Health Canada Grants Marketing Authorization for TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) in Children With Cystic Fibrosis Ages 6 Through 11 years With At Least One F508del Mutation

April 20, 2022

-Approximately 500 Canadians ages 6-11 are now eligible for TRIKAFTA®-

-Vertex has submitted this indication to CADTH & INESSS for Health Technology Assessments-

BOSTON--(BUSINESS WIRE)--Apr. 20, 2022-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that Health Canada has granted Marketing Authorization for the expanded use of TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) to include children with cystic fibrosis (CF) ages 6 through 11 years who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. With this announcement, approximately 500 Canadians with CF ages 6-11 are now eligible for TRIKAFTA®. As a result of this approval, an additional dosage strength of TRIKAFTA® tablets is now available (elexacaftor 50 mg/tezacaftor 25 mg/ivacaftor 37.5 mg and ivacaftor 75 mg).

"We are delighted that TRIKAFTA is now available for these young patients in Canada. It provides a new treatment option for those with CF ages 6-11 with at least one *F508del* mutation and a first-in-class treatment option for the approximately 500 6-11-year-olds who are newly eligible for a medicine that treats the underlying cause of their disease," said Reshma Kewalramani, M.D., Chief Executive Officer and President at Vertex. "This important milestone brings us one step closer to our ultimate goal of developing treatments for all patients living with CF. We will now work closely with all provinces and territories to secure access for eligible patients as quickly as possible."

Vertex completed a 24-week Phase 3 open-label, multicenter study which enrolled 66 children ages 6 through 11 years old with CF who have either two copies of the *F508del* mutation or one copy of the *F508del* mutation and one minimal function mutation to evaluate the safety, pharmacokinetics and efficacy of TRIKAFTA®. The regimen was generally well tolerated, and safety data were consistent with those observed in previous studies in patients ages 12 years and older.

"As a trial investigator, I have seen firsthand the demonstrated efficacy of TRIKAFTA in people ages 6-11 living with cystic fibrosis," said Larry C. Lands, M.D., Ph.D., Director, Pediatric Respiratory Medicine, Pediatric Cystic Fibrosis Clinic, and Pediatric Pulmonary Function Laboratory, Montreal Children's Hospital, McGill University Health Center, and Professor, Department of Pediatrics, Faculty of Medicine, McGill University. "This is an exciting next step that will allow eligible patients to begin treatment earlier."

Vertex has also submitted this indication to both the Canadian Agency for Drugs and Technologies in Health (CADTH) and the Institut national d'excellence en santé et en services sociaux (INESSS) in Québec for Health Technology Assessments.

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 83,000 people globally. CF is a progressive, multi-organ disease that affects the lungs, liver, pancreas, GI tract, sinuses, sweat glands 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, and these mutations can be identified by a genetic test. While there are many different types of *CFTR* mutations that can cause the disease, the vast majority of people with CF have at least one *F508del* mutation. *CFTR* mutations lead to CF by causing CFTR protein to be defective or by leading to a shortage or absence of CFTR protein 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, chronic lung infections and progressive lung damage that eventually leads to death for many patients. The median age of death is in the early 30s.

About TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor)

In people with certain types of mutations in the *CFTR* gene, the CFTR protein is not processed or folded normally within the cell, and this can prevent the CFTR protein from reaching the cell surface and functioning properly. TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) is an oral medicine designed to increase the quantity and function of the CFTR protein at the cell surface. Elexacaftor and tezacaftor work together to increase the amount of mature protein at the cell surface by binding to different sites on the CFTR protein. Ivacaftor, which is known as a CFTR potentiator, is designed to facilitate the ability of CFTR proteins to transport salt and water across the cell membrane. The combined actions of elexacaftor, tezacaftor and ivacaftor help hydrate and clear mucus from the airways. TRIKAFTA® is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients ages 6 years and older who have at least one copy of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

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, cell and genetic therapies in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, pain, type 1 diabetes, alpha-1 antitrypsin deficiency and Duchenne muscular dystrophy.

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 12 consecutive years on Science magazine's Top Employers list and one of the 2021 Seramount (formerly Working Mother Media) 100 Best Companies. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements made by Dr. Kewalramani and Dr. Lands in this press release, and statements regarding the estimated number of children eligible for TRIKAFTA® for the first time, our beliefs regarding the benefits of our medicines, and anticipated patient access to TRIKAFTA®. 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 or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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