Vertex Announces Reimbursement Agreement in Australia for TRIKAFTA® (elexacaftor/tezacaftor /ivacaftor and ivacaftor) for Patients With Cystic Fibrosis Ages 12 Years and Older With at Least One F508del Mutation in the CFTR Gene

March 26, 2022

- With this reimbursement agreement in place, more than 2,200 people with CF will have PBS-funded access to TRIKAFTA®, including more than 700 who will now have access to a CFTR modulator therapy for the first time -

LONDON--(BUSINESS WIRE)--Mar. 26, 2022-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that as of 1 April 2022, TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor*)* will be reimbursed on the Australian Pharmaceutical Benefits Scheme (PBS) for the treatment of cystic fibrosis (CF) in people ages 12 years and older who have at least one *F508del* mutation in the *CFTR* gene, the most common CF-causing mutation worldwide.

"Today's announcement is a significant milestone in ensuring Australians living with CF receive timely and sustainable access to TRIKAFTA," said Ludovic Fenaux, Senior Vice President, Vertex International. "This is the fourth treatment we have brought to Australians over the last eight years, working tirelessly alongside the CF patient and clinical communities. We thank the Australian Government for recognizing the significant need for TRIKAFTA and the value it brings."

TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) was approved by the Australian Therapeutic Goods Administration (TGA) in March 2021 based on the results of four global Phase 3 clinical trials, which included multiple Australian trial sites and patients.

CF is a rare, life-shortening, genetic disease affecting approximately 3,500 people in Australia. It is caused by a defective and/or missing CFTR protein, resulting from mutations in the *CFTR* gene. Up to 90 percent of people living with CF have at least one *F508del* mutation.

"As a genetic disease, cystic fibrosis is a prime candidate for precision medicine. Now, with PBS listing of TRIKAFTA, eligible Australians living with CF ages 12 years and older can broadly access a therapy that treats the underlying cause of their disease. Clinicians across Australia will be excited about this most welcome news," said Professor John Wilson AM, Head, Cystic Fibrosis Service, Alfred Health Australia.

Australia now joins the list of 30 countries where the triple combination therapy is approved and reimbursed including Denmark, Finland, France, Germany, Italy, Ireland, Israel, Poland, Spain, Switzerland and the countries within the U.K.

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 the 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.

For complete product information, please see the Summary of Product Characteristics that can be found on https://www.tga.gov.au/apm-summary/trikafta.

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 https://global.vrtx.com/ or follow us on Twitter and LinkedIn.

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 Ludovic Fenaux, Senior Vice President, Vertex International, and Professor John Wilson AM, Head, Cystic Fibrosis Service, Alfred Health, in this press release and statements regarding the reimbursement of and access to TRIKAFTA® for certain patients, the estimated number of patients eligible for a CFTR modulator therapy in Australia, including patients that will now have access to a CFTR modulator therapy for the first time, and our beliefs about the benefits of our medicines. 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 indicated 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, risks related to obtaining approval for and commercializing our medicines, and other risks listed under the heading "Risk Factors" in Vertex's annual report filed with the Securities and Exchange Commission (SEC) and available through the company's website at https://global.vrtx.com/ and on the SEC's website at www.sec.gov. 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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