX-135 in combination with Janssen's protease inhibitor simeprevir (TMC435). Additional details were provided today in separate press releases.
- **Additional all-oral studies with VX-135:** Based on positive viral kinetic data for VX-135 announced in the third quarter, Vertex plans to conduct 12-week Phase 2 all-oral studies of VX-135, including a study in combination with ribavirin and a study in combination with telaprevir, pending regulatory approval. The study in combination with ribavirin is expected to begin by the end of 2012, followed by the study with telaprevir in early 2013. Data on VX-135 will be presented at The Liver Meeting[®], the 63rd Annual Meeting of the American Association for the Study of Liver Diseases (AASLD) in November 2012.
- **Telaprevir twice-daily dosing data:** Data from a study examining twice-daily dosing (BID) for telaprevir will be presented at the upcoming AASLD annual meeting in Boston. The results demonstrated that twice-daily dosing of telaprevir in combination with peginterferon alfa and ribavirin compared to dosing telaprevir every eight hours, the currently approved dosing schedule, achieved similar sustained virological response (SVR12) rates, or viral cure rates, thereby meeting the study's primary objective of non-inferiority. Adverse events were generally similar between both arms of the study and consistent with previous studies. Vertex plans to submit data supporting this new dosing regimen to the U.S. Food and Drug Administration (FDA) in 2013 for potential inclusion in the telaprevir label in the U.S.

Pipeline Programs

- **Phase 2b study of VX-509 underway in rheumatoid arthritis:** Enrollment is ongoing in the U.S. and Europe in a Phase 2b 350-person study of VX-509, an oral, selective JAK3 inhibitor, being evaluated in combination with methotrexate for people with moderate to severe rheumatoid arthritis (RA). Vertex expects to start additional studies of VX-509 in other immune-mediated inflammatory diseases in 2013.
- **Ongoing Phase 2 study of VX-787 in influenza:** Vertex expects final data from an ongoing Phase 2 proof-of-concept study of VX-787 in experimental influenza by the end of 2012. VX-787 is an investigational medicine that is designed to treat influenza A, including recent H1N1 (pandemic) and H5N1 (avian) influenza strains.

Third Quarter 2012 Financial Results

Total Revenues: Total revenues were \$336.0 million for the third quarter of 2012, compared with \$659.2 million for the third quarter of 2011, which included one-time milestone revenue of \$200.0 million from Janssen. Key components of total revenues for the third quarter 2012 were:

- **Net Product Revenues from INCIVEK:** Net product revenues from INCIVEK were \$254.3 million, compared with \$419.6 for the third quarter of 2011.
- **Net Product Revenues from KALYDECO:** Net product revenues from KALYDECO were \$49.2 million. Because KALYDECO was approved in January 2012, there were no net product revenues from KALYDECO for the third quarter of 2011.

- **Royalty Revenues:** Vertex recognized \$25.6 million in royalty revenues, including \$20.0 million in INCIVO royalty revenues from our collaborator Janssen, compared to \$8.5 for the third quarter of 2011. INCIVO was approved in Europe in September 2011.
- **Collaborative Revenues:** Vertex recognized \$6.9 million in collaborative revenues, compared to \$231.1 million for the third quarter of 2011, which included an aggregate of \$200.0 million in milestone revenue from Janssen related to the approval and launch of INCIVO in Europe.

Cost of Product Revenues: Cost of product revenues was \$30.7 million in the third quarter of 2012, compared to \$35.3 million for the third quarter of 2011.

Research and Development (R&D) Expenses: R&D expenses were \$200.2 million in the third quarter of 2012, including \$21.3 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, compared to \$189.1 million for the third quarter of 2011, including \$20.9 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex.

Sales, general and administrative (SG&A) expenses: SG&A expenses were \$97.7 million in the third quarter of 2012, including \$10.8 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, compared to \$110.7 million for the third quarter of 2011, including \$13.2 million of Vertex stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex.

GAAP Net Income (Loss) Attributable to Vertex: Vertex's GAAP net loss was \$(57.5) million, or \$(0.27) per share, for the third quarter of 2012 compared to the GAAP net income of \$221.1 million, or \$1.02 per diluted share, for the third quarter of 2011.

Non-GAAP Net Income Attributable to Vertex: Vertex's non-GAAP net income was \$28.2 million, or \$0.13 per diluted share, for the third quarter of 2012, excluding \$28.2 million in stock-based compensation expense and restructuring expense and a \$57.6 million charge related to an increase in the fair value of expected future payments under Vertex's collaboration with Alios. The non-GAAP net income was \$151.2 million, or \$0.70 per diluted share, for the third quarter of 2011, excluding \$28.9 million in stock-based compensation expense, \$(0.4) million in restructuring expense (credit), \$188.9 million related to certain September 2009 financial transactions, a \$73.1 million intangible asset impairment charge, net of tax, and a \$17.5 million charge related to an increase in the fair value of expected future payments under Vertex's collaboration with Alios.

Cash Position: As of September 30, 2012, Vertex had \$1.3 billion in cash, cash equivalents and marketable securities compared to cash, cash equivalents and marketable securities on December 31, 2011 of \$968.9 million.

2012 Financial Guidance

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

Full-Year INCIVEK Revenues: Vertex is today reiterating its guidance for full-year 2012 INCIVEK net revenues to be in the range of \$1.1 billion to \$1.25 billion. This guidance was initially established on July 30, 2012.

Total Operating Expenses: Vertex is also reiterating its guidance for 2012 total operating expenses, excluding cost of revenues, stock-based compensation expense and Alios expenses related to the accounting for the collaboration with Vertex, to be in the range of \$1.03 billion to \$1.13 billion. This guidance was initially established 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 third quarter and first nine months of 2012 and 2011 net income (loss) excluding stock-based compensation expense, restructuring expense, inventory write-off, revenues and expenses related to certain September 2009 financial transactions, intangible asset impairment charges, net of tax and charges related to changes in the fair value of expected future payments under Vertex's collaboration with Alios. These results are provided as a complement to results provided in accordance with GAAP because management believes these non-GAAP financial measures help indicate underlying trends in the company's business, are important in comparing current results with prior period results and provide additional information regarding its financial position. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally and to manage the company's business and to evaluate its performance. A reconciliation of the non-GAAP financial results to GAAP financial results is included in the attached financial statements.

Vertex Pharmaceuticals Incorporated
Third Quarter and Nine Month Results
Consolidated Statements of Operations Data
(in thousands, except per share amounts)

noncontrolling interest (Alios), which decrease (increase) net income attributable to Vertex on the Consolidated Statements of Operations Data.

Note 2: In the three and nine months ended September 30, 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 three and nine months ended September 30, 2011, the company earned \$200.0 million and \$250.0 million, respectively in milestone revenue from its collaborator, Janssen, which are reflected in total collaborative revenues in the Condensed Consolidated Statements of Operations Data.

Note 3: In the second quarter of 2012, the company recorded within cost of product revenues a \$78.0 million lower of cost or market charge for excess and obsolete INCIVEK inventories.

Note 4: 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.

In the third quarter of 2011, the company recorded an impairment charge of \$105.8 million related to VX-759, a back-up HCV polymerase inhibitor to VX-222 that had been discovered by ViroChem Pharma Inc. The fair value of VX-759 following the impairment charge was zero. In connection with this impairment charge, the company recorded a benefit from income taxes of \$32.7 million resulting in a net effect on its income (loss) related to this impairment of \$73.1 million in the three and nine months ended September 30, 2011.

Note 5: Shares used in Non-GAAP net income (loss) per diluted share attributable to Vertex common shareholders were 217,797,000 and 219,349,000 for the three months ended September 30, 2012 and 2011, respectively, and 214,580,000 and 204,262,000 for the nine months ended September 30, 2012 and 2011, respectively.

About Vertex

Vertex creates new possibilities in medicine. Our team discovers, develops and commercializes innovative therapies so people with serious diseases can lead better lives.

Vertex scientists and our collaborators are working on new medicines to cure or significantly advance the treatment of hepatitis C, cystic fibrosis, rheumatoid arthritis and other life-threatening diseases.

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

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

INDICATION AND IMPORTANT SAFETY INFORMATION FOR KALYDECO (ivacaftor)

KALYDECO (150mg tablets) is indicated for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a G551D mutation in the *CFTR* gene.

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.

High liver enzymes (transaminases, ALT and AST) have been reported in patients receiving KALYDECO. It is recommended that ALT and AST be assessed prior to initiating KALYDECO, 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 KALYDECO dosing. Moderate transaminase elevations are common in subjects with CF. Overall, the incidence and clinical features of transaminase elevations in clinical trials was similar between subjects in the KALYDECO and placebo treatment groups. In the subset of patients with a medical history of elevated transaminases, increased ALT or AST have been reported more frequently in patients receiving KALYDECO compared to placebo.

Use of KALYDECO 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 KALYDECO, which may diminish effectiveness. Therefore, co-administration is not recommended.

The dose of KALYDECO must be adjusted when concomitantly used with potent and moderate CYP3A inhibitors.

KALYDECO can cause serious adverse reactions including abdominal pain and high liver enzymes in the blood. The most common side effects associated with KALYDECO 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 KALYDECO. A list of the adverse reactions can be found in the full product labeling for each country where KALYDECO is approved. Patients should tell their healthcare providers about any side effect that bothers them or doesn't go away.

Please see full U.S. Prescribing Information for KALYDECO at www.KALYDECO.com and the EU Summary of Product Characteristics for KALYDECO at <http://goo.gl/N3Tz4>.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR INCIVEK (telaprevir)

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, without limitation, Dr. Leiden's statements in the third and fourth paragraphs of the press release, the information provided in the two paragraphs following the statement "This section contains forward-looking guidance about the financial outlook for Vertex Pharmaceuticals" and statements regarding (i) the expectation that the pivotal program for ivacaftor and VX-809 will begin in early 2013; (ii) the expectations regarding the timing and structure of all-oral Phase 2 studies with VX-135; (iii) information regarding the company's ongoing and planned studies, including studies to evaluate ivacaftor, ivacaftor in combination with VX-809, VX-135, VX-509 and VX-787; (iv) expectations regarding the availability of data from ongoing studies, including Cohort 3 of the Phase 2 study of ivacaftor and VX-809, the ongoing Phase 2 study of VX-661 and the ongoing Phase 2 study of VX-787, (v) Vertex's CF research program and (vi) the company's plans to submit data supporting a twice-daily dosing regimen to the U.S. Food and Drug Administration in 2013 for potential inclusion in the telaprevir label in the U.S. While Vertex believes the forward-looking statements contained in this press release are accurate, there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that the company's expectations regarding its 2012 INCIVEK net revenues and/or operating expenses may be incorrect (including because one or more of the company's assumptions underlying its revenue or expense expectations may not be realized), that the outcomes of Vertex's ongoing and planned clinical studies may not be favorable, that the initiation of planned studies may be delayed or prevented, and other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

Conference Call Information

Vertex will host a conference call and webcast today, November 1, 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' 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 38809529.

The call will be available for replay via telephone commencing November 1, 2012 at 8:00 p.m. ET running through 5:00 p.m. ET on November 15, 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 November 8, 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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