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Vertex Announces Positive Results for VX-770, an Oral Investigational Agent That Targets a Defective Protein Responsible for Cystic Fibrosis

--Interim analysis of 14-day study of VX-770 shows a significant improvement in lung function --

--Lung function data supported by corresponding improvements in disease-related biomarkers--

--Results to be shared with health authorities in order to identify the most rapid path forward --

CAMBRIDGE, Mass., Mar 27, 2008 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced results from a planned interim analysis of an ongoing Phase 2a clinical trial in patients who carry the G551D mutation in the gene that causes cystic fibrosis (CF). The interim analysis showed that dosing of VX-770, an investigational CF potentiator, as an oral agent for 14 days resulted in improved lung function and in improved function of the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) protein as measured by changes in sweat chloride levels and changes in nasal potential difference (NPD). Results from the interim analysis support the hypothesis that improving chloride ion transport in CF patients may correlate to improvements in lung function and provide benefit to patients. Results are being shared with regulatory authorities and leading CF investigators in order to identify the most rapid path forward for the compound.

"While these are early data, it is unprecedented for an investigational oral compound for the treatment of CF to have such a marked effect on multiple measures of CF disease activity. We saw an average improvement in lung function of 10 percent in patients receiving the highest dose, compared to no observed improvement in patients who received placebo," said Frank Accurso, M.D., Director of the Cystic Fibrosis Center and Professor of Pediatrics at the University of Colorado School of Medicine in Denver. "These data suggest that VX-770 may be able to improve lung function by targeting an underlying defect in CFTR that causes the disease. In patients with CF, inadequately functioning or missing CFTR is believed to result in abnormal balance of fluid and salt in the airways. These are early clinical data in a subset of patients with malfunctioning CFTR, but an important proof-of-concept, and we look forward to evaluating the longer-term safety and efficacy of VX-770 in additional studies."

Interim Analysis Summary

The results being reported are from 20 patients with the G551D mutation in CFTR who received either VX-770 or placebo in addition to standard therapies for 14 days as part of a blinded, randomized, two-period crossover study design. Four subjects received placebo during both 14-day dosing periods; 8 subjects received 25 mg of VX-770 twice-daily during one period and 75 mg in the other; and 8 subjects received 75 mg of VX-770 twice-daily during one period and 150 mg, the highest dose in the study, during the other dosing period. There was a one to four week wash-out between each dosing period.

-- Safety: In the 14-day trial, VX-770 appeared to be well-tolerated. Observed adverse events were similar between VX-770 and placebo treatment. Two serious adverse events were observed in one patient and were not attributed to VX-770.

-- Lung Function: In the Phase 2a trial, lung function in patients was assessed with FEV(1), a standard test that measures the amount of air that can be exhaled in one second. FEV(1) is the lung function test most commonly used to monitor progression of airway disease in CF patients. Patients with CF typically experience a decline in lung function of 1-2% per year during their life, as measured by FEV(1). Over the 14-day dosing period in the Phase 2a study, patients receiving the highest dose of VX-770 (150mg twice daily) showed a mean increase from baseline in FEV(1) of 10.1%, or 0.22L (pless than0.008). In contrast, patients receiving placebo showed a slight decrease in FEV(1) (less than 1%; 0.03L) over the 14-day period.

-- Sweat Chloride: Elevated sweat chloride levels are a diagnostic hallmark that occur in all CF patients and result directly from defective CFTR activity in epithelial cells in the sweat duct. The amount of chloride in the sweat is measured using a standard skin test. Patients with CF typically have sweat chloride levels in excess of 60 mmol/L, while normal values are less than40 mmol/L. In patients receiving the highest dose of VX-770 (150mg twice daily) in the Phase 2a study, sweat chloride decreased

from a mean 95.5 mmol/L at baseline to 53.2 mmol/L over the 14-day dosing period (pless than0.0001). Six of 8 patients in the 150 mg dose group achieved a decrease in sweat chloride to below 60 mmol/L. There was no notable change in sweat chloride in patients receiving placebo.

-- Nasal Potential Difference (NPD): NPD assesses several aspects of ion channel activity by measuring voltage changes across the nasal epithelia and is used as a direct measure of CFTR activity and chloride ion movement in upper airway epithelial cells. Typical assessments of CF patients using NPD show very low chloride ion secretion in the nasal passage. After 14 days of dosing, patients receiving the highest dose of VX-770 (150 mg twice daily) showed a significant mean change from baseline in chloride ion secretion in NPD of -5.4 mV (p=.01). In contrast, in those treated with placebo, a mean change of -1.74 mV was observed.

Robert J. Beall, Ph.D., President and Chief Executive Officer of the Cystic Fibrosis Foundation, said, "More than 10 years ago, the Cystic Fibrosis Foundation believed that Vertex's approach to treating CF by directly targeting CFTR function - the underlying mechanism of the disease - was an important opportunity to meaningfully change the course of cystic fibrosis. Today, we are highly encouraged by the early clinical data for VX-770, as these results represent a significant step forward in understanding the potential for new treatment approaches for the more than 70,000 cystic fibrosis patients worldwide."

"These early data provide clinical proof-of-concept that a CFTR modulator such as VX-770 can have a direct effect on CFTR activity in patients with CF and potentially improve lung function," said John Alam, M.D., Executive Vice President, Medicines Development, and Chief Medical Officer of Vertex. "While additional data and evaluation are needed to fully understand VX-770's clinical profile and its disease-modifying potential in CF, we are pleased to see these early results. We look forward to moving into the second part of this Phase 2 trial for VX-770, which will enroll approximately 16 patients for dosing of VX-770 or placebo for 28 days, beginning in the second quarter of 2008. This result further supports the collaborative approach that the Cystic Fibrosis Foundation and Vertex have taken to finding drugs that treat the underlying defect responsible for the disease."

Based on the results of the interim analysis, in addition to proceeding to Part 2 of the Phase 2a study, Vertex plans to meet with regulatory authorities and leading CF investigators in order to identify the most rapid path forward for the compound.

About the Phase 2a Clinical Trial

Results reported today are from an interim analysis of Part 1 of an ongoing Phase 2a clinical trial evaluating the safety and pharmacokinetics of VX-770, and investigating the effect of VX-770 on clinical measures of lung function and CFTR activity in patients 18 years of age or older who possess the G551D mutation. In Part 1 of the trial, 20 patients with the G551D mutation in CFTR received either VX-770 or placebo in addition to standard therapies for 14 days as part of the trial's blinded, randomized, crossover design. Based on the interim results, Vertex expects to proceed to Part 2 of the trial, which will enroll approximately 16 patients for dosing of VX-770 or placebo for 28 days, beginning in the second quarter of 2008. In the trial, VX-770 is being dosed as an oral therapy. Vertex expects to present the interim results from Part 1 of this trial at a medical forum in 2008.

About CF and VX-770

Cystic Fibrosis affects about 30,000 people in the United States and approximately 70,000 people worldwide. Cystic fibrosis is caused by a genetic mutation that results in a malfunctioning or missing CFTR protein on cell surfaces, that results in an imbalance of salt and water. This fluid imbalance in the lungs causes a cascade of mucus plugging, infection and inflammation that characterizes CF, and accounts for a large portion of the morbidity and mortality seen with CF. Current therapies treat CF by managing the symptoms caused by the salt/water imbalance. While aggressive management of CF has improved long-term outcomes in CF patients, the median predicted life expectancy is currently 37 years.

VX-770, an investigational oral potentiator, is designed to help restore the balance of salt and water by acting directly on the malfunctioning CFTR protein. Clinical development of VX-770 is currently focused on a subset of CF patients who have a specific type of CFTR mutation known as G551D that constitutes approximately 4 percent of the CF patient population in the U.S. Vertex also plans to evaluate VX-770 in patients with other mutations that result in malfunctioning CFTR on the cell surface. VX-770 was advanced into preclinical development based on successful collaborative research with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) that incorporated capabilities and proprietary ion channel research from Vertex's San Diego research site.

Collaborative History with CFFT

Vertex initiated its CF research program in 1998 as part of a collaboration with CFFT. In March 2006 Vertex and CFFT entered into a collaboration for the accelerated development of VX-770. In addition, in January 2006, Vertex and CFFT entered into an expanded research collaboration for the discovery of novel corrector compounds, the first of which, VX-809, is in a Phase 1 clinical study. Vertex retains worldwide rights to develop and commercialize these compounds.

About the Cystic Fibrosis Foundation

The Cystic Fibrosis Foundation, the leading organization focused on curing and controlling cystic fibrosis, has invested nearly $300 million in drug research with biotech companies since 1998 to develop therapies to fight CF. The result is a drug development pipeline with more than 30 promising therapies. Virtually all of the approved cystic fibrosis therapies available today were made possible because of the support of the Cystic Fibrosis 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, inflammation, autoimmune diseases, cancer, pain and bacterial infection. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

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

Webcast and Conference Call on March 27

Vertex Pharmaceuticals will host a conference call on March 27, 2008 at 8:30 a.m. EDT to review the VX-770 interim analysis data. This call will be broadcast via the Internet at www.vrtx.com from the 'Events & Presentations' page. To listen to the call live on the telephone, dial (800) 374-0296 (US/Canada) and (706) 634-2224 (International) using Conference Code 40827959. Please dial in 5-10 minutes prior to the scheduled start time. The call will be available for replay via telephone commencing March 27, 2008 at 8:00 p.m. EDT running through 5:00 p.m. EDT on April 3, 2008. The replay phone number for the is (800) 642-1687 (US and Canada) and (706) 645-9291 (international) and the conference ID number is 40827959.

The webcast will be available for replay on the Internet commencing March 27, 2008 at 8:00 p.m. EDT running through 5:00 p.m. EDT on April 10, 2008. You may access the replay at www.vrtx.com, Events & Presentations. Alternatively, Vertex is providing a podcast MP3 file available for download on the Vertex website, www.vrtx.com, until April 10, 2008.

Vertex Safe Harbor Statement

This press release may contain forward-looking statements, including statements that (i) Vertex expects to share these interim clinical trial results for VX-770 with health authorities to identify the most rapid development path forward; (ii) results from this interim analysis support the hypothesis that improving chloride ion transport in CF patients may correlate to improvements in lung function and provide patient benefit; (iii) VX-770 may be able to improve lung function by targeting an underlying defect in CFTR that is a cause of CF; (iv) Vertex expects to move into the second part of its Phase 2 trial of VX-770 beginning in the second quarter of 2008; (v) Vertex expects to present the interim results from Part 1 of this Phase 2 clinical trial at a medical meeting in 2008; and (vi) Vertex plans to evaluate VX-770 in patients with other mutations that result in malfunctioning CFTR on the cell surface. While management makes its best efforts to be accurate in making forward-looking statements, such statements are subject to risks and uncertainties that could cause Vertex's actual results to vary materially. These risks and uncertainties include, among other things, the risks that efforts to develop VX-770 may not proceed due to financial, technical, scientific, commercial or other reasons, that clinical trials may not proceed as planned due to technical, scientific, supply or patient enrollment issues, that additional clinical studies of VX-770 will not reflect the results obtained in the study to date and will not support additional development activity, that regulatory authorities will require additional data before engaging in development planning discussions with Vertex, and other risks listed under Risk Factors in Vertex's Form 10-K filed with the Securities and Exchange Commission.

Vertex's press releases are available at www.vrtx.com.

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