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Vertex Submits Application for Priority Review and Approval of KALYDECO™ (VX-770, ivacaftor) in the U.S. as First Potential Medicine to Target the Underlying Cause of Cystic Fibrosis

-- Accelerated assessment of KALYDECO granted by the European Medicines Agency; MAA submission planned in EU by the end of October --

-- Phase 3 registration studies of KALYDECO showed significant improvements in lung function and other measures of disease among a subset of people with CF --

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced the submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for KALYDECO™ (VX-770, ivacaftor), a medicine in development that targets the defective protein that causes cystic fibrosis (CF). KALYDECO (kuh-LYE-deh-koh) was studied among people with CF ages 6 and older who have at least one copy of the G551D mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. In the United States, approximately 4 percent of people with CF are estimated to have at least one copy of the G551D mutation in the *CFTR* gene.

Global Phase 3 pivotal studies of KALYDECO showed significant and sustained improvements in lung function and other measures of disease in people with CF who had at least one copy of the G551D mutation. The majority of adverse events associated with KALYDECO were mild to moderate in severity and non-serious. Fewer people in the KALYDECO treatment groups than in the placebo groups discontinued treatment due to adverse events. If approved, KALYDECO will be the first treatment to target the underlying cause of CF.

The U.S. submission includes a request for Priority Review, which, if granted, would shorten the FDA's anticipated review time from 10 to six months. The FDA grants Priority Review status for several reasons, including if the medicine is considered a major advance in treatment. Vertex also plans to submit a marketing authorization application (MAA) for KALYDECO with the European Medicines Agency (EMA) by the end of October 2011. The EMA has accepted Vertex's request for accelerated assessment, which is granted to new medicines of major public health interest and shortens the review time from 210 days to 150 days following the start of the review. Additionally, Vertex submitted requests to the FDA and EMA to use the trade name KALYDECO (ivacaftor) for VX-770.

"KALYDECO represents a completely new approach to the treatment of CF by targeting the underlying cause of the disease," said Matthew Emmens, Chairman, President and Chief Executive Officer of Vertex. "This is our second new drug application in less than a year, which is a significant achievement and underscores our commitment to developing new medicines for people with serious diseases."

"The CF Foundation is thrilled that our collaboration with Vertex has contributed to an important potential new medicine for the treatment of some people with CF," said Robert J. Beall, Ph.D., President and CEO of the CF Foundation. "The results of the KALYDECO studies have opened the door to a new way of treating CF and we hope that this approach will lead to the development of other targeted medicines for all people with this disease."

CF is a life-threatening genetic disease that is caused by mutations in the *CFTR* gene that result in defective or missing CFTR proteins. The absence of functional CFTR proteins results in poor flow of salt and water across cell membranes in a number of organs, including the lungs. This leads to the buildup of abnormally thick, sticky mucus that can cause chronic lung infections and progressive lung damage. Currently available medicines have helped improve treatment and care for people living with CF by treating the symptoms and some of the complications of the disease.

Various mutations in the *CFTR* gene lead to CF. In some people, CFTR proteins are present at the cell surface but do not function properly. This dysfunction is known as a gating defect, the most common of which is the G551D mutation. Approximately 4 percent of those with CF, or about 1,200 people in the United States, are believed to have the G551D mutation. KALYDECO is designed to keep the CFTR channels at the cell surface open longer to improve the transport of chloride ions across the cell membrane in people who have gating mutations. KALYDECO has been studied in people with CF ages 6 and older who carry at least one copy of the G551D mutation.

"These regulatory applications are a reflection Vertex's 13-year research and development effort and the commitment of hundreds of doctors, nurses, patients and their caregivers who participated in the studies of KALYDECO," said Peter Mueller, Ph.D., Chief Scientific Officer and Executive Vice President of Global Research and Development at Vertex. "We look forward to working with U.S. and European regulatory agencies to make KALYDECO available as quickly as possible."

Highlights of the KALYDECO Phase 3 Registration Program

These regulatory submissions are supported by results from two Phase 3 studies, STRIVE and ENVISION, in which people with CF who had at least one copy of the G551D mutation and were treated with KALYDECO experienced rapid, significant and sustained improvements across a variety of disease measures, including lung function. These data support the hypothesis that treating the underlying cause of CF may improve outcomes for people with the disease. A Phase 2 study, DISCOVER, was conducted in people who had two copies of the F508del mutation, the most common *CFTR* mutation, and provided additional safety information to support the regulatory applications for KALYDECO.

Overview of KALYDECO Discovery and Development Effort

KALYDECO was discovered as part of a collaboration with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), the nonprofit drug discovery and development affiliate of the CF Foundation, to discover and develop novel CFTR modulators.

Expanded Access Programs for KALYDECO

In recognition of the immediate needs of some people with CF, an expanded access program for KALYDECO is currently open at participating clinical trial sites in the United States. This program is designed to provide KALYDECO to people ages 6 and older who have at least one copy of the G551D mutation, are in critical medical need and may benefit from treatment prior to potential FDA approval in the United States.

Vertex is working toward implementing additional expanded access programs in other countries, with a goal of opening programs for eligible patients by the end of 2011.

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

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 38 years. According to the 2010 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 40 percent of the total CF patient population have one copy of the F508del mutation.

Collaborative History with Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT)

Vertex initiated its CF research program in 1998 as part of a collaboration with CFFT, the nonprofit 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 KALYDECO and VX-809. VX-809, known as a CFTR corrector, is designed to help the protein reach the cell surface, while KALYDECO, known as a CFTR potentiator, aims to help the protein function more normally once it reaches the cell surface.

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.

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, 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, rheumatoid arthritis, 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. Today, Vertex has more than 1,900 employees around the world, and *Science* magazine named Vertex number one on its 2011 list of Top Employers in the life sciences.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements, as defined in the Private Securities Litigation Reform Act of 1995, as amended, including statements regarding (i) the plan to submit the registration process for KALYDECO with the EMA by the end of October 2011; (ii) the expectation that, if approved, KALYDECO will be the first treatment to target the underlying cause of CF; (iii) the possibility that the FDA review period for KALYDECO could be shortened from ten to six months; (iv) Vertex's commitment to developing new medicines for people with serious diseases; (v) the possibility that other targeted medicines could be developed for all people with this disease; (vi) Vertex looking forward to working with U.S. and European regulatory agencies to make KALYDECO available as quickly as possible and (vii) the hypothesis that treating the underlying cause of CF may improve outcomes for people with CF. While the company 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 in obtaining approval to market KALYDECO; that future outcomes from clinical trials of KALYDECO may not be favorable; that future scientific, clinical, competitive or other market factors may adversely affect the potential for KALYDECO and the other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through Vertex'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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