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Vertex Announces Positive 28-Day Results for VX-770, an Oral Investigational Agent That Targets a Defective Protein Responsible for Cystic Fibrosis

- **VX-770 was well-tolerated when dosed as 150 mg and 250 mg twice daily for 28 days**
- **VX-770 resulted in a significant increase in FEV1, and significant changes in sweat chloride and nasal potential difference, important markers of CFTR function**
- **Researchers to present 28-day results at the North American Cystic Fibrosis Conference on October 23**

CAMBRIDGE, Mass., Oct 20, 2008 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced positive results from a preliminary analysis of data from Part 2 of the Phase 2a clinical trial of the investigational oral drug VX-770 in cystic fibrosis (CF) patients who carry the G551D CFTR mutation. VX-770, an investigational CFTR potentiator, was well-tolerated when dosed orally as 150 mg or 250 mg twice daily for 28 days. In this analysis, no patients discontinued treatment and no serious adverse events were reported. At both the 150 mg and 250 mg doses, significant improvements in lung function, as measured by an increase in FEV1, and significant improvements in the function of the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) protein, as measured by changes from baseline in sweat chloride levels and changes in nasal potential difference (NPD), were observed. In patients receiving placebo, a smaller increase in FEV1 was observed at 28 days that was not statistically significant, and no significant changes from baseline in sweat chloride levels or NPD were observed. Based on these results, Vertex intends to work with global regulatory authorities to finalize the design of a registration program for VX-770 targeted to begin in 2009. VX-770 was developed with support from Cystic Fibrosis Foundation Therapeutics, Inc., the nonprofit affiliate of the Cystic Fibrosis Foundation.

"VX-770 targets the underlying defect in the CFTR protein that causes cystic fibrosis, and represents a potentially new approach to changing the treatment of this disease," 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. "In patients with CF, dysfunctional or missing CFTR is believed to result in abnormal balance of fluid and salt in the airways. These data from patients with a particular type of dysfunctional CFTR are consistent with earlier findings obtained in Part 1 of this trial, in which VX-770 was dosed for 14 days, and further support the clinical potential of CFTR modulators such as VX-770."

Robert J. Beall, Ph.D., President and Chief Executive Officer of the Cystic Fibrosis Foundation, said, "Data from the Phase 2 trial of VX-770 provides evidence that a small molecule can address the basic defect in cystic fibrosis and suggests that modulation of CFTR may play an important role in CF therapy. The CF community looks forward to reviewing these data at this week's North American Cystic Fibrosis Conference where the world's leading CF caregivers will see these data in detail for the first time."

Data Analysis Summary

The preliminary results reported are from 19 patients with the G551D mutation in CFTR who received either VX-770 or placebo in addition to standard therapies for 28 days as part of a blinded, randomized study design. Four subjects received placebo; 8 subjects received 150 mg of VX-770 twice daily; and 7 subjects received 250 mg of VX-770 twice daily. Together with results generated from Part 1 of the study obtained earlier this year, which evaluated a separate cohort of 20 patients dosed with VX-770 or placebo for 14 days, these results further support the hypothesis that improving chloride ion transport in CF patients may correlate with improvements in lung function.

Safety: Through 28 days of 150 mg and 250 mg twice-daily dosing in Part 2 of the Phase 2a study, VX-770 appeared to be well-tolerated. No serious adverse events were reported and no patients discontinued treatment. All reported adverse advents were mild or moderate in severity. Safety was the primary endpoint of the study, and a detailed safety analysis is ongoing.

Efficacy Evaluation: In both Part 1 and Part 2 of this Phase 2a study, lung function and CFTR protein function were measured.

- Lung function was assessed as a secondary endpoint with FEV1, a standard test that measures the amount of air that can be exhaled in one second. FEV1 is the lung function test most commonly used to monitor CF disease progression, which is characterized by decreases in FEV1 values compared to FEV1 values observed in healthy individuals.

- CFTR activity was measured by the secondary endpoints of sweat chloride and nasal potential difference (NPD). 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 test. Patients with CF typically have elevated sweat chloride levels in excess of 60 mmol/L, while normal values are less than 40 mmol/L. 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 CFTR-mediated chloride ion transport in the nasal passage. CFTR-mediated chloride ion transport values in NPD that are more negative are indicative of increased CFTR activity.

Preliminary Results by Dose Group

150 mg Dose Group: In Part 2 of the Phase 2a trial, 8 subjects received 150 mg of VX-770 twice daily. The results for this dose group are as follows:

- The mean increase from baseline in FEV1 at 28 days was 11.6% ($p < 0.01$). The pattern of FEV1 response was consistent and characterized by a rapid and sustained increase in FEV1 through 28 days.

- The mean decrease from baseline in sweat chloride at 28 days was -52.8 mmol/L ($p < 0.01$). The mean baseline for patients in this dose group was 102 mmol/L.

- The mean change from baseline in CFTR-dependent chloride ion transport as measured by NPD was -4.3 mV ($p < 0.05$) after 28 days.

The Part 2 results for the 150 mg dose are consistent with the results from the interim analysis of Part 1 of the trial for the same dose.

250 mg Dose Group: In Part 2 of the Phase 2a trial, 7 subjects received 250 mg of VX-770 twice daily. The results for this dose group are as follows:

- The mean increase from baseline at 28 days in FEV1 was 7.4% ($p < 0.05$). The pattern of FEV1 response was consistent and characterized by a rapid and sustained increase in FEV1 through 28 days.

- The mean change from baseline in sweat chloride at 28 days was -32.4 mmol/L ($p < 0.05$). The mean baseline for patients in this dose group was 94.9 mmol/L.

- The mean change from baseline in CFTR-dependent chloride ion transport as measured by NPD was -10.1 mV ($p < 0.05$) after 28 days.

Placebo Dose Group: In Part 2 of the Phase 2a trial, 4 subjects received a placebo twice daily. The results for this dose group are as follows:

- The mean increase from baseline in FEV1 at 28 days was 7.0% ($p=0.13$, not statistically significant).

- The mean change from baseline in sweat chloride at 28 days in patients receiving placebo was + 4.8 mmol/L, which was not statistically significant.

- The mean change from baseline in CFTR-dependent chloride ion transport as measured by NPD was + 0.3 mV after 28 days, which was not statistically significant.

About CF and VX-770

Cystic Fibrosis (CF) is a life-threatening genetic disease that affects about 30,000 people in the United States and approximately 70,000 people worldwide. CF is caused by a genetic mutation that results in malfunctioning or reduced levels of CFTR protein on cell surfaces, which 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 of the disease. Aggressive management of CF has improved long-term outcomes in CF patients. The current median predicted age of survival for people with CF is 37

years.

VX-770, an investigational oral potentiator, is intended to increase chloride ion transport through the defective CFTR protein. As an oral CFTR modulator, VX-770 may have the potential to restore CFTR function systemically. 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 is present in approximately four 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 and subsequent clinical trials based on a collaborative research program 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 an agreement to accelerate development of VX-770. In addition, in January 2006, Vertex and CFFT entered into an expanded research collaboration for the discovery of compounds that may correct how the defective CFTR protein is processed so that it arrives at the proper location on the cell surface. The first corrector compound, VX-809, has completed two Phase 1 clinical studies in healthy volunteers. 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 $320 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 cystic fibrosis. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

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

Vertex Safe Harbor Statement

This press release may contain forward-looking statements, including statements that (i) Vertex intends to work with regulatory authorities to finalize the design of a registration program for VX-770 targeted to begin in 2009; (ii) VX-770 is a potentially new approach to the treatment of cystic fibrosis; (iii) VX-770 provides evidence that a small molecule can address the basic defect in cystic fibrosis and CFTR modulation may play an important role in cystic fibrosis therapy; (iv) results of the study reported in this press release support the hypothesis that improving chloride ion transport in cystic fibrosis patients may correlate with improvements in lung function; (v) VX-770 appeared to be well-tolerated through 28 days of dosing; (vi) VX-770 may have the potential to restore CFTR function systemically; and (vii) Vertex plans to evaluate VX-770 in patients with mutations, other than G551D, that result in malfunctioning CFTR on the cell surface. 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 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 will not reflect the results obtained in the studies to date or confirm the current hypotheses that CFTR modulation with VX-770 could be a useful cystic fibrosis therapy, that an adverse event profile for VX-770 may be revealed in further clinical studies that could put further development of VX-770 in jeopardy or adversely impact its therapeutic value, that studies of VX-770 in patients with mutations other than G551D will not be successful, 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.

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

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