



January 9, 2011

Vertex Announces Key Business Objectives To Support Planned Launch of Telaprevir in Hepatitis C and Continued Progress in Other Serious Diseases

-Hepatitis C: Submission of New Drug Application complete for telaprevir-

-Cystic Fibrosis: First Phase 3 data for VX-770 expected in first quarter 2011-

-Additional ongoing trials in HCV, CF, epilepsy and rheumatoid arthritis-

-Vertex enters 2011 with cash and cash equivalents position of more than \$1 billion-

SAN FRANCISCO, Jan 9, 2011 (BUSINESS WIRE)-- [Vertex Pharmaceuticals Incorporated](http://www.vrtx.com) (NASDAQ: VRTX) today announced its 2011 business objectives in conjunction with the 29th Annual J.P. Morgan Healthcare Conference in San Francisco. Matthew Emmens, Chairman, President and Chief Executive Officer of Vertex, will discuss these objectives as part of a live webcast presentation, which will be available on Vertex's website, www.vrtx.com, on Monday, January 10 at 9:30 a.m. PT (12:30 p.m. ET).

"2011 will be a landmark year for Vertex as we prepare for the expected launch of telaprevir in hepatitis C and advance other new therapies in development," said Mr. Emmens.

"Our commercial team is in place and prepared for the planned launch of telaprevir this year. We believe that telaprevir will dramatically change the treatment of hepatitis C and establish Vertex as a company capable of discovering, developing and launching transformative medicines to treat serious diseases.

"We are in a unique position, as just behind telaprevir is VX-770, a medicine in development that aims to treat the underlying cause of cystic fibrosis. We will soon obtain data from the Phase 3 registration program of VX-770 that may support the planned submission of a New Drug Application to the FDA in the second half of this year.

"We also expect to receive important data from multiple ongoing Phase 2 trials this year, including those evaluating new combination regimens for hepatitis C and cystic fibrosis, which may provide further development opportunities," Mr. Emmens concluded.

Hepatitis C: Preparing for Launch of Telaprevir

Submission of New Drug Application Completed in November 2010 with Request for Priority Review

- On November 22, 2010, Vertex completed the submission of its New Drug Application (NDA) for telaprevir to the United States Food and Drug Administration (FDA). A response from the FDA regarding the company's request for Priority Review of the telaprevir NDA is expected this month. The FDA's goal for completion of its review for NDA submissions granted Priority Review status is six months from the NDA submission date.
- In December 2010, Vertex's collaborator, Janssen-Cilag International NV, submitted a Marketing Authorization Application (MAA) for telaprevir to the European Medicines Agency (EMA). The EMA accepted telaprevir for accelerated assessment, which is granted to new medicines of major public health interest.
- To support the planned launch of telaprevir, Vertex has hired more than 200 new employees into its expanding commercial function. The sales and commercial leadership team is in place, and more than 100 field-based employees have been hired to date and are prepared to support the future use of telaprevir across the United States following the planned launch.

Phase 3b Study of Twice-daily Dosing of Telaprevir to Support Supplemental NDA by end of 2012

- Patient enrollment is ongoing in a Phase 3b clinical trial to evaluate twice-daily dosing of telaprevir (1,125 mg; BID) compared to three-times-daily dosing of telaprevir (750 mg; q8h) in combination with pegylated-interferon and ribavirin for people with genotype 1 hepatitis C. The study, known as OPTIMIZE, is the first Phase 3 study to evaluate twice-daily dosing of a protease inhibitor for the treatment of hepatitis C. The study 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 2012, which could potentially support the submission of a supplemental NDA for twice-daily (BID) dosing of telaprevir by the end of 2012.

Cystic Fibrosis: Phase 3 Registration Program for VX-770 Nears Completion

VX-770 NDA Submission Planned for Second Half of 2011

- Three trials of the novel cystic fibrosis transmembrane conductance regulator protein (CFTR) potentiator VX-770 are fully enrolled and ongoing as part of a global Phase 3 registration program focused on patients with the G551D mutation. The G551D mutation is present in approximately four percent of people with CF.
- The first Phase 3 data for VX-770 are expected in the first quarter of 2011 and will come from the Phase 3 STRIVE trial in people aged 12 and older with at least one copy of the G551D mutation. Data from 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, are also expected in the first quarter of 2011.
- Data from the Phase 3 ENVISION trial in people aged six to 11 with at least one copy of the G551D mutation are expected in mid-2011.
- If positive, the results from the Phase 3 program for VX-770 could support the submission of an NDA for VX-770 in the second half of 2011.

Opportunities to Further Advance Future Treatment of Hepatitis C and Cystic Fibrosis

Interim Data from Phase 2 Study of Telaprevir and VX-222 Expected in First Quarter of 2011

- Vertex is conducting a Phase 2 clinical trial evaluating multiple 12-week, response-guided regimens of telaprevir dosed in combination with its lead investigational HCV polymerase inhibitor, VX-222. The study currently includes three treatment arms. Two of the treatment arms are fully enrolled and are evaluating four-drug combinations of telaprevir (1,125 mg; BID), VX-222 (400 mg or 100 mg; BID), Pegasys[®] (pegylated-interferon alfa-2a) and Copegus[®] (ribavirin). Approximately two-thirds of the people in the four-drug treatment arms have received eight weeks or more of treatment. More than one-third of patients have received 10 weeks or more of treatment, with some people having completed all therapy. Interim data from both of the four-drug treatment arms are expected in the first quarter of 2011.
- In November 2010, Vertex announced the planned addition of a three-drug treatment arm to evaluate the potential of an all-oral, interferon-free regimen of telaprevir (1,125 mg), VX-222 (400 mg) and ribavirin dosed twice daily. Enrollment in this new treatment arm is anticipated to begin in the first quarter of 2011.

Additional Trials of Telaprevir to Advance Leadership Position in Hepatitis C

- Vertex and Tibotec also plan to conduct several additional clinical trials of telaprevir in 2011 that aim to expand the future patient population for telaprevir-based regimens. These trials include:
 - Phase 3 HCV/Human Immunodeficiency Virus Co-Infection Trial: Vertex recently completed enrollment in a Phase 2 clinical trial of telaprevir-based regimens in people who are infected with genotype 1 hepatitis C virus and the human immunodeficiency virus (HIV), also known as HCV-HIV co-infection. If positive, results from this trial could support the planned initiation of a Phase 3 study of telaprevir-based regimens in people co-infected with HCV and HIV in 2011. 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 Short-Duration Treatment Study: Also in 2011, Vertex and Tibotec plan to initiate a clinical trial to evaluate the role of telaprevir as part of hepatitis C treatment regimens involving less than six total months of therapy. One part of the trial may evaluate a telaprevir-based treatment regimen as short as 12 total weeks in duration for certain subsets of patients.
 - Phase 2 Post-Transplant Study: Vertex recently completed a drug-drug interaction study of telaprevir with immunosuppressive agents commonly used following a liver transplant. Based on results from this study, Vertex and Tibotec plan to initiate in 2011 a Phase 2 study of telaprevir-based regimens in people with recurrent hepatitis C following a liver transplant.

Combination of Two CFTR Modulators for the Treatment of People with the Most Common Mutation of Cystic Fibrosis

- Vertex is conducting a Phase 2a clinical trial to evaluate multiple combination regimens of its lead CFTR Modulators - VX-770, a CFTR potentiator, and VX-809, a CFTR corrector - in people with the most common mutation of CF, known as F508del. Enrollment is ongoing in Part One of the trial, which is designed to evaluate 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 first half of 2011.

Additional Phase 2 Trials and Continued Productivity in Research for Other Serious Diseases

Data from Phase 2 Trials in Epilepsy and Rheumatoid Arthritis Expected in 2011

- Vertex recently completed a Phase 2 trial of the novel caspase-1 inhibitor VX-765 in people with epilepsy. The double-blind, randomized, placebo-controlled trial was designed to evaluate the safety, tolerability and clinical activity of VX-765. The primary endpoints of the trial were safety and tolerability. Analyses of the data are ongoing and Vertex expects to announce top-line data from the trial in the first quarter of 2011.
- In the first quarter of 2011, Vertex expects to complete 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. 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 scores (ACR) and Disease Activity Scores (DAS).

Continued Investment in Research to Support Discovery of Future Medicines

- Vertex continues to focus its research efforts in the areas of infectious diseases, including viral infections - such as influenza - and bacterial infections, inflammatory diseases, cancer and neurological disorders, including pain. Vertex expects additional development candidates for the treatment of one or more of these diseases to emerge from research in 2011.

As of December 31, 2010, Vertex had more than \$1 billion in cash, cash equivalents and marketable securities. The company also recently entered into a \$100 million commercial line of credit from Bank of America for a term of 18 months.

Vertex anticipates a GAAP net loss for 2010, including certain charges, of approximately \$750 million. Vertex anticipates a 2010 non-GAAP loss, excluding certain charges, of approximately \$600 million.

Vertex will report full-year 2010 financial results on February 3, 2011.

Non-GAAP Financial Measures

In this press release, Vertex's financial results 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 guidance for its full-year 2010 loss, excluding stock-based compensation expense, restructuring expense and expenses related to certain September 2009 financial transactions, which results in a non-GAAP financial measure. 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 Vertex's business and are important in comparing current results with prior period results. Management also uses these non-GAAP financial measures to establish budgets and operational goals that are communicated internally and externally to manage the Vertex's business and to evaluate its performance.

Safe Harbor Statement

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including the statements made by Mr. Emmens in the second through fifth paragraphs of the press release, and statements regarding (i) Vertex's planned launch of telaprevir in 2011; (ii) the expectation that the first Phase 3 data for VX-770 will be available in the first quarter of 2011; (iii) the expectation that Vertex will obtain a response from the FDA regarding its request for Priority Review in January 2011 and the FDA's goal for completion of its review of NDA submissions granted Priority Review; (iv) the preparedness of the field-based employees to support the future use of telaprevir; (v) the timing of SVR data from OPTIMIZE and the potential Supplemental NDA for telaprevir; (vi) the status of the VX-770 registration program and the possibility that if the results from the Phase 3 program are positive the company could submit an NDA for VX-770 in the second half of 2011; (vii) expectations regarding the timing of data from the Phase 2 studies of (a) telaprevir and VX-222, (b) VX-770 and VX-809, © VX-765 and (d) VX-509; (viii) the anticipation that enrollment in the three-drug treatment arm of the VX-222/telaprevir clinical trial will begin in the first quarter of 2011; (ix) planned clinical trials of telaprevir that aim to expand the future patient population and the potential for these trials to support further trials and/or a supplemental NDA; (x) the expectation that additional development candidates will emerge in 2011; and (xi) the anticipation that Vertex's projected GAAP and non-GAAP 2010 annual loss and year-end cash, cash equivalents and marketable securities balance will be as set forth above. While the company 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 its planned clinical trials and studies may not be favorable, that regulatory authorities may require supplemental clinical trials in order to support the registration of telaprevir and/or VX-770, that planned or potential clinical trials may be delayed or may not be conducted, that the company may not be able to successfully develop telaprevir, VX-770, VX-509, VX-765 or combination therapies involving telaprevir and VX-222 or VX-770 and VX-809, that the company's expectations regarding its 2010 GAAP and non-GAAP net loss may be incorrect, 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. The company disclaims any obligation to update the information contained in this press release as new information becomes available.

Webcast

Vertex Pharmaceuticals will webcast its corporate presentation at the 29th Annual J.P. Morgan Healthcare Conference on January 10, 2011 at 9:30 a.m. PT (12:30 p.m. ET). A link to the live webcast will be available via Vertex's website, www.vrtx.com, in the Events & Presentations section. An archived webcast of the presentation will be available on Vertex's website through January 24, 2011.

About Vertex

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

For more information and to view Vertex's press releases, please visit www.vrtx.com.

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Source: Vertex Pharmaceuticals Incorporated

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