Health Canada Grants Marketing Authorization for TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) in People Ages 12 and Older Who Have at Least One F508del Mutation

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-Approximately 1,100 F/MF patients now eligible for a CFTR modulator to treat the underlying cause of their disease-

BOSTON--(BUSINESS WIRE)--Jun. 18, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced Health Canada has granted Marketing Authorization for TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) for the treatment of cystic fibrosis (CF) in people ages 12 years and older who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene, the most common CF-causing mutation. With this approval, for the first time, approximately 1,100 eligible patients with CF ages 12 years and older who have at least one *F508del* mutation have a medicine that targets the underlying cause of their CF.

“The approval of TRIKAFTA marks a significant milestone for Canadians with CF, their families and Vertex,” said Reshma Kewalramani, M.D., Chief Executive Officer and President, Vertex. “I would like to thank the people with CF who participated in our clinical trials, our dedicated scientists and the investigators who have enabled this innovative medicine to be approved in Canada today. Without their commitment, this milestone would not have been possible.”

“I have seen substantial improvements in patients treated with TRIKAFTA in clinical practice, including improved lung function,” said Dr. Elizabeth Tullis, Medical Director, Toronto Adult CF Centre, Professor of Medicine, University of Toronto. “I’m excited that more Canadians may be able to benefit from CFTR modulators and look forward to seeing the impact of this medicine for all patients who can benefit from it.”

About TRIKAFTA®

TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients ages 12 years and older who have at least one copy of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. TRIKAFTA® is designed to increase the quantity and function of the F508del-CFTR protein at the cell surface. The approval of TRIKAFTA® was supported by positive results of three global Phase 3 studies in people ages 12 years and older with CF: a 24-week Phase 3 study (Study 445-102) in 403 people with one *F508del* mutation and one minimal function mutation (F/MF), a four-week Phase 3 study (Study 445-103) in 107 people with two *F508del* mutations (F/F), and a Phase 3 study (Study 445-104) in 258 people heterozygous for the *F508del-CFTR* mutation and a *CFTR* gating mutation (F/G) or a residual function mutation (F/RF).

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 80,000 people globally. 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 cell and genetic 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. 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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com.

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, statements made by Dr. Kewalramani and Dr. Tullis in this press release, and statements regarding our expectations for eligible patient population and access to TRIKAFTA® and our beliefs regarding the benefits of our medicine. 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 the heading "Risk Factors" in Vertex's most recent annual report and subsequent quarterly reports filed with the Securities and Exchange Commission at www.sec.gov and available through the company's website at www.vrtx.com. You should not place undue reliance on these statements. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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