Vertex to Present Data Highlighting Long-Term Benefits of TRIKAFTA® at the North American Cystic Fibrosis Conference

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BOSTON--(BUSINESS WIRE)--Nov. 2, 2023-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that data on TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor), which can treat the underlying cause of cystic fibrosis (CF) in ~90% of people with the disease, will be presented at the 2023 North American Cystic Fibrosis Conference (NACFC) taking place November 2-4, 2023 in Phoenix, Arizona. Presentations will highlight results from two open-label extension studies demonstrating the long-term clinical benefits and safety profile of TRIKAFTA® among CF pediatric populations.

Vertex will present interim data on TRIKAFTA® from its longest pediatric follow-up study to date. This interim analysis through Week 144 of a 192-week open-label extension study of TRIKAFTA® in children 6 years of age and older with CF and at least one *F508del* allele showed sustained improvements in CFTR function and respiratory symptoms following the pivotal study. In addition, there was no evidence of a decline in lung function during the treatment period of 3.5 years in the pivotal and extension studies. The data also showed a continued favorable safety profile, with no new safety findings (Poster #133).

In addition, 48-week interim results from a 192-week open-label study of TRIKAFTA® in children 2 years of age and older with cystic fibrosis and at least one *F508del* allele showed that the clinical benefits from the parent pivotal study were sustained, including improvements in lung function and CFTR function. There was also evidence suggesting continued improvement in exocrine pancreatic function with ongoing use of TRIKAFTA®. TRIKAFTA® continued to be generally safe and well tolerated through an additional 48 weeks of treatment (Poster #137).

“TRIKAFTA has fundamentally changed the course of CF treatment, and the data presented at this year’s NACFC is further evidence of the long-term benefits it brings to children with this disease,” said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex. “We remain committed to developing medicines that can be given to people with CF as young as possible, because we now know treating the disease early in life can slow disease progression.”

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 88,000 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 30s, but with treatment, projected survival is improving.

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

INDICATIONS AND USAGE

TRIKAFTA (elexacaftor/tezacaftor/ivacaftor and ivacaftor) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients aged 2 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 2 years of age.

IMPORTANT SAFETY INFORMATION

Before taking TRIKAFTA, patients should tell their doctor about all of their medical conditions, including if they: are allergic to TRIKAFTA or any ingredients in TRIKAFTA, 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.

Patients should tell their doctor about all the medicines they take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. TRIKAFTA may affect the way other medicines work, and other medicines may affect how TRIKAFTA works. The dose of TRIKAFTA may need to be adjusted when taken with certain medicines. Patients should ask their doctor or pharmacist for a list of these medicines if they are not sure. Patients should especially tell their doctor if they take: antibiotics such as rifampin or rifabutin; seizure medicines such as phenobarbital, carbamazepine, or phenytoin; St. John's wort; antifungal medicines including ketoconazole, itraconazole, posaconazole, voriconazole, or fluconazole; antibiotics including telithromycin, clarithromycin, or erythromycin.

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

TRIKAFTA can cause serious side effects, including:

Liver damage and worsening of liver function in patients with severe liver disease that can be serious and may require transplantation. Liver damage has also happened in patients without liver disease.

High liver enzymes in the blood, which is a common side effect in patients treated with TRIKAFTA**.** These can be serious and may be a sign of liver injury. 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.

Serious allergic reactions have happened to patients who are treated with TRIKAFTA. Call your healthcare provider or go to the emergency room right away if you have any symptoms of an allergic reaction. Symptoms of an allergic reaction may include: rash or hives; tightness of the chest or throat or difficulty breathing; swelling of the face, lips and/or tongue; difficulty swallowing; and light-headedness or dizziness.

Abnormality of the eye lens (cataract) has been noted 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, upper respiratory tract infection (common cold) including stuffy and runny nose, stomach (abdominal) pain, diarrhea, rash, increase in liver enzymes, increase in a certain blood enzyme called creatine phosphokinase, flu (influenza), inflamed sinuses, and increase in blood bilirubin.

Patients should tell their doctor if they have any side effect that bothers them or that does not go away. These are not all the possible side effects of TRIKAFTA. For more information, patients should ask their doctor or pharmacist.

Please click here to see the full U.S. Prescribing Information for TRIKAFTA (elexacaftor/tezacaftor/ivacaftor and ivacaftor).

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 clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, acute and neuropathic pain, type 1 diabetes and alpha-1 antitrypsin deficiency.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters 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 14 consecutive years on Science magazine's Top Employers list and one of Fortune's 100 Best Companies to Work For. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on LinkedIn, Facebook, Instagram, YouTube and Twitter/X.

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 by Carmen Bozic, M.D., in this press release, and our plans to present data on TRIKAFTA® at the NACFC, including long-term clinical benefits and safety profile in CF pediatric populations. 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 research and development programs may not support registration or further development of its compounds due to safety, efficacy, 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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