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Vertex and Cystic Fibrosis Foundation Therapeutics to Collaborate on Discovery and Development of New Medicines to Treat the Underlying Cause of Cystic Fibrosis

-Expanded collaboration supports development of a second corrector, VX-661, and accelerated discovery and development of next-generation correctors-

-Phase 2 study of VX-661 planned for 2011 in people with CF who have the F508del mutation-

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) today announced they will collaborate on the continued discovery and development of new medicines known as correctors that aim to treat the underlying cause of cystic fibrosis (CF) in people with the most common form of the disease. The expanded collaboration will support development activities for VX-661, Vertex's second corrector to enter clinical development, and the accelerated discovery and development of next-generation correctors. Vertex plans to begin the first study of VX-661 by the end of 2011 in people with CF who have the F508del mutation.

Vertex and CFFT, a nonprofit drug discovery and development affiliate of the CF Foundation, began their collaborative research and development efforts in 1998, and to date, three potential new medicines, known as CFTR modulators, have resulted from the collaboration — the potentiator VX-770 and the correctors VX-809 and VX-661. Correctors and potentiators are medicines in development that aim to treat the underlying cause of CF by improving the function of the defective protein known to cause the disease. Vertex retains worldwide rights to develop and commercialize these potential medicines.

"The CF Foundation is widely recognized by doctors, nurses, scientists and those with CF as a driving force in the search for new CF medicines, and we are pleased to further expand our strong collaboration with them," said Matthew Emmens, Chairman, President and Chief Executive Officer of Vertex. "The collaboration announced today underscores our commitment to CF and accelerates our efforts to develop new medicines as quickly as possible for people with the most common type of this disease. By advancing VX-809 and VX-661 in parallel, we hope to generate data to inform future studies of corrector regimens while continuing to invest in additional research for CF."

"With the recently announced positive Phase 3 results for VX-770, we believe that - together with Vertex - we are on the right path to fundamentally change the treatment of CF by targeting the cause of the disease," said Robert J. Beall, Ph.D., President and CEO of the CF Foundation and CFFT. "This new collaboration is a milestone in our long-standing relationship with Vertex and provides for additional opportunities to accelerate our discovery efforts and to potentially improve treatment for people with the most common type of CF."

CF is caused by defective or missing CF transmembrane conductance regulator (CFTR) proteins, which result in poor ion flow across cell membranes, including in the lungs, causing the accumulation of abnormally thick, sticky mucus that leads to chronic lung infections and progressive lung damage. In people with the F508del mutation, which is the most common *CFTR* mutation, CFTR proteins do not reach the cell surface in normal amounts. VX-809 and VX-661, known as CFTR correctors, aim to increase CFTR function by increasing the movement of CFTR to the cell surface. 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 their ability to transport ions across the cell membrane of CFTR at the cell surface.

About The Collaboration

The collaboration announced today is focused on development activities for VX-661 and on the accelerated discovery and development of additional correctors for the treatment of people with the F508del mutation. As part of the collaboration, CFFT will provide Vertex with up to $75 million to support research and development activities. Vertex expects to receive payments from CFFT as reimbursement for research and development activities over a period of five years beginning in 2011.

The collaboration will help support the development of VX-661. Vertex intends to begin a Phase 2 study of VX-661 by the end of 2011 and expects the study to enroll people with CF who have the F508del mutation. The collaboration will also help support the accelerated discovery and early development of next-generation correctors that aim to treat the underlying cause of CF in people with the F508del mutation. The majority of Vertex's CF research is conducted at Vertex's San Diego, CA research site, where each of Vertex's medicines in development for CF were discovered. Development activities for Vertex's CF medicines are

being led by Vertex's research and development site in Cambridge, MA.

Under the terms of the collaboration, CFFT is entitled to receive a royalty on future net sales of correctors developed as part of the research collaboration. As part of previous collaborations, CFFT is entitled to receive a royalty on future net sales of VX-770, VX-809 and VX-661. Vertex retains worldwide rights to VX-770, VX-809 and VX-661.

Development Program for Vertex's CF Medicines

Vertex recently reported positive results from two Phase 3 studies of VX-770 — the STRIVE trial in adolescents and adults with CF and the ENVISION trial in children ages 6 to 11. The Phase 3 program for VX-770 is focused on people with CF who have at least one copy of the G551D mutation. The results from the Phase 3 program will form the basis for Vertex's planned submission of applications for approval in the United States and Europe in the second half of 2011.

Vertex is also conducting a Phase 2 clinical trial to evaluate different dose combinations of VX-809 and VX-770 in people with two copies of the F508del mutation. The first part of the study is designed to evaluate VX-809, or placebo, dosed alone for 14 days and in combination with VX-770, or placebo, for seven days. Vertex expects to obtain data from Part One of the trial in the first half of 2011.

VX-661 is the third potential new medicine for CF to emerge from Vertex's CF research efforts. As a corrector, VX-661 aims to increase CFTR function by increasing the movement of CFTR to the cell surface. In *in vitro* studies, a combination of VX-661 and VX-770 resulted in greater CFTR activity, as compared to treatment with VX-661 alone. Vertex plans to initiate a Phase 2 study of VX-661 by the end 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 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. 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. As part of these collaborations, Vertex has received approximately $75 million from CFFT to date 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. The CF Foundation is a donor-supported nonprofit organization. For more information, go to www.cff.org.

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 and CFFT collaborating on the discovery and development of new medicines to treat the underlying cause of CF; (ii) the expanded collaboration supporting development of a second corrector and accelerated discovery and development of next-generation correctors; (iii) the plan to initiate a Phase 2 study of VX-661 by the end of 2011 in people with the F508del mutation; (iv) Vertex's commitment to CF and the acceleration of efforts to develop new medicines as quickly as possible for people with the most common type of CF; (v) the advancement of VX-809 and VX-661 in parallel potentially generating data to inform future studies of corrector regimens; (vi) Vertex's plan to continue to invest in additional research for CF; (vii) the belief that Vertex and CFFT are on the right path to fundamentally change the treatment of CF by targeting the cause of the disease; (viii) the collaboration providing additional opportunities to accelerate our discovery efforts and to potentially improve treatment for people with the most common type of CF; (ix) Vertex's expectation that CFFT will provide up to $75 million to support research and development activities over a period of five years beginning in 2011; (x) Vertex's planned submissions of applications for approval of VX-770 in the United States and Europe in the second half of 2011; and (xi) the expectation that Vertex will obtain data from Part One of a combination study of VX-809 and VX-770 in the second quarter 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 ongoing clinical studies of VX-770, VX-809 and VX-661 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, VX-809 or VX-661; that the company may not be able to successfully develop VX-770, VX-809 or VX-661 or combination therapies involving these compounds, that additional research directed at identifying additional correctors may not result in additional drug candidates 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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