Vertex Announces U.S. FDA Acceptance of Supplemental New Drug Application for TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) in Children With Cystic Fibrosis Ages 6 through 11 With Certain Mutations

January 26, 2021

-FDA grants Priority Review of the application and sets a PDUFA target action date of June 8, 2021-

BOSTON--(BUSINESS WIRE)--Jan. 26, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the U.S. Food and Drug Administration (FDA) has accepted its supplemental New Drug Application (sNDA) to expand the use of TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) to include children ages 6 through 11 years old who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene or a mutation in the *CFTR* gene that is responsive based on *in vitro* data. The FDA has granted Priority Review of the sNDA and assigned a Prescription Drug User Fee Act (PDUFA) target action date of June 8, 2021. The submission was supported by data from a global Phase 3 study of TRIKAFTA in children ages 6 through 11 years old with cystic fibrosis (CF) who have either two copies of the *F508del* mutation or one copy of the *F508del* mutation and one minimal function mutation.

“If approved for this expanded use, we will have the opportunity to treat the underlying cause of the disease earlier in life with TRIKAFTA and potentially benefit approximately 1,500 additional children with CF,” said Carmen Bozic, M.D., Executive Vice President and Chief Medical Officer at Vertex. “Since our initial approval of TRIKAFTA in 2019, we have continued to work tirelessly to bring this medicine to those waiting as quickly as possible. We look forward to working with the Agency as they review the application over the course of the coming months.”

Vertex plans to submit a Marketing Authorization Application (MAA) variation for the triple combination in the European Union in the first half of 2021 for children ages 6 through 11. Global regulatory filings in additional markets, including Canada and Australia, are planned in the coming months for this age group.

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.

INDICATION AND IMPORTANT SAFETY INFORMATION FOR TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor)

What is TRIKAFTA?

TRIKAFTA is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients aged 12 years and older who have at least one copy of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene or another mutation that is responsive to treatment with TRIKAFTA. Patients should talk to their doctor to learn if they have an indicated CF gene mutation. It is not known if TRIKAFTA is safe and effective in children under 12 years of age.

Patients should not take TRIKAFTA if they take certain medicines or herbal supplements, such as: the antibiotics rifampin or rifabutin; seizure medications such as phenobarbital, carbamazepine, or phenytoin; or St. John’s wort.

Before taking TRIKAFTA, patients should tell their doctor about all of their medical conditions, including if they: have kidney problems; have or have had liver problems; are pregnant or plan to become pregnant because it is not known if TRIKAFTA will harm an unborn baby; or are breastfeeding or planning to breastfeed because it is not known if TRIKAFTA passes into breast milk.

TRIKAFTA may affect the way other medicines work, and other medicines may affect how TRIKAFTA works. Therefore, the dose of TRIKAFTA may need to be adjusted when taken with certain medications. Patients should especially tell their doctor if they take antifungal medications such as ketoconazole, itraconazole, posaconazole, voriconazole, or fluconazole; or antibiotics such as telithromycin, clarithromycin, or erythromycin.

TRIKAFTA can cause dizziness in some people who take it. Patients should not drive a car, operate machinery, or do anything that needs them to be alert until they know how TRIKAFTA affects them.

Patients should avoid food or drink that contains grapefruit while they are taking TRIKAFTA.

TRIKAFTA can cause serious side effects, including:

High liver enzymes in the blood, which is a common side effect in people treated with TRIKAFTA. The patient's doctor will do blood tests to check their liver before they start TRIKAFTA, every 3 months during the first year of taking TRIKAFTA, and every year while taking TRIKAFTA. Patients should call their doctor right away if they have any of the following symptoms of liver problems: pain or discomfort in the upper right stomach (abdominal) area; yellowing of the skin or the white part of the eyes; loss of appetite; nausea or vomiting; dark, amber-colored urine.

Abnormality of the eye lens (cataract) in some children and adolescents treated with TRIKAFTA. If the patient is a child or adolescent, their doctor should perform eye examinations before and during treatment with TRIKAFTA to look for cataracts.

The most common side effects of TRIKAFTA include headache, diarrhea, upper respiratory tract infection (common cold) including stuffy and runny nose, stomach (abdominal) pain, inflamed sinuses, increase in liver enzymes, increase in a certain blood enzyme called creatine phosphokinase, rash, flu (influenza), and increase in blood bilirubin.

These are not all the possible side effects of TRIKAFTA. **Please click the product link to see the full Prescribing Information for** 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 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. 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 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, including, without limitation, statements made by Dr. Carmen Bozic in this press release, statements regarding our plans to submit an MAA in the EU and other global regulatory filings in additional markets, our expectations regarding the number of patients newly eligible for TRIKAFTA, and statements regarding the potential benefits of 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 the sNDA to expand the use of TRIKAFTA to include children ages 6 through 11 could not be approved on a timely basis, or at all, 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 (SEC) 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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