



Vertex Receives a Positive PBAC Recommendation for Reimbursement of ORKAMBI® (lumacaftor/ivacaftor) to Treat Australians Ages Six and Over with Cystic Fibrosis and Two Copies of the F508del Mutation

August 20, 2018

- *PBAC recommendation moves approximately 1,300 patients in Australia closer to access to lumacaftor/ivacaftor and Vertex is now working with the Australian Government to finalize a reimbursement agreement as soon as possible* -

LONDON--(BUSINESS WIRE)--Aug. 20, 2018-- Vertex Pharmaceuticals (Europe) Limited announces that it has received the Pharmaceutical Benefits Advisory Committee's (PBAC) recommendation for ORKAMBI® (lumacaftor/ivacaftor) to be listed on the Pharmaceutical Benefits Scheme (PBS) in Australia 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. Lumacaftor/ivacaftor is the first medicine to treat the underlying cause of CF in people ages six and older who have two copies of the *F508del* mutation.

"We're very pleased that lumacaftor/ivacaftor received a positive PBAC recommendation for CF patients in Australia, which brings those who have long been waiting closer to receiving this important medicine," said Simon Bedson, International General Manager at Vertex. "We welcome the PBAC decision and are now working with the Australian Government to finalize the agreement as quickly as possible to make lumacaftor/ivacaftor available to patients."

Many thousands of patients worldwide are already receiving lumacaftor/ivacaftor in countries where it is reimbursed including Austria, Denmark, Germany, Ireland, Italy, Sweden, the Netherlands and the U.S.

Vertex remains steadfast in its commitment to ensuring people living with CF have rapid access to its medicines.

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 the Australian Pharmaceutical Benefits Advisory Committee (PBAC)

The PBAC is an independent, expert statutory body which reviews submissions from pharmaceutical companies regarding new medicines to be considered for subsidy within the Pharmaceutical Benefits Scheme (PBS). The PBAC makes recommendations to the Government as to whether or not a medicine should be subsidized and the Health Minister cannot add a medicine to the PBS without prior recommendation from the PBAC.

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's Innovation 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. Bedson's statement 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, that Vertex could experience unforeseen delays in reaching an agreement with the Australian government 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.

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