October 3, 2011

Vertex Announces Presentation of New Data on VX-770 and VX-809 at North American Cystic Fibrosis Conference

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that nine abstracts from its cystic fibrosis (CF) research program, which is aimed at discovering and developing medicines that target the underlying cause of CF, will be presented at the 25th Annual North American Cystic Fibrosis Conference (NACFC) in Anaheim, Calif., November 3-5, 2011. Presentations will include data from studies of Vertex's medicines in development for the treatment of CF, VX-770 alone and in combination with VX-809.

Complete 48-week data from the Phase 3 ENVISION study will be presented for the first time. ENVISION evaluated VX-770 in children ages 6 to 11 years with at least one copy of the G551D mutation in the CFTR gene. Also being presented for the first time are data from a subset of patients in the open-label PERSIST extension study. This rollover study enrolled patients who had completed 48 weeks of treatment (placebo or VX-770) in one of the VX-770 Phase 3 trials and who met certain additional criteria. Data from the first 12 weeks of the rollover study will be presented at the meeting.

In addition, complete data from the first part of a Phase 2 study evaluating multiple combinations of VX-770 and VX-809 in people with two copies of the most common mutation in the CFTR gene, F508del, will be presented for the first time. Topline data from this study were announced in June 2011.

"Emerging data from these studies further support the idea that targeting the underlying cause of CF could potentially improve outcomes for people with cystic fibrosis," said Peter Mueller, Ph.D., Chief Scientific Officer and Executive Vice President of Global Research and Development at Vertex. "We continue to make progress in developing potential new treatments for CF that target the underlying cause of the disease, with the goal of improving the lives of people with CF."

The accepted abstracts are now available in *Pediatric Pulmonology: Special Issue: The 25th Annual North American Cystic Fibrosis Conference, Anaheim Convention Center, Anaheim, California, November 3—5, 2011* which is available online: http://onlinelibrary.wiley.com/doi/10.1002/ppul.v46.34s/issuetoc.

VX-770 Abstracts *(Oral presentations will also be presented as posters.)*

1. **Study 102 (STRIVE):** "Efficacy and Safety of VX-770 in Subjects with Cystic Fibrosis and the *G551D-CFTR* Mutation." November 5, 2011, 3:35 p.m. PDT (oral). Poster #211.
2. **Study 103 (ENVISION):** "VX-770 in Subjects 6 to 11 Years with Cystic Fibrosis and the *G551D-CFTR* Mutation." Poster #203.
3. **Study 104 (DISCOVER):** "VX-770 in Subjects with CF and Homozygous for the *F508DEL-CFTR* Mutation." Poster #206.
4. **Study 105 (PERSIST):** "Long-Term Safety and Efficacy of Investigational CFTR Potentiator, VX-770, in Subjects with CF." Poster #204.
5. **Study 106**: "Lung Clearance Index as an Outcome Measure in Cystic Fibrosis Clinical Trials." November 3, 2011, 11:20 a.m. PDT (oral). Poster #201.
6. **Study 107:** "Hyperpolarized Helium-3 Magnetic Resonance Imaging of CFTR Potentiator Therapy in Subjects with Cystic Fibrosis and the G551D Mutation." Poster #205.
7. "VX-770 Potentiation of CFTR Forms with Channel Gating Defects In Vitro." November 4, 2011, 3:20 p.m. PDT (oral). Poster #10.
8. "Variability of Nasal Potential Difference Measurements in Clinical Testing of CFTR Modulators." Poster #202.

VX-770/VX-809 Abstract

1. "VX-809, an Investigational CFTR Corrector, in Combination with VX-770, an Investigational CFTR Potentiator, in Subjects with CF and Homozygous for the F508DEL-CFTR Mutation." November 5, 2011, 3:50 p.m. PDT (oral). Poster #212.

ABOUT VX-770

VX-770 is Vertex's lead medicine in development for the treatment of people with cystic fibrosis. VX-770, known as a cystic fibrosis transmembrane conductance regulator (CFTR) potentiator, aims to help the protein function more normally once it reaches the cell surface. In October, Vertex plans to submit both its VX-770 New Drug Application (NDA) in the United States and its VX-770 Marketing Authorization Application (MAA) to the European Medicines Agency (EMA). Additional regulatory submissions are planned for Canada and other countries following the submissions of the NDA and MAA. Vertex is seeking approval of VX-770 in people six years of age and older who have at least one copy of the G551D mutation in the *CFTR* gene. Three studies are included in the registration program for VX-770; two Phase 3 trials, ENVISION and STRIVE, and one Phase 2 trial, DISCOVER. All three studies are now complete and will form the basis of global regulatory applications for approval.

ABOUT VX-809

VX-809 is an investigational oral cystic fibrosis transmembrane conductance regulator (CFTR) modulator for the treatment of cystic fibrosis. VX-809, known as a CFTR corrector, is designed to help the protein reach the cell surface. Vertex is also conducting a Phase 2 clinical trial to evaluate combination regimens of VX-770 and VX-809 in people with two copies of the most common mutation in the CFTR gene, F508del.

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. 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 $80 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. For more information, visit www.cff.org.

About Vertex

Vertex creates new possibilities in medicine. Our team discovers, develops and commercializes 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, Mass., 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) the aim of Vertex's CF research program; (ii) emerging data from the studies referred to in this press release further supporting the idea that targeting the underlying cause of CF could potentially improve outcomes for

people with CF; (iii) the progress Vertex is continuing to make in developing potential new treatments for CF that target the underlying cause of the disease; (iv) Vertex's goal of improving the lives of people with CF and (v) Vertex's plan to submit an NDA and MAA for VX-770 in October 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 future clinical, competitive and other factors may delay regulatory submissions for VX-770 or adversely affect the potential for VX-770; that the company may not be able to successfully develop new treatments for CF; 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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Source: Vertex Pharmaceuticals Incorporated