Phase 3 STRIVE Study of VX-770 Showed Durable Improvements in Lung Function (FEV1) and Other Measures of Disease Among People With a Specific Type of Cystic Fibrosis

- Complete data presented at ECFS showed improvements in lung function and reductions in sweat chloride observed at week two of treatment were sustained through 48 weeks -

- Vertex also announces that 48-week data from the ENVISION study of VX-770 among children ages 6 to 11 years are consistent with previously announced 24-week results -

HAMBURG, Germany--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq:VRTX - News) today announced the final results from its pivotal Phase 3 STRIVE study that evaluated VX-770, a medicine in development that targets the defective protein that causes cystic fibrosis (CF). STRIVE was designed to evaluate VX-770 among 161 people 12 years or older with a mutation known as G551D in the CF gene. Approximately 4 percent of people with CF have at least one copy of the G551D mutation.

Data from the study showed rapid improvements in lung function (FEV_1) that were sustained through 48 weeks among those who received VX-770, compared to those treated with a placebo. Significant improvements in all key secondary endpoints were observed among people who received VX-770 compared to placebo. Adverse events that occurred more frequently among those treated with VX-770 compared to placebo were headache, upper respiratory tract infections, nasal congestion, rash, dizziness and bacteria in the sputum. These data were presented today at the 34th European Cystic Fibrosis Society (ECFS) Conference in Hamburg, Germany.

The results of STRIVE showed a mean absolute improvement in lung function of 10.6 percent through week 24 (primary study endpoint) and 10.5 percent through week 48 (secondary study endpoint) among those treated with VX-770 (n=83). The mean relative improvement from baseline in lung function among people treated with VX-770 compared to placebo (n=78) was 16.9 percent through week 48. 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.

"Improving lung function is vitally important in the treatment of cystic fibrosis, but the disease affects many organs in a person's body," said Stuart Elborn, M.D., Belfast City Hospital, Ireland, UK, and one of the study's lead investigators. "These data are significant because they are the first to show that treating the underlying cause of cystic fibrosis may have profound effects on the disease, even among people who have been living with it for decades. The remarkable reductions in sweat chloride observed in this study support the idea that VX-770 improves CFTR function thereby addressing the fundamental defect that leads to CF."

"In this study, people treated with VX-770 experienced significant improvements in lung function and reductions in sweat chloride within the first two weeks of treatment, and these benefits were sustained through the nearly year-long study," said Robert Kauffman, M.D., Ph.D., Senior Vice President and Chief Medical Officer for Vertex. "Given the significance of these findings, we are moving quickly to submit regulatory applications for approval in the United States and Europe in the second half of 2011."

Adverse events that were 5 percent or greater among those treated with VX-770 compared to placebo were headache, upper respiratory tract infections, nasal congestion, rash, dizziness and bacteria in the sputum. The most commonly reported serious adverse events included pulmonary exacerbation (13 percent in the VX-770 group compared to 33 percent in the placebo group), hemoptysis (or bloody cough; 1 percent in the VX-770 group and 5 percent in the placebo group) and hypoglycemia (2 percent in the VX-770 group and zero in the placebo group). Discontinuations through 48 weeks due to adverse events were less frequent in the VX-770 treatment group compared to placebo (1 percent compared to 5 percent).

Summary of Additional Key Data from STRIVE

Lung Function: The STRIVE study met its primary endpoint of mean absolute change from baseline compared to placebo in percent predicted FEV_1 (lung function) through week 24. Data from the study showed a mean absolute improvement in lung function from baseline compared to placebo through week 24 of 10.6 percent among those treated with VX-770.

Significant improvements in lung function were observed at the time of the first post-treatment assessments at two weeks.

These improvements were sustained through 48 weeks.

Significant improvements in key secondary endpoints in this study were also reported through week 48 of the STRIVE study.

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 STRIVE study, those who received VX-770 gained weight throughout the study and experienced an average weight gain of approximately seven pounds (3.1 kilograms) at week 48 compared to baseline. Patients in the placebo group gained approximately 0.9 pounds (0.4 kilograms) at week 48 compared to baseline.

Pulmonary Exacerbation: Pulmonary exacerbations are periods of worsening in signs and symptoms of the disease requiring treatment with antibiotics. In STRIVE, people treated with VX-770 were 55 percent less likely to experience a pulmonary exacerbation compared to those treated with the placebo. Through week 48, 67 percent of patients who received VX-770 had not experienced a pulmonary exacerbation compared to 41 percent treated with the placebo.

Sweat Chloride: The amount of chloride in the sweat is measured using a standard test. Elevated sweat chloride is a diagnostic characteristic of CF, and sweat chloride is a marker of CFTR protein dysfunction. A reduction in sweat chloride is considered to be a marker of improved CFTR function.

People with CF typically have elevated sweat chloride levels in excess of 60 mmol/L, while normal values are less than 40 mmol/L.

In this study, people who received VX-770 experienced a significant and rapid reduction in the amount of salt in their sweat (sweat chloride concentration). By the first post-treatment assessment at week two of the study, patients treated with VX-770 experienced an average reduction in sweat chloride of approximately 45 mmol/L. The decreases in sweat chloride among these patients were maintained through week 48. Patients treated with the placebo maintained baseline sweat chloride levels of approximately 100 mmol/L through 48 weeks. The mean absolute improvement in sweat chloride among those treated with VX-770 was -48.1 mmol/L through week 48 ($p < 0.0001$).

Patient Reported Outcomes: The Cystic Fibrosis Questionnaire – Revised (CFQ-R) is a validated patient-reported outcomes tool that was used in the STRIVE study to measure the impact of VX-770 on study participants' overall health, daily life, perceived well-being and symptoms. A ≥4-point change from baseline in CFQ-R is considered clinically meaningful.

One aspect of the CFQ-R, referred to as the respiratory domain, addresses patient reported symptoms, including coughing, congestion, wheezing and other respiratory symptoms. In this study, people who received VX-770 reported improvements in respiratory symptoms compared to those treated with a placebo. Through 48 weeks, there was a statistically significant 8.6 point difference in the responses between the two treatment groups. Among those treated with VX-770, there was a 5.9 point change from baseline compared to a -2.7 change from baseline among those in the placebo group.

Vertex Plans Expanded Access Programs for VX-770

VX-770 is a medicine in development for people with cystic fibrosis who are 6 years or older and have at least one copy of the G551D mutation. Vertex is on track to submit global regulatory applications for approval in the United States, Canada and Europe, including a New Drug Application (NDA) in the United States and a Marketing Authorization Application (MAA) in the European Union in the second half of 2011.

In recognition of the immediate needs of some people with CF, Vertex is planning an expanded access program for VX-770. This program is designed to provide VX-770 to people who have at least one copy of the G551D mutation who are in critical medical need and who may benefit from treatment prior to potential FDA approval in the United States. Vertex expects to open the expanded access program at clinical trial sites in the United States as early as July, pending FDA review and approval.

Vertex is working with regulatory authorities outside of the United States toward implementing additional expanded access programs in other countries, with a goal of opening programs for eligible patients in the second half of 2011.

For more information, please call Vertex Medical Information at 1- 877-634-VRTX (8789).

About STRIVE

STRIVE evaluated 161 patients 12 years or older who received at least one dose of either VX-770 as a single 150 mg tablet (n=83) or placebo (n=78) twice daily. The study was designed to evaluate VX-770 in people with at least one copy of the G551D CFTR mutation. The primary endpoint of the study was mean absolute change from baseline in predicted FEV_1 (lung function) 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).

VX-770 Registration Program

VX-770 is Vertex's lead medicine in development for the treatment of people with cystic fibrosis. Three studies are included in the registration program for VX-770: ENVISION, STRIVE and DISCOVER. All three studies are now complete and will form the basis of global regulatory applications for approval.

ENVISION

In March 2011, Vertex announced positive preliminary 24-week results from the Phase 3 ENVISION study (n=52) that was designed to evaluate VX-770 in children with CF between the ages of 6 years and 11 years old who have at least one copy of the G551D mutation.

Significant improvements in lung function and other measures of disease, including weight gain and a reduction in sweat chloride were observed among people treated with VX-770 compared to placebo with 24 weeks of treatment. Today, Vertex announced that the results with 48 weeks of treatment were consistent with the 24-week results.

No new safety concerns were identified with VX-770 through 48 weeks. The most commonly reported serious and non-serious adverse events were respiratory in nature and occurred with comparable frequency in the VX-770 and placebo treatment groups.

All patients who completed 48 weeks of treatment in ENVISION, including those in the placebo group, were eligible to receive VX-770 as part of an extension study called PERSIST. All patients who completed dosing in the VX-770 arm (n=26) and were eligible and all patients who completed dosing in the placebo arm (n=23) and were eligible chose to enroll in the extension study and receive VX-770 for up to an additional 96 weeks or until VX-770 is approved. Three patients in the placebo group discontinued treatment prior to the end of the study and were not eligible for enrollment in PERSIST.

Complete data from the ENVISION study will be submitted for presentation at a medical meeting in the second half of 2011.

DISCOVER

The Phase 2 DISCOVER study (n=140) was primarily designed to provide additional safety data for VX-770 as part of the registration program. DISCOVER enrolled people 12 years and older with two copies of the F508del mutation, which prevents the CFTR protein from moving to its proper location at the cell surface. The majority of people with CF have at least one copy of the F508del mutation. All patients, including those in the placebo group, who completed study treatment in DISCOVER and met other criteria, were eligible to receive VX-770 as part of an extension study called PERSIST. Study investigators were notified in May 2011 to discontinue treating patients from DISCOVER who had enrolled in PERSIST because the improvements in lung function observed in DISCOVER were not considered clinically meaningful. Final data from this study were presented yesterday at the 34th European Cystic Fibrosis Conference in Hamburg, Germany.

About the Cystic Fibrosis Transmembrane Conductance Regulator Protein (CFTR)

CF is caused by a genetic defect that results in defective or missing CFTR proteins, which result in poor ion flow across cell membranes, including in the lungs, and the accumulation of abnormally thick, sticky mucus that lead 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, another CF medicine in development by Vertex and known as a CFTR corrector, aims to increase CFTR function by increasing the movement of CFTR to the cell surface.

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 United States 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. From 2000 through 2006, Vertex and CFFT amended and expanded the collaboration four times to support the accelerated discovery and development of VX-770 and VX-809. In April 2011, Vertex and CFFT further expanded the collaboration to support development activities for VX-661, Vertex's second corrector to enter clinical development, and the discovery and development of next-generation correctors. As part of these collaborations, Vertex has received approximately $75 million from CFFT to support CF research and development efforts led by Vertex.

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 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 moving quickly to submit regulatory applications for approval in the United States and Europe and the expectation that these submissions will be made in the second half of 2011; (ii) the expectation that the expanded access program will open at clinical trial sites in the United States as early as July 2011; (iii) the goal of opening additional expanded access programs in other countries in the second half of 2011; (iv) the expectation that complete data from ENVISION will be submitted for presentation at a medical meeting in the second half of 2011; and (v) VX-809 aiming to increase CFTR function by increasing the movement of CFTR to the cell surface. 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 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 and/or 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.

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