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Vertex Receives European CHMP Positive Opinion for KALYDECO™ (ivacaftor), the First Medicine to Treat the Underlying Cause of Cystic Fibrosis

- Global studies showed significant and sustained improvements in lung function and other measures of disease among people with a specific genetic mutation -

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) announced today that the European Committee for Medicinal Products for Human Use (CHMP) has issued a positive opinion by consensus recommending the approval of KALYDECO™ (ivacaftor) for people with cystic fibrosis (CF) ages 6 and older who have at least one copy of the G551D mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. KALYDECO is the first medicine to treat the underlying cause of CF, a rare, genetic disease caused by defective or missing *CFTR* proteins resulting from mutations in the *CFTR* gene. In people with the G551D mutation, KALYDECO helps the defective *CFTR* protein function more normally. An estimated 1,100 people in Europe have this mutation.

The CHMP opinion was based on positive findings from two global Phase 3 studies in which KALYDECO demonstrated unprecedented improvements in breathing and other measures of disease for people ages 6 and older with this specific genetic mutation. People treated with KALYDECO experienced significant and sustained improvements in lung function, weight gain and certain quality of life measurements compared to those on placebo. In addition, people who took KALYDECO were 55 percent less likely to have pulmonary exacerbations, or periods of worsening in the signs and symptoms of the disease that often require treatment with antibiotics and hospital visits, than those who received placebo. Fewer people in the KALYDECO treatment groups discontinued treatment due to adverse events than in the placebo groups. The majority of adverse events associated with KALYDECO were mild to moderate. Adverse events most commonly observed in those taking KALYDECO included headache, upper respiratory tract infection (common cold), stomach pain and diarrhea.

"While there has been great progress in cystic fibrosis treatment during the last few decades, we are still only treating the symptoms and complications of the disease," said Stuart Elborn, M.D., KALYDECO investigator and President of the European Cystic Fibrosis Society. "KALYDECO is a fundamentally different approach to the way we treat cystic fibrosis because it targets the underlying cause of the disease. In clinical trials, KALYDECO helped people with a specific genetic mutation breathe more easily, gain weight and generally feel better."

The CHMP's positive opinion will now be reviewed by the European Commission, which has the authority to approve medicines for the European Union. The European Commission generally follows the recommendation of the CHMP and typically issues marketing approval within three to four months.

"Since 1998, Vertex has been committed to developing new medicines to treat the underlying cause of cystic fibrosis," said Peter Mueller, Ph.D., Chief Scientific Officer and Executive Vice President of Global Research and Development at Vertex. "KALYDECO represents an important achievement in this ongoing effort. We look forward to working with the European Medicines Agency to bring KALYDECO, our first new medicine in Europe, to people with CF as quickly as possible."

KALYDECO was discovered as part of a collaboration with Cystic Fibrosis Foundation Therapeutics, Inc., the nonprofit drug discovery and development affiliate of the Cystic Fibrosis Foundation.

About Cystic Fibrosis

Cystic fibrosis is a rare, life-threatening genetic disease affecting approximately 35,000 people in Europe and 70,000 people worldwide. Today, the median predicted age of survival for a person with CF is approximately 38 years but the median age of death remains in the mid-20s. There are more than 1,800 known mutations in the *CFTR* gene. Some of these mutations, which can be determined by a genetic, or genotyping test, lead to CF by creating non-working or too few *CFTR* proteins at the cell surface. The absence of working *CFTR* proteins results in poor flow of salt and water into and out of the cell 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.

In some people, *CFTR* proteins are present at the cell surface but do not work properly. One type of this dysfunction is known as the G551D mutation.

About KALYDECO

KALYDECO™ (ivacaftor) is the first treatment to target the underlying cause of CF. KALYDECO (150mg, q12h) was approved by the U.S. Food and Drug Administration in January 2012 for use in people with CF ages 6 and older who have at least one copy of the G551D mutation in the *CFTR* gene. Approximately 600 people with CF have started treatment with KALYDECO since then.

Vertex retains worldwide rights to develop and commercialize KALYDECO.

Indication and Important Safety Information

KALYDECO (150mg, q12h) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have a certain mutation in their *CFTR* gene called the G551D mutation.

KALYDECO is not for use in people with CF due to other mutations in the *CFTR* gene. It is not effective in CF patients with two copies of the F508del mutation (F508del/F508del) in the *CFTR* gene.

It is not known if KALYDECO is safe and effective in children under 6 years of age.

KALYDECO should not be used with certain medicines, including the antibiotics rifampin and rifabutin; seizure medications (phenobarbital, carbamazepine, or phenytoin); and the herbal supplement St. John's Wort.

KALYDECO can cause serious side effects. High liver enzymes in the blood have occurred in patients taking KALYDECO as well as those receiving placebo. Regular assessment is recommended.

The most common side effects associated with KALYDECO include headache; upper respiratory tract infection (common cold) including sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; nausea; and dizziness.

These are not all the possible side effects of KALYDECO. Patients should tell their healthcare providers about any side effect that bothers them or doesn't go away.

Please see full U.S. Prescribing Information for KALYDECO at www.KALYDECO.com.

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. This collaboration was expanded to support the accelerated discovery and development of Vertex's *CFTR* modulators.

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 2,000 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, including statements regarding (i) the European Commission generally following the recommendations of the CHMP and typically issuing marketing approval within three to four months and (ii) Vertex working with the European Medicines Agency to bring KALYDECO to people with CF as quickly as possible. 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 in obtaining approval to market KALYDECO from the European Commission 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 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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