July 13, 2017

Vertex Announces Reimbursement Agreement in Italy for ORKAMBI® (Lumacaftor/Ivacaftor), the First Medicine to Treat the Underlying Cause of Cystic Fibrosis in People Ages 12 and Older with Two Copies of the F508del Mutation

- Effective immediately, agreement enables hundreds of people in Italy to access this important medicine -

- Recent pricing and reimbursement agreements have enabled broad access to ORKAMBI for thousands of eligible patients in multiple European countries; negotiations continue in a number of other countries, including France and the United Kingdom -

LONDON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the Italian Medicines Agency (Agenzia Italiana del Farmaco, or AIFA) has agreed to reimburse ORKAMBI® (lumacaftor/ivacaftor), the first medicine to treat the underlying cause of cystic fibrosis (CF) in people ages 12 and older who have two copies of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. The agreement is published online in the *Italian Official Gazette*. Regional authorities will now begin implementation to provide the hundreds of eligible patients in Italy access to this important medicine. Recent European pricing and reimbursement agreements have enabled broad access to ORKAMBI for thousands of eligible patients in Austria, Denmark, Germany, Ireland, Italy and Luxembourg. Negotiations continue in a number of other countries where CF is prevalent, including France and the United Kingdom.

"We are pleased to have reached this agreement on behalf of CF patients in Italy who have been waiting for this important medicine," said Simon Bedson, Senior Vice President and International General Manager at Vertex. "We continue negotiations with other countries including France and the United Kingdom, and we encourage these national health authorities and governments to work quickly with us to achieve reimbursement for all patients who may benefit."

About ORKAMBI® (lumacaftor/ivacaftor) and the *F508del* mutation

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. Patients with two copies of the *F508del* mutation are easily identified by a simple genetic test.

ORKAMBI is a combination of lumacaftor, which is designed to increase the amount of mature protein at the cell surface by targeting the processing and trafficking defect of the F508del-CFTR protein, and ivacaftor, which is designed to enhance the function of the CFTR protein once it reaches the cell surface. ORKAMBI is available as tablets and is typically taken twice per day.

For complete product information, please see the Summary of Product Characteristics that can be found on www.ema.europa.eu.

About CF

CF is a rare, life-shortening 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 approximately 2,000 known mutations in the *CFTR* gene. Some of these mutations, which can be determined by a genetic test, or genotyping test, lead to CF by creating non-working or too few CFTR proteins at the cell surface. The defective function or absence of CFTR protein results in poor flow of salt and water into and out of the cell in a number of organs. In the lungs, this leads to the buildup of abnormally thick, sticky mucus that can cause chronic lung infections and progressive lung damage in many patients that eventually leads to death. The median age of death is in the mid-to-late 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 seven 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.

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

Vertex initiated its CF research program in 2000 as part of a collaboration with CFFT, the nonprofit drug discovery and development affiliate of the Cystic Fibrosis Foundation. KALYDECO® (ivacaftor), ORKAMBI® (lumacaftor/ivacaftor) and tezacaftor were discovered by Vertex as part of this collaboration.

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 the quote in the second paragraph of this press release and statements regarding the country-by-country reimbursement approval process. 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, risks related to commercializing ORKAMBI 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.

(VRTX-GEN)

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Source: Vertex Pharmaceuticals Incorporated