January 6, 2013

Vertex Outlines Corporate Strategy and Defines Key 2013 Business Priorities

-2013 investment focused on key development programs in cystic fibrosis, hepatitis C and autoimmune diseases-

-First two Breakthrough Therapy Designations from U.S. FDA granted to ivacaftor monotherapy and to the combination regimen of VX-809 and ivacaftor for the treatment of cystic fibrosis-

SAN FRANCISCO--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today outlined the company's strategy and defined key 2013 business priorities to support continued growth and the creation of long-term shareholder value. Vertex's Chairman, President and Chief Executive Officer, Jeffrey Leiden, M.D., Ph.D., will discuss the company's strategy and 2013 priorities as part of a live presentation at the 31st Annual J.P. Morgan Healthcare Conference in San Francisco on Monday, January 7 at 10:00 a.m. PT (1:00 p.m. ET). The presentation will be webcast on Vertex's website, www.vrtx.com.

"Since the approval of our first medicine less than two years ago, Vertex has undergone a rapid evolution that has positioned the company to move forward with a clear focus on using innovative science to develop transformative medicines for serious diseases in specialty markets," said Dr. Leiden.

"In cystic fibrosis and hepatitis C, KALYDECO and INCIVEK are just the beginning of what we hope to provide to people with these diseases. In CF, multiple ongoing and planned studies of KALYDECO monotherapy and other combinations of our CF medicines in development aim to help many more people with this disease. In hepatitis C, we are focused on developing multiple all-oral regimens that could further improve treatment. In addition to our development programs, we continue to invest in research for future medicines, with a focus on serious diseases where we have significant scientific expertise and commercial capabilities."

Entering 2013, Vertex's key strategies and business priorities include:

1. Focusing investment on key development programs for:

- **Cystic Fibrosis (CF):** Vertex's CF strategy is to provide benefit to as many CF patients as possible, and to maximize the benefit for these patients, with our approved and investigational medicines. KALYDECO™ (ivacaftor) is currently approved for people with CF ages 6 and older who have at least one copy of the G551D mutation in the *CFTR* gene (approximately 2,000 people with CF worldwide). In 2013, the company is conducting multiple Phase 3 label-expansion and other proof-of-concept studies of ivacaftor monotherapy in people with certain mutations not studied in prior Phase 3 studies. Vertex also expects to initiate a pivotal Phase 3 development program for a combination regimen of VX-809 and ivacaftor in people with CF who have two copies of the F508del mutation in the first quarter of 2013.
- **Hepatitis C:** Vertex's strategy in hepatitis C is to develop new all-oral treatment regimens of 12 weeks or less in duration with a goal of providing a high viral cure rate and improved tolerability. In 2013, Vertex plans to conduct multiple Phase 2 studies of 12-week all-oral treatment regimens that include the company's nucleotide analogue VX-135. These studies are expected to provide safety and viral cure rate data in the second half of 2013 to support the start of pivotal development of one or more all-oral regimens in 2014.
- **Autoimmune Diseases:** Vertex's strategy in autoimmune diseases is to maximize the value of VX-509 across multiple autoimmune diseases globally. The company will evaluate collaborative opportunities that provide funding and capabilities to broaden and accelerate global development of VX-509.

2. Investing in innovative research programs to support development of additional specialty medicines for serious diseases

- Vertex's research efforts are concentrated on additional advancements in CF and other genetic diseases and additional serious diseases in specialty markets.

3. Maximizing revenues and cash flow from the appropriate use of INCIVEK® (telaprevir) and KALYDECO in the U.S. and Canada and from the growth of KALYDECO in Europe and other countries

4. Maintaining financial strength to support future growth and shareholder returns

Cystic Fibrosis

In CF, Vertex is rapidly advancing multiple studies aimed at expanding the number of people who may benefit from our approved and investigational CF medicines, including ivacaftor monotherapy for people with certain mutations not evaluated in prior studies and a combination of VX-809 and ivacaftor in people with two copies of the F508del mutation. The company also is advancing its second-generation corrector research program, which may lead to further improvements in CF treatment in the coming years. Vertex today provided the following updates to its CF development program:

Vertex Receives First Two Breakthrough Therapy Designations from U.S. FDA

- Vertex today announced that the U.S. Food and Drug Administration (FDA) granted the first two Breakthrough Therapy Designations to ivacaftor monotherapy and the combination regimen of VX-809 with ivacaftor for CF. Enacted as part of the 2012 Food and Drug Administration Safety and Innovation Act (FDASIA), Breakthrough Therapy Designation is intended to expedite the development and review of a potential new medicine if it is "intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development."[1]

- The FDA granted Breakthrough Therapy Designation to ivacaftor for potential additional indications beyond the currently approved use of this medicine for people with CF ages 6 and older who have the G551D mutation. The designation for ivacaftor was based on data from clinical and pre-clinical studies, including Phase 3 data in people with the G551D mutation and pre-clinical data in the G551D mutation as well as a number of other mutations. The Breakthrough Therapy Designation for the combination regimen of VX-809 with ivacaftor was based on the Phase 2 combination data announced in 2012. Multiple studies are currently underway in an effort to determine whether patients with other *CFTR* mutations may benefit from ivacaftor alone, and Vertex is also preparing to start a pivotal program of VX-809 in combination with ivacaftor in people who have two copies of the most common CF mutation (F508del).
- The implications of Breakthrough Therapy Designation cannot be determined at this time. Vertex is working with the FDA and other global regulatory agencies to determine any potential implications of the Breakthrough Therapy Designations to its ongoing and planned development activities, and subsequent regulatory submissions, for ivacaftor monotherapy and the combination regimen of VX-809 with ivacaftor.

KALYDECO (ivacaftor) Reimbursement Progress in Europe

- Since approval of KALYDECO in January 2012, the vast majority of the eligible patients with the G551D mutation in the U.S. have initiated treatment with KALYDECO. Growth in 2013 KALYDECO revenues will be dependent on completion of reimbursement discussions in other countries outside the U.S. The company is now seeking reimbursement for KALYDECO in multiple countries in Europe. In December, the National Health Service (NHS) recommended funding of KALYDECO in England. In England, Vertex will make ivacaftor available to eligible people with CF as quickly as possible as part of a patient access program with the NHS and anticipates reimbursement to begin in the second quarter of 2013.

Ivacaftor Label-Expansion Studies

- 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. Enrollment of this study is underway.
    - A Phase 2 proof-of-concept study is underway evaluating ivacaftor in people with CF who have clinical evidence of residual CFTR function. This is the first study to evaluate the safety and 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.
    - Vertex expects to obtain the first data from these studies in the second half of 2013. In 2013, Vertex expects to discuss with the U.S. FDA any potential implications of Breakthrough Therapy Designation on the timing and content of regulatory submissions in the U.S. to support expansion of the KALYDECO label. The company will also

discuss plans for submission of these data with other global regulatory authorities.

Combination of VX-809 and ivacaftor for People with Two Copies of the F508del Mutation

- Vertex recently completed an end-of-Phase 2 meeting with the FDA and is on track to submit final protocols and initiate a pivotal Phase 3 program of VX-809 in combination with ivacaftor in the first quarter of 2013, pending regulatory approval.

Combination of VX-661 and ivacaftor for People with Two Copies of the F508del Mutation

- A Phase 2 study of VX-661 and ivacaftor is ongoing in people with two copies of the F508del mutation. Data from this study are expected in the first half of 2013.

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, among others.

Hepatitis C

In hepatitis C, Vertex plans to initiate multiple studies of the nucleotide analogue VX-135 as part of 12-week all-oral combination treatment regimens. Vertex expects to generate data from these studies in the second half of 2013 to enable progression into pivotal development in 2014. The all-oral studies for VX-135 include:

VX-135 in Combination with Ribavirin

- In the first quarter of 2013, Vertex expects to begin dosing in a study of VX-135 and ribavirin as part of a 12-week all-oral treatment regimen. The study will evaluate safety, tolerability and viral cure rates (SVR12; undetectable hepatitis C virus 12 weeks after the end of treatment).

Collaborative Agreements for All-Oral Studies of VX-135

- In late 2012, Vertex entered into two non-exclusive agreements to conduct Phase 2 proof-of-concept studies of VX-135 in combination with simeprevir (TMC435), a once-daily protease inhibitor being jointly developed by Janssen R&D Ireland and Medivir AB, and with GSK2336805, a once-daily NS5A inhibitor in development by GlaxoSmithKline (GSK). These Phase 2 studies will evaluate safety, tolerability and viral cure rates (SVR12; undetectable hepatitis C virus 12 weeks after the end of treatment) using 12-week combination regimens. A drug-drug interaction study with simeprevir will be initiated shortly, and Vertex expects the Phase 2 studies to be initiated in the first half of the year.

Autoimmune Diseases

In mid-2012, Vertex initiated a Phase 2b study of VX-509, a selective JAK3 inhibitor, in people with moderate to severe rheumatoid arthritis (RA). This study is ongoing, with data expected in the second half of 2013. Vertex believes there is a global opportunity for VX-509 to treat multiple autoimmune diseases. Vertex will evaluate collaborative opportunities that would enable the company to maximize the value of VX-509 by providing funding and capabilities to broaden and accelerate the company's evaluation of VX-509 across multiple additional autoimmune diseases globally.

Financial Strength to Invest in Research Innovation and Development of New Medicines

Vertex plans to announce fourth quarter and full-year 2012 financial results, and 2013 financial guidance, in late January. The company expects total non-GAAP operating expenses (that exclude cost of revenues, stock-based compensation expense, and Alios expenses related to the collaboration with Vertex) for 2013 to be similar to 2012. We anticipate a reduction in our 2013 SG&A expenses compared to 2012. The company's 2013 R&D expenses will primarily support:

- Investment in broad development activities for our late-stage CF and hepatitis C programs, including formulation and commercial supply chain investment
- Completion of Phase 2 evaluation of VX-509 in RA
- Investment in research programs aimed at the creation of future medicines

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.

Indication and Important Safety Information for KALYDECO (ivacaftor)

Ivacaftor (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.

Ivacaftor 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. The efficacy and safety of ivacaftor in children younger than 6 years of age have not been evaluated.

High liver enzymes (transaminases, ALT and AST) have been reported in patients receiving ivacaftor. It is recommended that ALT and AST be assessed prior to initiating ivacaftor, 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 ivacaftor 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 ivacaftor 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 ivacaftor compared to placebo.

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

The dose of ivacaftor must be adjusted when concomitantly used with potent and moderate CYP3A inhibitors. The dose of ivacaftor must be adjusted when used in patients with moderate or severe hepatic disease.

Ivacaftor can cause serious adverse reactions including abdominal pain and high liver enzymes in the blood. The most common side effects associated with ivacaftor 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 ivacaftor. A list of the adverse reactions can be found in the full product labeling for each country where ivacaftor 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, the EU Summary of Product Characteristics for KALYDECO at http://goo.gl/N3Tz4, and the KALYDECO Canadian Product Monograph at www.vrtx.ca.

Indication and Important Safety Information for INCIVEK (telaprevir)

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® (telaprevir) should always be used in combination with peginterferon alfa and ribavirin. INCIVEK combination treatment may cause serious side effects including skin rash and serious skin reactions, anemia (low red blood cell count) that can be severe, and birth defects or death of an unborn baby.

Skin rashes are common with INCIVEK combination treatment. Sometimes these skin rashes and other skin reactions can become serious, require treatment in a hospital, and may lead to death. Patients should call their healthcare provider right away if they develop any skin changes during treatment with INCIVEK. Their healthcare provider will decide if they need treatment or if they need to stop INCIVEK or any of their other medicines. Patients should not

stop taking INCIVEK combination treatment without talking with their healthcare provider first.

Patients' healthcare providers will do blood tests regularly to check for anemia. If anemia is severe, the healthcare providers may tell them to stop taking INCIVEK.

INCIVEK combined with peginterferon alfa and 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. Females who can become pregnant and females whose male partner takes these medicines must have a negative pregnancy test before starting treatment, every month during treatment, and for 6 months after treatment ends. Patients must use two forms of effective birth control during treatment and for 6 months after all treatment has ended. These two forms of birth control should not contain hormones, as these 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.

The most common side effects of INCIVEK combination treatment include itching, nausea, diarrhea, vomiting, anal or rectal problems (including hemorrhoids, discomfort, burning or itching around or near the anus), 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 provider about any side effect that bothers them or doesn't go away.

Please see full Prescribing Information including Boxed Warning, and the Medication Guide for INCIVEK 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 second and third paragraphs of the press release, the information provided in the four numbered key strategies and business priorities for 2013 and statements regarding (i) Vertex rapidly advancing multiple studies aimed at expanding the number of people who may benefit from our approved and investigational CF medicines and Vertex's CF research program; (ii) expectations regarding the submission of final protocols and the initiation of a pivotal Phase 3 program of VX-809 in combination with ivacaftor; (iii) the dependence of growth in KALYDECO revenues in 2013 on the completion of reimbursement discussions in countries outside the U.S. and the anticipation that reimbursement in England will begin in the second quarter of 2013; (iv) expectations regarding the timing and structure of all-oral studies of VX-135; (v) information regarding the company's ongoing and planned studies and the timing of the availability of data from these studies; (vi) the expectation that Vertex will generate data from studies of VX-135 in the second half of 2013 to enable progression into pivotal development in 2014; (vii) potential collaborative opportunities and (viii) expectations regarding 2013 non-GAAP operating expenses. 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 future KALYDECO revenues and/or operating expenses may be incorrect, that the outcomes of Vertex's ongoing and planned clinical studies may not be favorable, that the initiation of planned studies and/or pivotal programs may be delayed or prevented, that collaborative arrangements may not be available on acceptable terms 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.

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[1] http://www.gpo.gov/fdsys/pkg/PLAW-112publ144/pdf/PLAW-112publ144.pdf

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