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Vertex Announces Long-Term Reimbursement Agreement with the Republic of Ireland for ORKAMBI® (lumacaftor/ivacaftor), KALYDECO® (ivacaftor) and Future Cystic Fibrosis Medicines

-Agreement provides access to ORKAMBI for people who have two copies of the F508del mutation and expands access to KALYDECO for all eligible patients-

LONDON--(BUSINESS WIRE)-- Vertex Pharmaceuticals Incorporated (Nasdaq:VRTX) today announced it has reached an agreement with the Health Service Executive (HSE) in the Republic of Ireland to fund ORKAMBI® (lumacaftor/ivacaftor) for all of the approximately 500 people in Ireland with cystic fibrosis (CF) ages 12 and older who have two copies of the *F508del* mutation. The agreement also expands access to KALYDECO® (ivacaftor) for children ages 2 to 5 with any approved gating mutation (*G551D*, *G178R*, *S549N*, *S549R*, *G551S*, *G1244E*, *S1251N*, *S1255P* and *G1349D*) and to people ages 18 and older who have an *R117H* mutation. These reimbursements are effective immediately. This innovative long-term agreement also enables rapid access for people with these mutations if the labels of the existing medicines are expanded to cover additional age groups and if new Vertex medicines are approved for these populations.

"We are pleased that these additional Irish CF patients will finally join the thousands of others around the world who are already benefitting from ORKAMBI and KALYDECO," said Simon Bedson, Senior Vice President and International General Manager at Vertex. "We thank the leaders in Ireland for working with us toward an innovative reimbursement agreement that provides access to these important medicines and also recognizes the need for Vertex's continued investment in the research and development of new medicines for those people with CF, many of whom are still waiting for a treatment for the underlying cause of the disease."

CF is a rare and life-shortening genetic disease caused by a defective or missing cystic fibrosis transmembrane conductance regulator (CFTR) protein resulting from a mutation in the *CFTR* gene. ORKAMBI and KALYDECO are the first two medicines that treat the underlying cause of CF. Ireland has the highest rate of CF in the world, with approximately one in 19 Irish people carrying a disease-causing mutation in one copy of the *CFTR* gene.

In addition to Ireland, ORKAMBI is available to all eligible patients in Austria, Denmark, France, Germany, Luxembourg and the United States. People in 27 countries are benefitting from KALYDECO. Vertex remains actively involved in additional reimbursement discussions globally, with the goal of making these transformative medicines available to all eligible patients as soon as possible.

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 KALYDECO® (ivacaftor)

KALYDECO® (ivacaftor) is the first medicine to treat the underlying cause of CF in people with specific mutations in the *CFTR* gene. Known as a CFTR potentiator, ivacaftor is an oral medicine that aims to help the CFTR protein function more normally once it reaches the cell surface, to help hydrate and clear mucus from the airways.

For complete product information, please see the Summary of Product Characteristics that can be found at 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 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.

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