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Vertex Pharmaceuticals Initiates Phase 3 Registration Program for VX-770, an Oral CFTR Potentiator Targeting the Defective Protein Responsible for Cystic Fibrosis

-Registration program to evaluate improvements in lung function (FEV_1), restoration of CFTR activity and safety--Program designed to support registration in cystic fibrosis patients with the G551D mutation and to provide first evaluation of activity in patients with F508del mutations--Primary endpoint for patients with G551D mutation is improvement in FEV_1 through 24 weeks-

CAMBRIDGE, Mass., May 27, 2009 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced the initiation of a Phase 3 registration program for VX-770, an investigational Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) potentiator that targets the defective CFTR protein that causes cystic fibrosis (CF). The VX-770 registration program will consist of three clinical trials, including a primary 48-week Phase 3 trial that is currently open to enrollment of patients aged 12 years and older who carry the G551D mutation on at least one allele. Two additional trials will evaluate VX-770 in patients aged 6 to 11 years with the G551D mutation on at least one allele and in patients homozygous for the F508del mutation, respectively.

In the registration program, the primary endpoint for patients with the G551D mutation is forced expiratory volume in one second (FEV_1), which will be measured through 24 weeks, and additional FEV_1 measurements will be taken through 48 weeks as a secondary endpoint. Additional secondary endpoints, including sweat chloride, will be measured in each trial to evaluate the effect of VX-770 on improving the function of the defective CFTR protein. VX-770 was discovered as part of a collaboration with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) to develop novel CFTR modulators. CFFT is the nonprofit drug discovery and development affiliate of the Cystic Fibrosis Foundation. Vertex retains worldwide rights to develop and commercialize VX-770 and also the investigational CFTR corrector compound VX-809.

"Data from a Phase 2 study showed VX-770 may improve patient lung function by directly targeting the underlying defect of CF," said Bonnie Ramsey, M.D., a Principal Investigator in the VX-770 registration program and Director of the Cystic Fibrosis Therapeutics Development Network Coordinating Center at Seattle Children's Hospital. "These data were encouraging for the clinical potential of CFTR modulators, and the Phase 3 program announced today will seek to evaluate treatment with VX-770 for longer durations and in broader patient populations to determine the potential future role of this therapy in the treatment of CF."

"The initiation of the VX-770 Phase 3 registration program is a major advancement in our efforts to bring forward new therapies aimed at treating the underlying cause of CF," said Robert J. Beall, Ph.D., President and Chief Executive Officer of the Cystic Fibrosis Foundation. "While we have made significant progress with therapies that treat the symptoms of CF, CFTR modulation represents one of the most promising routes to changing the course of this disease. We are encouraged with the progress of VX-770 and of VX-809, which recently entered a Phase 2a clinical trial in CF patients."

About the VX-770 Phase 3 Registration Program

The VX-770 registration program will consist of three clinical trials:

1. A randomized, placebo-controlled, double-blind, parallel-group Phase 3 study of VX-770 in patients with CF aged 12 years and older with the G551D mutation on at least one allele. The trial is expected to enroll a minimum of 80 patients who will receive either VX-770 or placebo for 48 weeks. In the trial, VX-770 will be dosed as a single 150mg tablet twice daily. The primary endpoint is absolute change from baseline in percent predicted FEV_1 through week 24. Patients who complete the 48-week trial may have the option to enroll in a roll-over study following completion of 48 weeks of treatment in the trial. The trial is currently open to patient enrollment, and Vertex expects the trial to be fully enrolled in the first quarter of 2010.

2. A two-part, randomized, placebo-controlled, double-blind, parallel-group Phase 3 study of VX-770 in patients with CF aged 6 to 11 years with the G551D mutation on at least one allele. Part 1 of the trial is a single-dose pharmacokinetic study that is expected to enroll approximately 10 patients. Following an analysis of data from Part 1, Part 2 of the trial will enroll approximately 30 patients who will receive either VX-770 or placebo for 48 weeks. In the trial, VX-770 will be dosed nominally as a single 100mg tablet twice daily, following confirmation of the pharmacokinetic profile of VX-770 in patients aged 6 to 11 years.

The primary endpoint of the trial is absolute change from baseline in percent predicted FEV_1 through week 24. Patients who complete the 48-week trial may have the option to enroll in a roll-over study following completion of 48 weeks of treatment in the trial. Patient enrollment in Part 1 of the trial is expected to open in the second quarter of 2009.

3. A randomized, placebo-controlled, double-blind, parallel-group Phase 2 study of VX-770 in patients with CF aged 12 years and older who are homozygous for the F508del mutation. The trial is expected to enroll approximately 120 patients who will receive either VX-770 or placebo for 16 weeks. In the trial, VX-770 will be dosed as a single 150mg tablet twice daily. The primary endpoints of the trial are safety as well as absolute change from baseline in percent predicted FEV_1 through week 16. Patient enrollment in the trial is expected to begin in the third quarter of 2009.

The primary endpoint for patients with the G551D mutation will be $FEV_{1,}$ which will be measured through 24 weeks. Additional FEV_1 measurements will be taken through 48 weeks as a secondary endpoint for patients with the G551D mutation to assess durability of the response. The trials in patients with the G551D mutation will be conducted for 48 weeks to gain additional safety data in G551D patients. For patients with the F508del mutations, the primary endpoints will be safety and FEV_1, which will be measured through 16 weeks. Additional secondary endpoints, including sweat chloride, will be measured in each trial to evaluate the effect of VX-770 on improving the function of the defective CFTR protein. The trials will be conducted at approximately 110 clinical trial sites in North America, Europe and Australia.

About Cystic Fibrosis

Cystic fibrosis is a life-threatening genetic disease affecting approximately 30,000 people in the United States and 70,000 people worldwide. Mutations in the *CFTR* gene cause patients with CF to have defective or missing CFTR proteins at their cell surfaces. These defective or missing CFTR proteins result in poor chloride ion flow across cell membranes, causing the body to produce abnormally thick, sticky mucus that leads to chronic, life-threatening lung infections. Today, the median predicted age of survival for a person with CF is more than 37 years.

According to the *2007 Cystic Fibrosis Foundation Patient Registry Annual Data Report*, approximately four percent of the total CF patient population in the U.S. have the G551D mutation on at least one allele, 49 percent of the total CF patient population in the U.S. are homozygous for the F508del mutation and an additional approximately 38 percent of the total CF patient population are heterozygous for the F508del mutation.

About VX-770 and VX-809

VX-770 is a novel oral CFTR potentiator drug candidate designed to increase the activity of defective CFTR proteins at the cell surface. In a two-part Phase 2a clinical trial completed in 2008, treatment with VX-770 for 14 or 28 days resulted in a significant improvement in lung function, as measured by an increase in FEV_1, and significant improvements in the function of the CFTR protein, as measured by changes from baseline in sweat chloride levels and changes in nasal potential difference (NPD). The Phase 2a trial enrolled 39 patients, including 20 patients in Part 1 and 19 patients in Part 2, who received either VX-770 or placebo. Based on these results, Vertex advanced VX-770 into the Phase 3 registration program designed to support registration of VX-770 in patients with the G551D mutation.

Vertex is also developing VX-809, a novel oral CFTR corrector drug candidate designed to increase the concentration of F508del CFTR proteins at the cell surface. Vertex completed multiple Phase 1 studies of VX-809 in healthy volunteers and CF patients in 2008 and early 2009 and initiated a Phase 2a clinical trial of VX-809 in CF patients homozygous for the F508del mutation in March 2009.

Based on *in vitro* data observed to date for both investigational corrector and potentiator compounds, Vertex believes there is a rationale to explore the clinical potential of combining both types of compounds in future studies.

Patients interested in further information about clinical trials of VX-770 or VX-809 should visit www.clinicaltrials.gov or www.cff.org/clinicaltrials.

Collaborative History with CFFT

Vertex initiated its CF research program in 1998 as a part of a collaboration with CFFT. Vertex and CFFT expanded the agreement in 2000 and again in 2004, and in March 2006, entered into a collaboration for the accelerated development of VX-770. In addition to the development collaboration for VX-770, in January 2006 Vertex and CFFT entered into an expanded research collaboration to develop novel corrector compounds. Vertex has received approximately $75 million from CFFT to support CF research and development efforts.

About the Cystic Fibrosis Foundation

The Cystic Fibrosis Foundation is the leading organization in the United States devoted to curing and controlling cystic fibrosis. To advance the search for a cure, the Foundation has invested more than $320 million in promising drug research in the biotech industry since 1998. Virtually every the approved CF therapy available today was made possible because of the support from the Foundation. For more information, visit www.cff.org.

About Vertex

Vertex Pharmaceuticals Incorporated is a global biotechnology company committed to the discovery and development of breakthrough small molecule drugs for serious diseases. The Company's strategy is to commercialize its products both independently and in collaboration with major pharmaceutical companies. Vertex's product pipeline is focused on viral diseases, cystic fibrosis, inflammation, autoimmune diseases, cancer, and pain. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

Lexiva is a registered trademark of the GlaxoSmithKline group of companies.

Safe Harbor Statement

This press release contains forward-looking statements, including statements regarding (i) the registration program being designed to support registration in cystic fibrosis patients with the G551D mutation and to provide the first evaluation of activity in patients homozygous for the F508del mutation, (ii) the clinical trial design of each of the three clinical trials in the registration program, including the primary and secondary endpoints, the anticipated number of patients to be enrolled, the anticipated dosing schedule, and the number and location of the clinical trial sites, (iii) data from a Phase 2 clinical trial showing that VX-770 may improve lung function by directly targeting the underlying defect of CF, (iv) that the Phase 3 program will evaluate longer treatment durations and broader patient populations to determine the potential future role of VX-770 in the treatment of CF, (v) the initiation of the registration program being a major advance in the efforts to bring forward new therapies aimed at the underlying cause of CF, (vi) CFTR modulation representing one of the most promising routes to changing the course of this disease, (vii) Vertex's expectations regarding when enrollment will commence and/or be completed for each of the three clinical trials in the registration program, and (viii) Vertex's beliefs regarding the rationale to explore the clinical potential of combining corrector and potentiator compounds in future studies. While the Company believes the forward-looking statements contained in this press release are accurate, those statements are subject to risks and uncertainties that could cause actual outcomes to vary materially from the outcomes referenced in the forward-looking statements. These risks and uncertainties include, among other things, the risks that efforts to develop VX-770 or VX-809 may not proceed due to technical, scientific, commercial, financial or other reasons, that clinical trials may not proceed as planned due to drug supply or patient enrollment issues, that additional clinical trials of VX-770 or VX-809 will not reflect the results obtained in the studies to date or confirm the current hypotheses that CFTR modulation with VX-770 or VX-809 could be a useful cystic fibrosis therapy, that an adverse event profile for VX-770 or VX-809 could be revealed in further nonclinical or clinical studies that could put further development of VX-770 or VX-809 in jeopardy or adversely impact its therapeutic value, 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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