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Vertex Announces Results from Phase 2a Trial of VX-809 Targeting the Defective Protein Responsible for Cystic Fibrosis

-VX-809 was well-tolerated at all dose levels when dosed once daily for 28 days-
-Statistically significant changes observed in measurement of sweat chloride suggest increased CFTR activity-
-Data support planned combination trial of VX-809 and VX-770 in second half of 2010 for CF patients with the F508del mutation-

CAMBRIDGE, Mass., Feb 03, 2010 (BUSINESS WIRE) -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced results from a preliminary analysis of data from a 28-day Phase 2a clinical trial of VX-809 in patients with cystic fibrosis (CF) who are homozygous for the F508del mutation. VX-809, an oral investigational Cystic Fibrosis Transmembrane Conductance Regulator protein (CFTR) corrector, was well-tolerated across all four dose groups studied. In the trial, VX-809 showed a statistically significant decline in sweat chloride at both the 100 mg and 200 mg once-daily doses, suggesting that the activity of the CFTR protein was increased in patients during dosing. Additionally, VX-809 demonstrated a dose response in change in sweat chloride across the four dose groups. On the basis of these results, Vertex plans to initiate a combination trial of VX-809 and VX-770, an investigational CFTR potentiator, in the second half of 2010. VX-809 and VX-770 were developed with support from Cystic Fibrosis Foundation Therapeutics, Inc., the nonprofit affiliate of the Cystic Fibrosis Foundation.

"This Phase 2a trial evaluated the potential effect of an oral compound to improve trafficking of the defective CFTR protein, and its results represent an encouraging step forward in the development of new therapies to treat the underlying cause of CF in patients with the most common *CFTR* mutation, known as F508del," said J.P. Clancy, M.D., Director of the Pediatric Pulmonary Center at the University of Alabama at Birmingham and Principal Investigator for the VX-809 Phase 2a trial. "In the trial, VX-809 was well-tolerated across the dose groups, and statistically significant changes in sweat chloride, an important biomarker of CFTR activity, were observed at certain dose levels. There is high interest in the CF community in new approaches to CF therapy, and we look forward to the future exploration of VX-809 and VX-770 as part of a novel combination regimen aimed at treating the majority of CF patients."

"While the median predicted age of survival for patients with CF has increased to more than 37 years of age, there are no approved therapies that directly target the underlying defect of this disease," said Robert J. Beall, Ph.D., President and Chief Executive Officer of the Cystic Fibrosis Foundation. "We believe that compounds such as VX-770 and VX-809 represent a promising potential approach to future CF treatment, and the results announced today for VX-809 support future clinical trials of this compound, including a planned clinical trial in combination with VX-770 expected to begin later this year."

About the Phase 2a Trial

The preliminary results reported today are from a randomized, double-blind, placebo-controlled, multiple dose Phase 2a clinical trial that enrolled 89 patients aged 18 or older who are homozygous for the F508del *CFTR* mutation. Patients in the trial received one of four doses of VX-809, or placebo, in addition to standard therapies for 28 days. The primary endpoint of the trial was to evaluate the safety and tolerability of VX-809. Multiple secondary endpoints were utilized to evaluate any effect of VX-809 on CFTR function or lung function.

Safety Evaluation

Through 28 days of 25 mg, 50 mg, 100 mg and 200 mg once-daily dosing, VX-809 was well-tolerated. In the trial, one patient discontinued treatment in each of the VX-809 treatment arms due to adverse events. Respiratory-related adverse events were the most commonly reported adverse event in the trial. Safety and tolerability were the primary endpoints of the trial, and a detailed safety analysis is ongoing.

Evaluation of CFTR Activity

At both the 100 mg and 200 mg dose levels, a statistically significant decline in sweat chloride, a secondary endpoint, was observed in analyses comparing each patient to baseline and to placebo. Additionally, a dose response for change in sweat chloride was observed across all four dose groups. The mean change in sweat chloride compared to placebo for each of the

dose groups is as follows:

Treatment Arm	Mean Change in Sweat Chloride Compared to Placebo*	P-Value
25 mg VX-809	.10 mmol/L	.9753
50 mg VX-809	-4.61 mmol/L	.1323
100 mg VX-809	-6.13 mmol/L	.0498
200 mg VX-809	-8.21 mmol/L	.0092

*Across the arms of the trial, patients' mean baseline sweat chloride measurements were approximately 100 mmol/L, which is consistent with sweat chloride measurements of patients who are homozygous for the F508del mutation.

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.

"This trial was meant to provide clinical data for VX-809 to inform the direction of future development for this compound," said Dr. Robert Kauffman, Vertex's Senior Vice President, Clinical Development and Chief Medical Officer. "Based on data generated in this Phase 2a trial, we plan to move forward with a proof-of-concept clinical trial of VX-809 dosed in combination with VX-770 in the F508del patient population, planned for the second half of 2010. We are pleased with the safety and biomarker data observed in this preliminary analysis, which, together with the Phase 2 data for VX-770, could contribute to the future treatment of CF with novel CFTR modulators."

The trial also included additional secondary endpoints, including exploratory endpoints, to evaluate CFTR function, including CFTR trafficking, and lung function. The trial was not powered to demonstrate a statistically significant effect in these additional secondary endpoints. Additional sub-analyses of the secondary endpoints are ongoing to determine any potential trends in other measures of CFTR-dependent chloride ion transport, such as nasal potential difference; or CFTR maturation, as measured by an exploratory Western blot assay, however no statistically significant changes in these measures were observed in the preliminary analysis of data from this trial. As expected, the results did not show any change in lung function, as measured by FEV1.

Vertex expects to report additional results of this Phase 2a trial at a medical meeting in 2010.

Future Development Plans for VX-809

Based on the Phase 2 data announced today, Vertex plans to initiate a combination trial of VX-809 with the CFTR potentiator VX-770 in patients with the most common *CFTR* mutation, F508del, in the second half of 2010. In *in vitro* studies, a combination regimen of VX-770 and VX-809 has been shown to increase CFTR activity when compared to dosing of VX-770 or VX-809 as single agents. In addition, Vertex is conducting further analyses of the Phase 2a data and may explore the option to evaluate VX-809 doses of greater than 200 mg when dosed as a single agent.

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-809 and VX-770

VX-809 is a novel oral CFTR corrector drug candidate aimed at increasing the concentration of F508del CFTR proteins at the cell surface. Vertex recently completed a Phase 2a trial of VX-809, as announced today.

Vertex is also developing VX-770, a novel oral CFTR potentiator drug candidate aimed at increasing the activity of defective CFTR proteins at the cell surface. Vertex is currently conducting the ENDEAVOR Phase 3 registration program of VX-770, an investigational Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) potentiator compound for the treatment of CF. The program consists of three ongoing clinical trials, known as STRIVE, ENVISION and DISCOVER, and is designed to evaluate

the utility of VX-770 across different age groups and genotypes, including children as young as six years of age.

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, epilepsy, cancer, and pain. Vertex co-discovered the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

Lexiva(R) is a registered trademark of the GlaxoSmithKline group of companies.

Special Note Regarding Forward Looking Statements

This press release contains forward-looking statements including statements regarding (i) the preliminary data from the Phase 2a clinical trial supporting a planned combination trial of VX-809 and VX-770 in the second half of 2010 for CF patients with the F508del mutation; (ii) the suggestion that the activity of the CFTR protein was increased in patients during dosing; (iii) the future exploration of VX-770 and VX-809 as part of a novel combination regimen aimed at treating the majority of CF patients; (iv) beliefs that compounds such as VX-770 and VX-809 represent a promising approach to future CF treatment and the results supporting clinical trials of VX-809 and (v) Vertex's future development plans as described under the caption "Future Development Plans for VX-809." 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-809 in combination with VX-770 may not proceed due to technical, scientific, commercial, financial or other reasons, that clinical trials may not proceed as planned, that additional clinical trials involving VX-809 may not generate data indicating that VX-809 is a useful in the treatment of cystic fibrosis, that an adverse event profile for VX-809 or VX-770 could be revealed in further nonclinical or clinical studies that could put further development of VX-809 or VX-770 in jeopardy or adversely impact the therapeutic value of VX-809 and/or VX-770, 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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