



March 29, 2011

Phase 3 Study of VX-770 in Children Ages 6 to 11 With a Specific Type of Cystic Fibrosis Showed Profound Improvements in Lung Function (FEV₁) and Other Measures of Disease Through 24 Weeks

-Mean relative improvement in lung function of 17.4% and mean absolute improvement of 12.5% from baseline compared to placebo in children treated with VX-770-

- No discontinuations due to adverse events through 24 weeks -

-Vertex on track to submit regulatory applications for the approval of VX-770 in the U.S. and Europe in the second half of 2011 -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced positive results from a 24-week analysis of the ongoing Phase 3 ENVISION study of VX-770, an oral medicine in development that targets the defective protein that causes cystic fibrosis (CF). ENVISION (n=52) was designed to evaluate VX-770 among children ages 6 to 11 with the G551D mutation in the *CFTR* gene. Approximately 4 percent of people with CF in the United States have at least one copy of the G551D mutation. The study met its primary endpoint of mean absolute change from baseline in percent predicted FEV₁ (forced expiratory volume in one second, or lung function) through week 24. A difference in mean absolute improvement from baseline in lung function of 12.5 percent and a difference in mean relative improvement from baseline in lung function of 17.4 percent compared to placebo (p<0.0001) were observed through week 24. Significant improvements in other measures of disease, including weight gain and a reduction in sweat chloride, were also observed through week 24 among those who received VX-770. At the time of this analysis, the most commonly reported adverse events were respiratory in nature and comparable across treatment groups. The study is ongoing, and additional assessments of key endpoints will be conducted at the end of the 48-week study.

Vertex is on track to submit regulatory applications for approval in the United States and Europe in the second half of 2011. The submissions will be based on data from the Phase 3 STRIVE and ENVISION studies as well as the Phase 2 DISCOVER study. Vertex plans to submit data from all studies in the VX-770 Phase 3 registration program for presentation at upcoming medical meetings.

"In this study, children with CF treated with VX-770 showed the same profound improvements in lung function seen in the recently-announced STRIVE study among an older group of people with the G551D mutation," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer for Vertex. "We are committed to making VX-770 available as soon as possible and are moving ahead quickly with our U.S. and European applications for regulatory approval that we plan to submit in the second half of this year."

"The data announced today are highly encouraging because children with CF tend to be healthier than adults and significant improvements in lung function may be harder to demonstrate in a clinical study," said Robert J. Beall, Ph.D., president and CEO of the Cystic Fibrosis Foundation. "Data from Phase 3 studies of VX-770 in people with the G551D mutation provide strong support for the concept that addressing the defective protein that leads to CF may significantly improve outcomes for patients and give hope for the future of other CF treatments that repair the basic defect of the disease."

Vertex's medicines in development for CF were discovered as part of a collaboration with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) to discover and develop novel CF Transmembrane Conductance Regulator (CFTR) modulators. CFFT is the nonprofit drug discovery and development affiliate of the Cystic Fibrosis Foundation. Vertex retains worldwide rights to develop and commercialize these potential medicines.

Summary of Key Data from ENVISION

In the ENVISION study, 52 children were enrolled and received VX-770 or placebo as a single 150 mg tablet every 12 hours. At the time of this analysis, the last patients had completed their week 40 visit. All children who remain on this study will be followed through 48 weeks. All children who complete 48 weeks of treatment in ENVISION, including those in the placebo group, and who meet eligibility criteria, may choose to receive VX-770 for up to an additional 96 weeks, or until VX-770 is approved, as part of an extension study called PERSIST.

ENVISION was designed to evaluate VX-770 in children between the ages of 6 and 11 with at least one copy of the G551D *CFTR* mutation. The primary endpoint of the study was mean absolute change from baseline in percent predicted FEV₁ through week 24. Lung function was assessed using a standard test that measures the amount of air a person can exhale in one second (forced expiratory volume in one second, or FEV₁).

Preliminary Efficacy Results

Lung Function: Absolute and relative changes in lung function are being reported in today's announcement. Phase 3 results and product labeling for currently available CF medicines generally describe relative improvements in lung function.

Baseline lung function (FEV₁) in ENVISION was 84.7 percent predicted for children in the VX-770 treatment group and 83.7 percent predicted among those in the placebo group. Results of the ENVISION study showed that the difference in mean absolute improvement from baseline in lung function through 24 weeks in children treated with VX-770 was 12.5 percent ($p < 0.0001$). The difference in mean relative improvement from baseline was 17.4 percent compared to placebo.

Additional secondary endpoints were measured to observe the effect of VX-770 through week 24. These secondary endpoints included:

Weight: Many people with CF have a hard time gaining and maintaining weight due to factors such as reduced pulmonary function, nutrition, chronic infection and inflammation. In the ENVISION study, those who received VX-770 experienced an average weight gain of approximately 8.1 lbs (3.7 kilograms) through 24 weeks compared to baseline while those in the placebo group gained approximately 3.9 lbs (1.8 kilograms) compared to baseline.

Sweat Chloride: Elevated sweat chloride levels are a diagnostic hallmark of CF and a marker of *CFTR* protein dysfunction, which is the underlying molecular mechanism responsible for CF. The amount of chloride in the sweat is measured using a standard test. People with CF typically have sweat chloride levels in excess of 60 mmol/L, while normal values are less than 40 mmol/L. Reduction in sweat chloride is considered to be a marker of improved *CFTR* function.

In ENVISION, the baseline sweat chloride level for both treatment groups was approximately 104 mmol/L. Significant decreases in measurements of sweat chloride were observed among those treated with VX-770 compared to those in the placebo group. Through week 24, mean sweat chloride level for individuals treated with VX-770 was below 60 mmol/L. Significant decreases in sweat chloride were not observed among those in the placebo group.

Patient Reported Outcomes: The Cystic Fibrosis Questionnaire — Revised (CFQ-R) is a validated patient reported outcome tool that was used in this study to measure the impact of VX-770 on overall health, daily life, perceived well-being and symptoms. One aspect of the CFQ-R, referred to as the respiratory domain, assesses patient reported symptoms such as coughing, congestion, wheezing and other respiratory symptoms. In the ENVISION study, children responded to the CFQ-R and reported a trend toward improvement in respiratory symptoms, which was a key secondary endpoint of the study.

Safety: At the time of this analysis, the most commonly reported adverse events were respiratory in nature and comparable between treatment groups. The most commonly reported adverse events through 24 weeks included cough, headache, CF pulmonary exacerbation, throat pain, and vomiting. Pulmonary exacerbations were uncommon in ENVISION regardless of treatment arm. There were no discontinuations due to adverse events in either treatment group of the study through 24 weeks. The ENVISION safety evaluation is ongoing through 48 weeks.

About the Cystic Fibrosis Transmembrane Conductance Regulator Protein (CFTR)

CF is caused by a genetic defect that results in defective or missing CF transmembrane conductance regulator (CFTR) proteins, which result in poor ion flow across cell membranes, including in the lungs, and the accumulation of abnormally thick, sticky mucus that leads to chronic lung infections and progressive lung damage. In people with the G551D mutation in the *CFTR* gene, CFTR proteins are present at the cell surface but do not function properly. VX-770, known as a CFTR potentiator, aims to increase the function of defective CFTR proteins by increasing the 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 movement of CFTR to the cell surface.

Combination Study of VX-770 and VX-809

Vertex is conducting a Phase 2a clinical trial to evaluate multiple combination regimens of VX-770 and VX-809 in people with two copies of the F508del mutation. The first part of the study is designed to evaluate VX-809 (200 mg), or placebo dosed alone for 14 days and in combination with VX-770 (150 mg or 250 mg), or placebo, for seven days. Vertex expects to obtain data from this part of the trial in the first half of 2011.

About Cystic Fibrosis

CF 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 approximately 36 years. According to the *2008 Cystic Fibrosis Foundation Patient Registry Annual Data Report*, approximately 4 percent of the total CF patient population in the U.S. have at least one copy of the G551D mutation, 48 percent of the total CF patient population in the United States have two copies of the F508del mutation and an additional 39 percent of the total CF patient population have one copy of the F508del mutation.

People 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. The CF Foundation is a donor-supported nonprofit organization.

About Vertex

Vertex creates new possibilities in medicine. Our team aims to discover, develop and commercialize innovative therapies so people with serious diseases can lead better lives.

Vertex scientists and our collaborators are working on new medicines to cure or significantly advance the treatment of hepatitis C, cystic fibrosis, epilepsy and other life-threatening diseases.

Founded more than 20 years ago in Cambridge, MA, we now have ongoing worldwide research programs and sites in the U.S., U.K. and Canada.

Special Note Regarding Forward-looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including statements regarding (i) Vertex being on track to submit U.S. and European regulatory applications for approval of VX-770 in the second half of 2011; (ii) ENVISION being ongoing and the expectation that additional assessments of key endpoints and safety analyses will be conducted at the end of the 48-week study; (iii) the plan to submit data from ENVISION, STRIVE and DISCOVER at upcoming medical meetings; (iv) Vertex's commitment to making VX-770 available as soon as possible; (v) data providing strong support for the concept that addressing the defective protein that leads to CF may significantly improve outcomes for patients and give hope for future of other CF treatments that repair the basic defect of the disease; and (vi) the expectation that data from the ongoing Phase 2a combination study of VX-770 and VX-809 will be available in the first half of 2011. While Vertex believes the forward-looking statements contained in this press release are accurate, there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, that Vertex could experience unforeseen delays, that future outcomes from ENVISION, which is still ongoing, and from the various extension studies of VX-770 may not be favorable or may be less favorable than observed to date, that unexpected side effects may appear as VX-770 is more broadly dosed, that regulatory authorities may require more extensive data for VX-770 regulatory filings than currently expected; that future clinical, competitive and other factors may adversely affect the potential for VX-770; that the company may not be able to successfully develop VX-770 or combination therapies involving VX-770 and VX-809, 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.

(VRTX-GEN)

Vertex Contacts:

Investors

Michael Partridge, 617-444-6108

or

Lora Pike, 617-444-6755

or

Matthew Osborne, 617-444-6057

or

Media

Dawn Kalmar or Zachry Barber, 617-444-6992

Source: Vertex Pharmaceuticals

News Provided by Acquire Media