Vertex Announces Innovative Reimbursement Agreement in Switzerland for ORKAMBI® (lumacaftor/ivacaftor) and SYMDEKO® (tezacaftor/ivacaftor and ivacaftor) for Eligible Cystic Fibrosis Patients

April 21, 2020

Agreement also enables the possibility of rapid patient access to future triple combination regimen (elexacaftor/tezacaftor/ivacaftor and ivacaftor) once approved in Switzerland

LONDON--(BUSINESS WIRE)--Apr. 21, 2020-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that it has reached an agreement with the Swiss Federal Office of Public Health (FOPH) and the Swiss Federal Social Insurance Office (FSIO) for the reimbursement of ORKAMBI® (lumacaftor/ivacaftor) and SYMDEKO® (tezacaftor/ivacaftor and ivacaftor) for eligible patients in Switzerland living with cystic fibrosis (CF).

Under the terms of the agreement announced today, eligible patients ages two years and older with CF who have two copies of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene can be treated with ORKAMBI®. Eligible patients ages 12 years and older who either have two copies of the *F508del* mutation, or one copy of the *F508del* mutation and another responsive residual function mutation in the *CFTR* gene, can be treated with SYMDEKO®. The agreement also includes any future indication extensions for patients of different ages for SYMDEKO®.

This agreement also enables the possibility for rapid patient access to a potential future triple combination regimen (elexacaftor/tezacaftor/ivacaftor and ivacaftor) pending marketing authorization from Swissmedic, the Swiss Agency for Therapeutic Products. Vertex submitted an application to Swissmedic on March 24, 2020.

Commenting about today's news in Switzerland, Ludovic Fenaux, Senior Vice President, Vertex International, said, "This agreement is an important milestone for the cystic fibrosis community in Switzerland. Access to Orkambi and Symdeko is especially important at this time given the COVID-19 outbreak and that people with CF are vulnerable to infections. We are pleased that Vertex and the Swiss authorities have been able to work closely and flexibly to enable this agreement, so that almost 400 eligible Swiss patients will now have access to CFTR modulators to treat the underlying cause of their disease."

Vertex's CF medicines are reimbursed in more than 20 countries around the world including Australia, France, Germany, the Republic of Ireland, Italy, the Netherlands, Spain, Sweden, the UK and the U.S.

About Cystic Fibrosis

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 Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple 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 small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of genetic and cell therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

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 10 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 by Mr. Fenaux in this press release, statements regarding our expectations for the patient populations that will be able to access Vertex's medicines and the timing of such access, and statements about our plans to submit a marketing authorization application for the triple combination regimen to Swissmedic. 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, obtaining approval and commercializing elexacaftor/tezacaftor/ivacaftor in Europe, developing additional medicines to treat cystic fibrosis, 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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