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New England Journal of Medicine Publishes Data from Two Phase 3 Studies of ORKAMBI™ (lumacaftor/ivacaftor) in People with Cystic Fibrosis who have Two Copies of the F508del Mutation

BOSTON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the *New England Journal of Medicine (NEJM)* published data from the two Phase 3 studies of ORKAMBI™ (lumacaftor/ivacaftor), an investigational medicine designed to treat the underlying cause of cystic fibrosis (CF) in people ages 12 and older with two copies of the F508del mutation, the most common form of the disease. The data were published online today in conjunction with the American Thoracic Society International Conference (May 15-20, Denver, Colo.) where the data were presented in a session titled, "Discussion on the Edge: Recent Pulmonary Research Published in NEJM or JAMA."

In November 2014, Vertex submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for the combination of lumacaftor and ivacaftor in people with CF ages 12 and older who have two copies of the F508del mutation. On May 12th, 2015, the FDA's Pulmonary Allergy Drugs Advisory Committee (PADAC) voted 12-1 to recommend that the FDA approve ORKAMBI for this group of people with CF. The FDA is expected to make a decision on the ORKAMBI NDA by July 5, 2015.

Cystic fibrosis is a rare genetic disease that is caused by defective or missing cystic fibrosis transmembrane conductance regulatory (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 an investigational medicine that 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.

About Cystic Fibrosis

Cystic fibrosis is a rare, life-threatening genetic disease affecting approximately 75,000 people in North America, Europe and Australia.

CF is caused by a defective or missing CFTR protein resulting from mutations in the *CFTR* gene. 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 that eventually leads 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.

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 timing of 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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