# Vertex Announces Long-Term Access Agreement in Sweden for Cystic Fibrosis Medicine ORKAMBI® (lumacaftor/ivacaftor)

June 18, 2018

*- The agreement allows for reimbursement of ORKAMBI for people with cystic fibrosis who have two copies of the F508del mutation from July 1 -*

*- A framework for assessment and access to our future cystic fibrosis medicines is included as part of the agreement -*

LONDON--(BUSINESS WIRE)--Jun. 18, 2018-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that ORKAMBI® (lumacaftor/ivacaftor), the first medicine to treat the underlying cause of cystic fibrosis (CF) in people with two copies of the *F508del* mutation, ages six and older, will be reimbursed in Sweden after concluding the three-party negotiations with TLV and the county councils. Reimbursement is effective from July 1. The innovative, long-term access agreement also provides a framework for the assessment and access of our future CF medicines.

"We are delighted that people with CF in Sweden will join the thousands of others around the world who are already benefitting from our CF medicines," said Simon Bedson, International General Manager at Vertex. "We commend the Swedish authorities for partnering with us on an innovative, long-term access agreement. In countries where Vertex remains actively involved in reimbursement discussions, we encourage these health authorities and governments to match the commitment to innovation shown in Sweden to secure access for all patients who may benefit."

CF is a devastating rare disease that causes continuous damage to multiple organs from birth. In the lungs, a build-up of sticky mucus causes progressive and permanent damage, severe infections and ultimately premature death. In addition to Sweden, countries where lumacaftor/ivacaftor is available to all eligible patients include Austria, Germany, Ireland, Italy, the Netherlands and the U.S.

**About Cystic Fibrosis**

Cystic fibrosis 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 build-up 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 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. Lumacaftor/ivacaftor 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 Vertex**

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious and life-threatening diseases. In addition to clinical development programs in CF, Vertex has more than a dozen ongoing research programs focused on the underlying mechanisms of other serious diseases.

Founded in 1989 in Cambridge, Mass., Vertex's headquarters is now located in Boston'sInnovation District. Today, the company has research and development sites and commercial offices in the United States, Europe, Canada and Australia. Vertex is consistently recognized as one of the industry's top places to work, including being named to *Science* magazine's Top Employers in the life sciences ranking for eight years in a row.

For additional information and the latest updates from the company, please visit www.vrtx.com.

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