January 8, 2012

Vertex Announces Key 2012 Business Objectives as Company Prepares for Planned Global Launch of KALYDECO in Cystic Fibrosis

-More than 25,000 people have started treatment for hepatitis C with INCIVEK®, positioning Vertex for continued growth, earnings and cashflow in 2012-

-Preparations for approval and launch of KALYDECO™ ongoing; additional studies of KALYDECO planned for mid-2012-

-Nine new medicines in development for serious diseases; multiple proof-of-concept and later-stage studies planned for 2012-

SAN FRANCISCO--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (NASDAQ: VRTX) today announced its 2012 business objectives in conjunction with the 30th Annual J.P. Morgan Healthcare Conference in San Francisco. Matthew Emmens, Chairman, President and Chief Executive Officer of Vertex, and Jeffrey Leiden, M.D., Ph.D., who will become Vertex's CEO on February 1, 2012, will discuss these objectives as part of a live presentation, which will be available on Vertex's website, www.vrtx.com, on Monday, January 9 at 11:00 a.m. PT (2:00 p.m. ET).

"In 2011, our team executed a highly successful launch for INCIVEK in hepatitis C, with more than 25,000 people starting treatment since its approval in mid-2011," said Mr. Emmens. "With the strength of the launch for INCIVEK, the submission of our global approval applications for KALYDECO in cystic fibrosis and the advancement of our pipeline programs, we are positioned for significant growth, earnings and cashflow in 2012."

Dr. Leiden commented, "Entering 2012, we are focused on becoming a sustainable business with strong revenues from INCIVEK and the planned global launch of KALYDECO for cystic fibrosis. Importantly, we are pursuing opportunities to further improve treatment with our all-oral regimens in development for hepatitis C and efforts to study our cystic fibrosis medicines in a larger group of people with this devastating disease. As these and other pipeline programs advance, we will manage our business with financial discipline and focused investment to ensure the greatest benefit for patients waiting for new treatments and for our shareholders."

Preparing for Global Launch of KALYDECO™ in Cystic Fibrosis and Expanding the KALYDECO Development Program

In December, Vertex announced that the U.S. Food and Drug Administration (FDA) accepted the New Drug Application for KALYDECO (ivacaftor) and granted the company's request for six-month Priority Review. A target review date of April 18, 2012 is set under the Prescription Drug User Fee Act for the FDA's approval decision. Vertex's marketing authorization application for KALYDECO has also been validated by the European Medicines Agency, which accepted Vertex's request for accelerated assessment in Europe.

As Vertex prepares for the potential launch of KALYDECO for people with the G551D mutation, the company is also planning to begin additional studies of KALYDECO in children with CF as young as two years of age and in people with CF who have certain mutations that were not evaluated in the previous Phase 3 studies. Pending final feedback from regulatory agencies, the company plans to begin three clinical studies of KALYDECO in mid-2012:

- ***Pediatric study:*** A study of KALYDECO in children ages 2 through 5 with gating mutations, including G551D, is expected to evaluate the safety, tolerability and effect on sweat chloride and other measures of clinical activity using a pediatric formulation of KALYDECO.
- ***Study in people with the R117H mutation:*** Vertex plans to begin the first clinical study of KALYDECO in people who have at least one copy of the R117H mutation in the CF gene. The R117H mutation causes abnormal function of the CFTR protein at the cell surface. Approximately 3 percent of people with CF in the U.S. have the R117H mutation.
- ***Study in other gating mutations:*** Vertex also plans to begin the first clinical study of KALYDECO in other gating mutations where CFTR proteins are present at the cell surface but do not function properly. G551D is the most common gating mutation, present in approximately 4 percent of people with CF in the U.S., and was the focus of previous Phase 3 KALYDECO studies. The remaining gating mutations to be evaluated in this study account for an additional

approximately 1 percent of people with CF in the U.S.

Additional Studies in Hepatitis C, CF and Other Serious Diseases

Multiple additional studies of INCIVEK, KALYDECO and Vertex's pipeline medicines in development are ongoing or planned for 2012, including:

Hepatitis C

- **Phase 2 ZENITH study:** The ongoing Phase 2 ZENITH study is designed to assess the safety, tolerability and efficacy of the polymerase inhibitor VX-222 dosed in combination with INCIVEK and ribavirin, with and without pegylated-interferon, in people with genotype 1a and 1b chronic hepatitis C who were new to treatment. In the first quarter, Vertex expects to announce data, including sustained viral response rates at 4 weeks post-treatment (SVR4), from the two all-oral, interferon-free study arms (Arms E and F) in which patients received VX-222, INCIVEK and ribavirin.
- **All-oral, interferon-free studies of the nucleotide analogues ALS-2200 and ALS-2158:** Vertex and Alios are currently conducting two Phase 1 studies of the pan-genotypic hepatitis C polymerase inhibitors ALS-2200 and ALS-2158. The studies are evaluating safety and tolerability in healthy volunteers as well as 7-day viral kinetics in people with chronic genotype 1 hepatitis C. Data are expected in the second quarter of 2012, which could enable the initiation of Phase 2 proof-of-concept studies to evaluate multiple all-oral, interferon-free combination regimens in the second half of 2012. These Phase 2 studies are expected to evaluate combination regimens of ALS-2200 or ALS-2158 with INCIVEK or VX-222, potential dual nucleotide regimens (adenosine and uracil) and other interferon-free combination regimens that may also include ribavirin.
- **Phase 3 study in people co-infected with hepatitis C and HIV**: Enrollment is ongoing in a Phase 3 trial of INCIVEK combination therapy in people co-infected with genotype 1 hepatitis C virus and HIV.
- **Phase 3 study of twice-daily dosing of INCIVEK**: Enrollment is complete in a Phase 3 clinical trial to evaluate twice-daily dosing of INCIVEK (1,125 mg; BID) compared to three-times-daily dosing of INCIVEK (750 mg; q8h) as part of INCIVEK combination therapy.
- **Phase 4 study of INCIVEK combination treatment in African Americans:** Vertex plans to begin in the first quarter of 2012 a study of INCIVEK combination therapy in African Americans with hepatitis C who were not cured with a prior treatment of pegylated-interferon and ribavirin.
- **Phase 3b study of INCIVEK combination treatment for a total duration of 12 weeks:** Enrollment is ongoing in a Phase 3b trial to evaluate the potential for INCIVEK combination therapy to be shortened to 12 weeks in people with genotype 1 chronic hepatitis C who have the 'CC' variation near the IL28B gene.
- **Phase 2b and 3b studies in people with hepatitis C following a liver transplant:** Enrollment is expected to begin in the first quarter for clinical studies of INCIVEK combination treatment in people who have recurrent hepatitis C following a liver transplant.

Cystic Fibrosis

- **Two CFTR correctors in development for people with the most common CF mutation, F508del**: Enrollment is ongoing in the second part of a Phase 2 clinical trial of combination regimens of KALYDECO, a CFTR potentiator, and VX-809, a CFTR corrector, in people with the most common mutation in CF, known as F508del. In addition, Vertex plans to begin Phase 2 development of VX-661, a second CFTR corrector, in the first quarter of 2012. Data from the study with VX-809 is expected mid-year, followed by data from the study with VX-661 later in 2012.

Rheumatoid Arthritis (RA)

- ***350-patient Phase 2b study of VX-509:*** A six-month Phase 2b study of the JAK3 inhibitor VX-509 is planned to begin in the first quarter of 2012 for the treatment of moderate to severe rheumatoid arthritis. This study will evaluate once-daily (QD) and twice-daily (BID) doses of VX-509 in combination with methotrexate, a commonly prescribed disease-modifying antirheumatic drug (DMARD) for RA that is frequently used in combination with other RA medicines.

Influenza

- ***Proof-of-concept study planned for mid-2012 with VX-787****:* A Phase 1 study is ongoing for VX-787, an investigational medicine that is designed to treat influenza A, including recent H1 (pandemic) and H5 (avian) influenza strains. Following the completion of this Phase 1 study, Vertex plans to initiate a proof-of-concept study for VX-787 in the second quarter of 2012.

Epilepsy

- Enrollment is ongoing in a Phase 2 study of VX-765 in people with treatment-resistant epilepsy.

Continued Productivity in Research

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 2012.

The company will report full-year 2011 financial results and financial guidance on February 2, 2012.

Webcast

Vertex Pharmaceuticals will webcast its corporate presentation at the 30th Annual J.P. Morgan Healthcare Conference on January 9, 2012 at 11:00 a.m. PT (2:00 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 23, 2012.

About Vertex

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

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

Founded more than 20 years ago in Cambridge, MA, we now have ongoing worldwide research programs and sites in the U.S., U.K. and Canada. Today, Vertex has more than 2,000 employees around the world, and *Science* magazine named Vertex number one on its 2011 list of Top Employers in the life sciences.

IMPORTANT SAFETY INFORMATION

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**.

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 and Dr. Leiden in the second and third paragraphs of the press release, and statements regarding (i) Vertex being positioned for continued growth, earnings and cashflow in 2012; (ii) regulatory timelines for KALYDECO; (iii) Vertex's preparations for the potential launch of KALYDECO; (iv) proof-of-concept studies and later-stage studies planned for 2012; (v) planned and ongoing studies of INCIVEK, KALYDECO and the company's drug candidates and the expected timelines for initiating and announcing data from these studies and (vi) the expectation that additional development candidates will emerge from the company's research programs in 2012. 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 Vertex's planned clinical trials and studies may not be favorable, that regulatory authorities may require supplemental clinical trials in order to support the registration of KALYDECO, that planned or potential clinical trials may be delayed or may not be conducted, that the company may not be able to successfully develop its drug candidates, 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.

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