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# Vertex Pharmaceuticals Initiates Phase I Development for VX-770 in Cystic Fibrosis

## - FDA Grants Fast Track Designation to VX-770 -

**Cambridge, MA, May 17, 2006**-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that it has initiated a Phase I clinical study for VX-770, a novel, oral drug candidate that specifically targets a key mechanism underlying cystic fibrosis (CF). The study will evaluate the safety, tolerability and pharmacokinetics of escalating single and multiple doses of VX-770 in healthy volunteers, and also will evaluate single doses of VX-770 in patients with CF. The study is expected to enroll more than 50 individuals. In March 2006, Vertex and Cystic Fibrosis Foundation Therapeutics Inc. (CFFT) entered into a collaboration to accelerate clinical development of VX-770. CFFT is the nonprofit drug discovery and development affiliate of the Cystic Fibrosis Foundation. Vertex retains worldwide rights to develop and commercialize VX-770.

"This first clinical study for VX-770 signifies an important milestone in the productive collaborative history that we have shared with Vertex in the discovery of novel CF therapies," said Robert J. Beall, Ph.D., President and Chief Executive Officer of the Cystic Fibrosis Foundation and CFFT. "We believe that compounds such as VX-770 have great potential to change the course of CF, and we are pleased to support the accelerated development of VX-770 in early clinical studies."

"VX-770 is the first drug candidate to have emerged from our innovative CF research efforts, and the initiation of this Phase I study represents an exciting new stage in the development of this compound," said John Alam, M.D., Executive Vice President, Medicines Development, and Chief Medical Officer of Vertex. "Laboratory results for VX-770 have been highly encouraging and support the initiation of this first clinical study. We look forward to evaluating VX-770 in both healthy volunteers and patients with CF in the coming months to determine the next steps for the VX-770 development program."

**Study Design**
The Phase I study for VX-770 announced today is expected to enroll more than 50 individuals, including healthy volunteers and patients with CF. Dosing has been initiated in the first cohort of healthy volunteers, and is expected to progress to patients with CF later this year. Healthy volunteers in the Phase I study will receive escalating doses of VX-770 for treatment durations of up to 14 days, and patients with CF will receive single doses of VX-770.

**Fast Track Designation for VX-770**
Vertex also today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to VX-770. The FDA granted Fast Track designation to VX-770 for the following reasons:

- Cystic fibrosis (CF) is a serious and life-threatening illness in which mucus plugging, infection, and inflammation in the lungs lead to a decline in pulmonary function and significant morbidity and mortality.
- VX-770 is intended to preserve pulmonary function, decrease morbidity, and prolong survival in patients with CF by decreasing cycles of mucus plugging, infection, and inflammation in the lungs.
- Under the FDA Modernization Act of 1997, Fast Track designation indicates that the FDA will facilitate the development and may expedite the review of a drug if it is intended for the treatment of a serious or life-threatening condition and demonstrates the potential to address an unmet medical need for such a condition.

**About VX-770**
VX-770 was advanced into preclinical development based on a successful research collaboration with CFFT that incorporated capabilities and proprietary research from Vertex's San Diego research site. VX-770 may act to restore the function of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, the defective cell membrane protein responsible for the progression of CF. Defects in the CFTR protein affect the transport of chloride and other ions across cells, and lead to the accumulation of thick, sticky mucus in the lungs of patients with CF. This mucus fosters chronic infection and inflammation, and results in irreversible lung damage. Potentiator compounds such as VX-770 are designed to increase the probability that the CFTR channel is open, which could result in an increase in chloride transport across the cell surface in some patients. In laboratory experiments, using cells from patients with CF where CFTR proteins are present on the cell surface, VX-770 has restored the gating activity of defective CFTR channels.

**Collaborative History with CFFT**
Vertex initiated its CF research program in May 2000 in collaboration with CFFT, which offers special expertise and experience in CF drug discovery and development. Vertex and CFFT expanded the agreement in May 2004, and in March 2006, entered

into a new collaboration for the excelerated development of VX-770. Under the collaboration, CFFT will provide to Vertex approximately $13.3 million to support clinical development of VX-770 through the fourth quarter of 2007. In addition to the development collaboration for VX-770, in January 2006, Vertex and CFFT entered into an expanded research collaboration to discover novel compounds known as correctors, which may work by increasing the number of CFTR channels on the cell surface. To date, CFFT has provided to Vertex more than $40 million for CF research.

**About Cystic Fibrosis and the Cystic Fibrosis Foundation**
Cystic fibrosis is a genetic disease affecting approximately 30,000 people in the United States. A defect in the CFTR gene causes the body to produce abnormally thick, sticky mucus that leads to chronic, life-threatening lung infections and impairs digestion. When the CF Foundation was established in 1955, few children lived to attend elementary school. Today, because of research and care supported by the CF Foundation-with money raised through donations from families, corporations and foundations-the median predicted age of survival for people with CF is now more than 36 years.

The Cystic Fibrosis Foundation, headquartered in Bethesda, MD, is a donor-supported, nonprofit organization committed to finding therapies and ultimately a cure for CF, and to improving the lives of those with the disease. For more information on CF and the programs of the CF Foundation, call (800) FIGHT CF or visit www.cff.org.

**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 principally focused on viral diseases, inflammation, autoimmune diseases and cancer. Vertex co-promotes the HIV protease inhibitor, Lexiva, with GlaxoSmithKline.

Lexiva is a registered trademark of the GlaxoSmithKline group of companies.

**Vertex Safe Harbor Statement**
This press release may contain forward-looking statements, including statements that Vertex expects (i) that the Phase I clinical study for VX-770 is expected to enroll more than 50 individuals; (ii) that the study will evaluate the safety, tolerability and pharmacokinetics of escalating single and multiple doses of VX-770 in healthy volunteers and single doses of VX-770 in patients with CF; (iii) that compounds such as VX-770 have the potential to change the course of CF; (iv) that it will evaluate VX-770 in the coming months to determine the next steps for the VX-770 development program; (v) to begin dosing VX-770 in patients with CF later this year; and (vi) VX-770 may act to restore the function of the cystic fibrosis transmembrane conductance regulator (CFTR) protein. While management makes its best efforts to be accurate in making forward-looking statements, such statements are subject to risks and uncertainties that could cause Vertex's actual results to vary materially. These risks and uncertainties include, among other things, the possibility that CFFT could terminate its financial support under its agreements with Vertex early, risks that efforts to develop VX-770 may not proceed due to financial, technical, scientific, commercial or other reasons, that clinical trials may not proceed as planned due to technical, scientific, supply or patient enrollment issues, that actual clinical studies of VX-770 will not reflect the results obtained in pre-clinical and nonclinical testing, and other risks listed under Risk Factors in Vertex's Form 10-K filed with the Securities and Exchange Commission on March 16, 2006.

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