December 19, 2016

Vertex Announces German Reimbursement Agreement 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

LONDON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq:VRTX) today announced it has reached a pricing and reimbursement agreement for ORKAMBI® (lumacaftor/ivacaftor) with the German Federal Association of the Statutory Health Insurances (GKV-SV). ORKAMBI is 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 CF transmembrane conductance regulator (*CFTR*) gene. CF is a rare and life-shortening genetic disease caused by a defective or missing CFTR protein resulting from a mutation in the *CFTR* gene. The disease is present from birth and causes chronic lung infections and progressive damage to a number of organs throughout the body.

Today's announcement follows a comprehensive benefit assessment of the medicine by the German Federal Joint Committee (G-BA), which recognized the "considerable additional benefit" of ORKAMBI for people with CF who have two copies of the F508del mutation. ORKAMBI has been available to eligible patients in Germany since it was granted marketing authorization from the European Commission in November 2015, and the reimbursement agreement announced today took effect on December 16, 2016.

"Cystic fibrosis is a progressive disease, meaning that with each passing day, lung function can deteriorate and patients get sicker," said David Gillen, M.D., Vice President of International Medical Affairs at Vertex. "We are pleased the German authorities approved ORKAMBI reimbursement for all eligible patients. The reimbursed price recognizes the clinical value of ORKAMBI and the need for Vertex's continued investment in the research and development of new medicines for the two out of three people with CF still waiting for a treatment for the underlying cause of their form of the disease."

In addition to Germany, ORKAMBI is now available to eligible people with CF in the United States, Austria, and France. Vertex remains actively involved in reimbursement discussions in many other countries to make ORKAMBI available to all people who can benefit from this important medicine. Earlier this month, ORKAMBI received the "*Drug Discovery of the Year*" award from the British Pharmacological Society and the French *"Prix Galien"* award for the most promising rare disease medicine in 2016.

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) and ORKAMBI® (lumacaftor/ivacaftor) 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 third 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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