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Vertex Pharmaceuticals Initiates Phase 2 Development for CFTR Corrector VX-809 in Patients with Cystic Fibrosis

- Primary endpoints of safety and tolerability to be evaluated in Phase 2a clinical trial -- Measurements of CFTR function to be assessed as secondary endpoints -- Trial to enroll approximately 90 patients with the F508del CFTR mutation -

CAMBRIDGE, Mass., Mar 25, 2009 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced the initiation of a Phase 2a clinical trial for VX-809, an investigational Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) corrector that targets the defective CFTR protein that causes cystic fibrosis (CF). The trial is designed primarily to evaluate the safety and tolerability of multiple doses of VX-809 in patients with CF. In addition to assessing safety, the trial will also evaluate the potential effect of VX-809 on measures of CFTR function. The trial is expected to enroll approximately 90 patients homozygous for the F508del mutation in the *CFTR* gene, the most common mutation in CF patients. VX-809 was discovered as part of a collaboration with Cystic Fibrosis Foundation Therapeutics Inc. (CFFT) to develop novel CFTR correctors. CFFT is the nonprofit drug discovery and development affiliate of the Cystic Fibrosis Foundation. Vertex retains worldwide rights to develop and commercialize VX-809 and its CFTR potentiator VX-770.

"CF treatment today involves the use of multiple drugs that address the symptoms and complications of CF, but no therapy currently exists that targets the underlying cause of this disease," said Robert J. Beall, Ph.D., President and Chief Executive Officer of the Cystic Fibrosis Foundation. "We are encouraged by Vertex's rapid progress in advancing two novel therapies aimed at the underlying defect in CF. We believe that VX-770, which is expected to enter registration studies in the first half of this year, and VX-809, which today entered Phase 2 clinical development, have the potential to play a significant role in changing the future course of this disease for people with CF."

"The advancement of VX-809 into Phase 2 development demonstrates our commitment to improve care for patients with CF," said Freda Lewis-Hall, M.D., Executive Vice President, Medicines Development, and Chief Medical Officer of Vertex. "This Phase 2a trial is designed primarily to provide a safety, tolerability and pharmacokinetic evaluation for VX-809. Any additional signals observed in this trial, including changes in sweat chloride and nasal potential difference, or any measurable impact on FEV_1, that indicate VX-809 has an effect on measures of CFTR function may be highly informative and encouraging for planning future clinical trials with VX-809."

Study Design

The Phase 2a trial for VX-809 announced today is expected to enroll approximately 90 patients with CF ages 18 years or older who are homozygous for the F508del *CFTR* mutation. According to the 2007 Cystic Fibrosis Foundation Patient Registry Annual Data Report, approximately 49 percent of the total CF patient population in the U.S. are homozygous for the F508del mutation, while approximately 38 percent of the total CF patient population are heterozygous for the F508del mutation. The trial will be conducted at approximately 22 clinical trial sites in North America and Europe. Patient screening is now underway at certain clinical trial sites. The randomized, double-blind study will initially evaluate two dose levels of VX-809 compared to placebo given orally once daily for 28 days in a parallel design. Following a planned interim safety analysis, the trial may evaluate an additional two dose levels of VX-809 compared to placebo. The trial is expected to be complete in early 2010.

About Cystic Fibrosis

Cystic fibrosis is a 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.

About VX-809 and VX-770

VX-809 is a novel oral CFTR corrector 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.

Vertex is also developing VX-770, a novel oral CFTR potentiator designed to increase the activity of defective CFTR proteins at the cell surface. VX-770 is expected to enter a registration program focused on patients with the G551D mutation in the first half of 2009. The G551D mutation accounts for approximately 4 percent of the total CF patient population in the U.S.

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 for combining both types of compounds in future studies.

Patients interested in enrolling in clinical trials of VX-809 or VX-770 should visit www.clinicaltrials.gov.

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 May 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 discover 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 nearly $300 million in promising drug research in the biotech industry since 1998. Virtually all the approved CF therapies available today were made possible because of the support form 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 may contain forward-looking statements, including statements that (i) VX-770 is expected to enter registration trials in the first half of 2009; (ii) VX-770 and VX-809 could play a significant role in changing the future course of CF for patients; (iii) any additional signals observed in the planned clinical trial that indicate that VX-809 has an effect on measures of CFTR function or FEV1 may be highly informative and encouraging for planning future clinical trials with VX-809; (iv) the Company expects to enroll approximately 90 patients in this trial at approximately 22 clinical sites in North America and Europe; (v) the planned trial may evaluate two dose levels in addition to the initial two dose levels planned, after a planned interim safety analysis; and (vi) the clinical trial is expected to be complete in early 2010. While management makes its best efforts to be accurate in making forward-looking statements, 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 financial, technical, scientific, commercial or other reasons, that clinical trials may not proceed as planned due to drug supply or patient enrollment issues, that additional clinical studies 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, that regulatory authorities will require additional data before committing to a registration program for VX-770, and other risks listed under Risk Factors in Vertex's Form 10-K filed with the Securities and Exchange Commission.

(VRTX - GEN)

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