Vertex Announces Reimbursement of Cystic Fibrosis Medicines SYMDEKO® (tezacaftor/ivacaftor and ivacaftor) for Eligible Patients Ages 12 and Older, and ORKAMBI® (lumacaftor/ivacaftor) in Children Ages 2 to 5, With Certain CFTR Mutations in Australia

October 19, 2019

LONDON--(BUSINESS WIRE)--Oct. 19, 2019-- Vertex Pharmaceuticals Incorporated (NASDAQ: VRTX) today announced that SYMDEKO® (tezacaftor/ivacaftor and ivacaftor) is reimbursed in Australia for people with cystic fibrosis (CF) ages 12 years and older who are homozygous for the *F508del* mutation or who have one copy of the *F508del* mutation and another responsive residual function (RF) mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. People with CF who have one copy of the *F508del* mutation and another responsive RF mutation in the *CFTR* gene will have access to a medicine for the cause of their CF for the first time. In addition, ORKAMBI® (lumacaftor/ivacaftor) is now also reimbursed for the treatment of children with CF ages 2 to 5 who have two copies of the *F508del* mutation in the *CFTR* gene. Patients over the age of 6 have already been able to access ORKAMBI® in Australia since October 2018.

Following previously received positive recommendations from the Pharmaceutical Benefits Advisory Committee (PBAC), eligible patients in Australia will be able to access both medicines immediately, and the medicines will be listed on the Pharmaceutical Benefits Scheme (PBS) from December 1st.

"We are pleased that SYMDEKO® and ORKAMBI® will be made available immediately to eligible cystic fibrosis patients in Australia. We appreciate that the PBAC has recognized the value of these medicines to patients and thank the Department of Health and the Minister for Health in Australia for their strong engagement and collaboration to finalize the agreement," said Ludovic Fenaux, Senior Vice President, Vertex International.

Vertex's CF medicines are reimbursed in 17 countries around the world including Austria, Denmark, Germany, the Republic of Ireland, Italy, the Netherlands, Sweden and the U.S.

About CF
Cystic Fibrosis (CF) is a rare, life-shortening genetic disease affecting approximately 75,000 people worldwide. CF is a progressive, multi-system disease that affects the lungs, liver, GI tract, sinuses, sweat glands, pancreas and reproductive tract. CF is caused by a defective and/or missing *CFTR* protein resulting from certain mutations in the *CFTR* gene. Children must inherit two defective *CFTR* genes — one from each parent — to have CF. While there are many different types of *CFTR* mutations that can cause the disease, the vast majority of all people with CF have at least one *F508del* mutation. These mutations, which can be determined by a genetic test, or genotyping test, lead to CF by creating non-working and/or too few *CFTR* proteins at the cell surface. The defective function and/or absence of *CFTR* protein results in poor flow of salt and water into and out of the cells 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 early 30s.

About SYMDEKO® (tezacaftor/ivacaftor) in combination with ivacaftor
Some mutations result in *CFTR* protein that is not processed or folded normally within the cell, and that generally does not reach the cell surface. Tezacaftor is designed to address the trafficking and processing defect of the *CFTR* protein to enable it to reach the cell surface and ivacaftor is designed to enhance the function of the *CFTR* protein once it reaches the cell surface.

Mutations in the *CFTR* gene, responsive to SYMDEKO®, that are currently registered in Australia include *F508del/f* and *P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G, 3849+10kbC→T, E56K, R74W, D110E, D110H, E193K, E831X, F1052V, K1060T, A1067T, F1074L and D1270N*.

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. Lumacaftor/ivacaftor 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.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has three approved medicines that treat the underlying cause of cystic fibrosis (CF) – a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational medicines in other serious diseases where it has deep insight into causal human biology, such as sickle cell disease, beta thalassemia, pain, alpha-1 antitrypsin deficiency, Duchenne muscular dystrophy and APOL1-mediated kidney diseases.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London, UK. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including nine consecutive years on Science magazine's Top Employers list and top five on the 2019 Best Employers for Diversity list by Forbes.

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, the statements in the second and third paragraphs of the press release. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that data from the company's development programs may not support registration or further development of its compounds due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and subsequent quarterly reports filed with the Securities and Exchange Commission and available through the company'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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