Vertex Receives Australian TGA Approval for TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) to Treat People With Cystic Fibrosis Ages 12 Years and Older Who Have At Least One F508del Mutation

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- With this approval approximately 750 people living with cystic fibrosis in Australia will be newly eligible for a CFTR modulator therapy -

LONDON--(BUSINESS WIRE)--Mar. 24, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the Australian Therapeutic Goods Administration (TGA) has approved the use of TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) for people with cystic fibrosis (CF) 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 worldwide. Up to 90% of people living with CF worldwide have at least one *F508del* mutation.

“We are delighted the TGA has approved TRIKAFTA for eligible people living with CF in Australia and will continue working with the Australian government to bring this important medicine to patients as quickly as possible,” said Reshma Kewalramani, M.D., Chief Executive Officer and President, Vertex. “It is our goal to develop and provide treatments for all people with CF around the world, and today is another significant milestone on that journey.”

CF affects approximately 3,500 people in Australia. It is caused by a defective and/or missing CFTR protein resulting from mutations in the *CFTR* gene.

“Cystic fibrosis is a complex, progressive, devastating disease that causes severe damage to the lungs, digestive system and other organs in the body. It is a condition that significantly affects not only the patient, but also those who care for them, with people living with cystic fibrosis spending multiple hours every day on treatment and requiring daily care from a family member or loved one,” said Professor John Wilson AM, Head, Cystic Fibrosis Service, Alfred Health. “The approval of any new treatment option for people living with cystic fibrosis is always welcome news. This new treatment is for patients ages 12 years and older with at least one *F508del* mutation and means more patients can potentially benefit from a medicine that targets the underlying cause of the disease, for the first time.”

The TGA approval of TRIKAFTA was based on the results of four global Phase 3 studies, which included multiple trial sites and patients from Australia.

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 TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor)

In Australia, TRIKAFTA® is indicated for the treatment of cystic fibrosis (CF) in patients ages 12 years and older who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

The complete product information (PI) can be found on www.tga.gov.au.

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, 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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign.

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 Reshma Kewalramani, M.D., and Professor John Wilsonin this press release, and statements regarding our expectations for the eligible patient population in Australia, including patients not previously eligible for treatment with a CFTR modulator. 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 factors 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 a license extension for TRIKAFTA in Australia, and other risks listed under the heading “Risk Factors” in Vertex's annual report filed with the Securities and Exchange Commission and available through the company's website at www.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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