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# Vertex Pharmaceuticals and Cystic Fibrosis Foundation Therapeutics Extend Collaboration for Cystic Fibrosis Drug Research

Cambridge, MA and Bethesda, MD, January 12, 2006- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT) today announced that they have extended their research collaboration. Under the extended agreement, CFFT will provide an additional \$22 million to Vertex for continued research funding through early 2008 to further develop Vertex's proprietary "corrector" compounds that act to restore the function of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, the defective cell membrane protein responsible for the progression of cystic fibrosis (CF). CFFT is the nonprofit drug discovery and development affiliate of the Cystic Fibrosis Foundation.

"The expansion of this collaboration with Vertex underscores our belief that the development of small molecule agents to address the underlying defect of cystic fibrosis is our highest priority and has the greatest potential to fundamentally change the treatment of the disease," said Robert J. Beall, Ph.D., President and CEO of the CF Foundation and CFFT. "CFFT and Vertex share a long-standing and fruitful collaborative history in CF research. Together, we look forward to the discovery and development of novel treatments that may provide new therapeutic options for thousands of CF patients."

"Vertex's cystic fibrosis research program has yielded two innovative approaches for the treatment of this disease," said Joshua Boger, Ph.D., Chairman, President and CEO of Vertex. "This research extension of the CFFT collaboration underscores our commitment to developing new treatments for this major unmet medical need and supports Vertex's continued investment focus in this key therapeutic area."

## **CF Drug Discovery**

Using proprietary expertise in ion channels, including high-content cell assays and medicinal chemistry, Vertex has identified selective ion channel modulators for the treatment of CF. With the support of CFFT, Vertex's CF research has focused on two possible alternative approaches to CF treatment, known as "potentiator" and "corrector" approaches. Each approach might address a different molecular defect in the CFTR protein that is responsible for 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. This mucus fosters chronic infection and inflammation, and ultimately results in irreversible lung damage

Potentiator compounds may work by increasing the probability that the CFTR channel is open, which could result in an increase in chloride transport across the cell surface. Corrector compounds may work by increasing the number of CFTR channels on the cell surface. At the 16th Annual Williamsburg Conference in June 2004, Vertex researchers demonstrated the potential of both corrector and potentiator compounds to improve CFTR function in vitro in bronchial epithelial cells isolated from CF patients.

## **CFFT Collaboration**

Vertex initiated its CF research program in May 2000 as part of a collaboration with CFFT, and expanded the agreement in May 2004. Under the extended agreement announced today, CFFT will provide to Vertex approximately \$22 million in additional contracted payments for corrector research through the first quarter of 2008, with the first payment to be received in the first quarter of 2006. Vertex retains the right to develop and commercialize any compounds discovered under the agreement, and will pay CFFT royalties on net sales.

## **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 in the mid-30s.

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 (i) CFFT will provide an additional \$22 million to Vertex for continued research funding through early 2008; (ii) small molecule agents have the potential to fundamentally change the treatment of the disease; and (iii) novel CF treatments discovered under this collaboration may provide new therapeutic options for CF patients. 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 the CFFT could terminate its financial support under the agreement without cause, risks that efforts to select and optimize development candidates may not proceed due to financial, technical, commercial or other reasons, that laboratory results may not be predictive of future clinical results for any compounds selected for human clinical study and other risks listed under Risk Factors in Vertex's Form 10-K filed with the Securities and Exchange Commission on March 16, 2005.

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