May 12, 2015

Food and Drug Administration Advisory Panel Voted 12 to 1 to Recommend Approval of ORKAMBI™ (lumacaftor/ivacaftor) to Treat People with Cystic Fibrosis Ages 12 and Older Who Have Two Copies of the F508del Mutation

-FDA decision expected by July 5, 2015 PDUFA date-

-Approximately 8,500 people with cystic fibrosis in the U.S. have two copies of the F508del mutation and are ages 12 and older-

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the U.S. Food and Drug Administration's (FDA) Pulmonary-Allergy Drugs Advisory Committee (PADAC) voted 12 to 1 to recommend that the FDA approve ORKAMBI™ (lumacaftor/ivacaftor) for use in people with cystic fibrosis (CF) ages 12 and older who have two copies of the F508del mutation in the *CFTR* gene. Advisory committees provide the FDA with independent scientific and medical advice on safety, effectiveness and appropriate use of potential new medicines. The FDA is expected to make a decision on the approval of ORKAMBI by July 5, 2015 under the Prescription Drug User Fee Act (PDUFA). The FDA is not bound by the committee's recommendation but often follows its advice. If approved, ORKAMBI will be the first and only medicine to treat the underlying cause of CF for eligible people with CF ages 12 and older with two copies of the F508del mutation in the *CFTR* gene. People with two copies of the F508del mutation represent the largest group of people with CF. There are approximately 8,500 people ages 12 and older with two copies of the F508del mutation in the U.S.

"Today's positive recommendation brings the cystic fibrosis community one step closer to potential approval of the first medicine to treat the underlying cause of this disease for many more people," said Jeffrey Chodakewitz, M.D., Executive Vice President and Chief Medical Officer at Vertex. "We look forward to continuing to work with the FDA and other regulatory agencies throughout the world to make ORKAMBI available to eligible patients as soon as possible."

Cystic fibrosis is a rare genetic disease that is caused by defective or missing CFTR proteins resulting from mutations in the *CFTR* gene. The defective or missing proteins result in poor flow of salt and water into and out of the cell in a number of organs, including the lungs. In people with two copies of the F508del mutation, the CFTR protein is not processed and trafficked normally within the cell, resulting in little to no CFTR protein at the cell surface.

ORKAMBI is a combination of lumacaftor, which is designed to increase the amount of functional protein at the cell surface by addressing the processing and trafficking defect of the protein, and ivacaftor, which is designed to enhance the function of the CFTR protein once it reaches the cell surface. ORKAMBI is an oral medicine that, if approved, would be taken as fully co-formulated tablets that contain both lumacaftor and ivacaftor.

About Cystic Fibrosis

CF is a rare, life-threatening genetic disease affecting approximately 75,000 people in North America, Europe and Australia. Children must inherit two defective *CFTR* genes — one from each parent — to have CF. There are more than 1,900 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 defective or missing CFTR protein 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, eventually leading to death.

Today, the median predicted age of survival for a person with CF is between 34 and 47 years, but the median age of death remains in the mid-20s.

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

Vertex initiated its CF research program in 1998 as part of a collaboration with Cystic Fibrosis Foundation Therapeutics (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 Vertex

Vertex is a global biotechnology company that aims to discover, develop and commercialize innovative medicines so people with serious diseases can lead better lives. In addition to our clinical development programs focused on cystic fibrosis, Vertex has more than a dozen ongoing research programs aimed at other serious and life-threatening diseases.

Founded in 1989 in Cambridge, Mass., Vertex today has research and development sites and commercial offices in the United States, Europe, Canada and Australia. For five years in a row, *Science* magazine has named Vertex one of its Top Employers in the life sciences. For additional information and the latest updates from the company, please visit www.vrtx.com.

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, without limitation, statements regarding the potential approval of ORKAMBI as a treatment for patients with CF twelve years or older who have two copies of the F508del mutation in their *CFTR* gene. 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 regulatory authorities may not approve, or approve on a timely basis, ORKAMBI for patients with CF twelve years or older who have two copies of the F508del mutation in their *CFTR* gene due to safety, efficacy or other reasons, 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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