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Vertex Pharmaceuticals Initiates Clinical Trial to Evaluate Combination Regimens of VX-770 and VX-809 Targeting the Defective Protein Responsible for Cystic Fibrosis

-Primary endpoints of safety, tolerability and effect on CFTR function as measured by sweat chloride--Phase 2a proof of mechanism clinical trial to enroll people with the most common mutation of cystic fibrosis, known as F508del-

CAMBRIDGE, Mass., Oct 18, 2010 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced the initiation of a Phase 2a clinical trial that will evaluate multiple combinations of VX-770 and VX-809, investigational oral cystic fibrosis transmembrane conductance regulator (CFTR) modulators for the treatment of cystic fibrosis (CF). The trial is designed to evaluate the safety and tolerability of VX-809 dosed alone for 14 days followed by dosing of VX-809 in combination with VX-770 for 7 days. The trial will also assess the effect of VX-809 alone and in combination with VX-770 on CFTR function, as measured by sweat chloride. The three-part trial is designed to enroll up to a total of 160 people with two copies of the F508del *CFTR* mutation, the most common mutation in the *CFTR* gene.

CF is caused by defective or missing CFTR proteins, which result in poor ion flow across cell membranes, including in the lung, and the accumulation of abnormally thick, sticky mucus that leads to chronic lung infections and progressive lung damage. VX-770, known as a CFTR potentiator, aims to increase the function of defective CFTR proteins by increasing the gating activity, or ability to transport ions across the cell membrane, of CFTR at the cell surface. In people with the F508del mutation, CFTR proteins do not reach the cell surface in normal amounts. VX-809, known as a CFTR corrector, aims to increase CFTR function by increasing the trafficking, or movement, of CFTR to the cell surface. The trial announced today will be the first to evaluate whether a combination regimen of VX-770 and VX-809 can improve CFTR function by increasing both the gating and trafficking of CFTR in people with CF.

"The start of this trial has been greatly anticipated by the CF community and marks a milestone in our efforts to discover and develop new treatments to address the underlying cause of this disease," said Robert J. Beall, Ph.D., president and CEO of the Cystic Fibrosis Foundation. "This is the first opportunity to evaluate the use of two CFTR modulator compounds with potentially complementary mechanisms and may provide important insight into the role these types of compounds may play in the future treatment of people with CF."

"Our approach to the treatment of CF underscores Vertex's commitment to innovative science that seeks to better understand and fundamentally change the way life-threatening diseases are treated," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer for Vertex. "Importantly, this trial will inform the development path for future studies combining novel CFTR modulators for the treatment of people with the most common mutation of CF, known as F508del."

VX-770 and VX-809 were discovered as part of a collaboration with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) to discover and 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 VX-809.

Study Design

This three-part, randomized, double-blind, placebo-controlled trial will enroll up to a total of 160 people with CF ages 18 or older who have two copies of the F508del mutation in the *CFTR* gene. People will be enrolled at approximately 21 clinical trial sites in the U.S., Europe, New Zealand and Australia. Patient screening is now underway at certain clinical trial sites. Vertex expects to have interim data from the first part of the trial from approximately 60 patients in the first half of 2011.

The primary goals of the trial are to evaluate the safety, tolerability and effect on CFTR function as measured by sweat chloride. Elevated sweat chloride levels are a diagnostic hallmark that occur in CF patients and result directly from defective CFTR function in skin cells in the sweat duct.

The trial will be conducted in three sequential parts, each of which may include up to three treatment arms. In Part 1, two of the arms will evaluate VX-809 dosed as monotherapy for 14 days followed by dosing in combination with VX-770 for an additional 7 days. The third arm is a control arm that will evaluate VX-809 placebo dosed for 14 days followed by dosing of VX-809 placebo in combination with VX-770 placebo for an additional 7 days. The design for Part 1 of the study is as follows:

PART 1 STUDY DESIGN	VX-809 Monotherapy for 14 Days	VX-809/VX-770 Combination for 7 Days
Arm 1 (n=20)	200 mg VX-809 QD	200 mg VX-809 QD + 150 mg VX-770 q12h
Arm 2 (n=20)	200 mg VX-809 QD	200 mg VX-809 QD + 250 mg VX-770 q12h
Arm 3 (n=20)	VX-809 Placebo QD	VX-809 Placebo QD + VX-770 Placebo q12h

Following a planned analysis of data from Part 1 of the study, Vertex may conduct up to two additional parts of the study to evaluate additional higher doses of VX-809 dosed as monotherapy for 14 days and combined with VX-770 for 7 days. A Phase 2 trial of VX-809 showed the compound was well-tolerated with respiratory events being the most commonly reported adverse events in the trial. Additionally, the trial showed a statistically significant decline in sweat chloride, a secondary endpoint, at both the 100 mg and 200 mg once-daily doses, suggesting that the function of the CFTR protein was increased in patients during dosing. Part 1 of the trial announced today will evaluate VX-809 doses of 200 mg followed by potentially higher doses in Parts 2 and 3 to determine whether there is any further increase in CFTR function at higher doses of VX-809 dosed as monotherapy and in combination with VX-770.

In addition to the Phase 2a trial announced today, Vertex is conducting a Phase 3 registration program for VX-770 that consists of three fully enrolled clinical trials. The Phase 3 program for VX-770 includes two 48-week Phase 3 trials (STRIVE and ENVISION) that enrolled patients with the G551D mutation and a 16-week Phase 2 trial (DISCOVER) that enrolled patients with two copies of the F508del mutation and was designed primarily as a safety study. The 48-week dosing periods of STRIVE and ENVISION are ongoing, and the 16-week dosing period of the DISCOVER trial is complete. Safety data from the 16-week dosing period of DISCOVER provided support for the initiation of the Phase 2a study evaluating combination regimens of VX-770 and VX-809 in people with two copies of the F508del mutation and will supplement safety data from the two Phase 3 trials (STRIVE and ENVISION) in the VX-770 registration program.

Vertex believes that safety data from the 16-week dosing period of DISCOVER support the company's plans to seek registration of VX-770 as a single agent using data from the Phase 3 registration program in people with CF who have at least one copy of the G551D mutation. A proportion of patients in the VX-770 and placebo dosing groups from the DISCOVER trial achieved certain eligibility criteria during the 16-week dosing period and have enrolled in a 96-week, open-label rollover study of VX-770, which is ongoing. Additional analyses of data from the DISCOVER rollover study will be conducted in the first half of 2011.

Full data from the Phase 3 registration program of VX-770 are expected in the first half of 2011. Pending the results, Vertex expects to submit a New Drug Application with the U.S. Food and Drug Administration and a Marketing Authorization Application with European regulatory authorities for VX-770 in the second half of 2011.

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. Today, the median predicted age of survival for a person with CF is more than 37 years. According to the *2008 Cystic Fibrosis Foundation Patient Registry Annual Data Report*, approximately 4% of the total CF patient population in the U.S. have at least one copy of the G551D mutation, 48% of the total CF patient population in the U.S. have two copies of the F508del mutation and an additional 39% of the total CF patient population have one copy of the F508del mutation.

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

Collaborative History with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT)

Vertex initiated its CF research program in 1998 as a part of a collaboration with CFFT, the non-profit drug discovery and development affiliate of the Cystic Fibrosis Foundation. 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 world's leader in the search for a cure for cystic fibrosis. The Foundation funds more CF research than any other organization and nearly every CF drug available today was made possible because of Foundation support. Based in Bethesda, Md., the Foundation also supports and accredits a national care center network that has been recognized by the National Institutes of Health as a model of care for a chronic disease.

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 expectation that the Phase 2a clinical trial described in this press release will evaluate multiple combinations of VX-770 and VX-809; (ii) the design of the trial, including the goals of the trial and the primary endpoints, the number of patients the Company expects to enroll and the number and location of clinical trial sites; (iii) the methods by which VX-770 and VX-809 aim to increase CFTR function; (iv) this trial being the first opportunity to evaluate the use of two CFTR modulator compounds with potentially complementary mechanisms and potentially providing important insight into the role these types of compounds may play in the future treatment of people with CF; (v) the expectation that this trial will inform the development path for future studies combining CFTR modulators; (vi) the expectation that Vertex will have interim data from the first part of the trial in the first half of 2011; (vii) the possibility that Vertex will conduct up to two additional parts of the clinical trial to evaluate higher doses of VX-809; (viii) safety data from the DISCOVER trial providing support for the initiation of the Phase 2a clinical trial evaluating combination regimens of VX-770 and VX-809 and supplementing the safety data from the two Phase 3 trials in the VX-770 registration program; (ix) Vertex's belief that safety data from the 16-week dosing period of DISCOVER support the company's plans to seek registration of VX-770 as a single agent using data from the Phase 3 registration program in people with CF who have at least one copy of the G551D mutation; (x) the plan to conduct additional analyses of data from the DISCOVER rollover study in the first half of 2011; (xi) the expectation that full data from the Phase 3 registration program of VX-770 are expected in the first half of 2011 and (xii) the expectation, pending the results from the Phase 3 registration program, that Vertex will submit a New Drug Application with the U.S. Food and Drug Administration and a Marketing Authorization Application with European regulatory authorities for VX-770 in the second half of 2011. 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 risk that efforts to develop VX-770 separately, or in combination with 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 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 their 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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