Vertex Announces Reimbursement Agreement in Australia for ORKAMBI® (lumacaftor/ivacaftor) for People with Cystic Fibrosis Ages Six Years and Older with Two Copies of the F508del Mutation

September 3, 2018

- Approximately 1,300 patients in Australia join the thousands of patients worldwide who already have access to lumacaftor/ivacaftor -

- A pathway to access for future Vertex CF medicine, tezacaftor/ivacaftor, has also been established -

LONDON--(BUSINESS WIRE)--Sep. 3, 2018-- Vertex Pharmaceuticals (Europe) Limited announces finalization of an agreement with the Australian Government that allows for reimbursement of ORKAMBI® (lumacaftor/ivacaftor) for people ages six and over with cystic fibrosis (CF) who have two copies of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. This agreement means lumacaftor/ivacaftor will be listed on the Pharmaceutical Benefits Scheme (PBS) from 1 October and follows a positive recommendation from the Pharmaceutical Benefits Advisory Committee (PBAC). A pathway to access for future Vertex CF medicine, tezacaftor/ivacaftor, has been established as part of this process.

"We are delighted that lumacaftor/ivacaftor will be made available to eligible CF patients in Australia," said Stuart Arbuckle, Executive Vice President and Chief Commercial Officer at Vertex. "We would like to recognize the PBAC for seeing the value of this medicine and acknowledge the Department of Health and the Minister for Health for their active engagement and willingness to finalize the agreement rapidly."

Australians with CF join thousands of patients worldwide who are already receiving lumacaftor/ivacaftor. Beyond Australia, countries where lumacaftor/ivacaftor is reimbursed include Austria, Denmark, Germany, Ireland, Italy, the Netherlands, Sweden and the U.S.

About CF
Cystic fibrosis is a rare, life-shortening genetic disease affecting approximately 75,000 people including countries within North America and Europe, as well as 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, Australia and Brazil. 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.

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, Mr. Arbuckle's statements in the second paragraph of this press release. 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, 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. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

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