European Commission Approves KAFTRIO® in Combination With Ivacaftor for the Treatment of Children With Cystic Fibrosis Ages 2 Through 5

November 23, 2023

-More than 1,200 children are newly eligible for a medicine that could treat the underlying cause of their disease-

BOSTON--(BUSINESS WIRE)--Nov. 23, 2023-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the European Commission has granted approval for the label expansion of KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor for the treatment of children with cystic fibrosis (CF) ages 2 through 5 years old who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

“In addition to data from clinical trials, long-term and real-world data have demonstrated the significant clinical benefit of KAFTRIO in eligible people living with CF, and today’s news means that young children across Europe can now benefit from this important medicine,” said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer, Vertex.

“As CF starts in early childhood and is a progressive disease, it is important to treat people with CF as early as possible. With the approval of KAFTRIO for children as young as 2 years, we can now treat young children with a medicine that has the potential to slow disease progression by addressing the underlying cause of the disease,” said Professor Marcus A. Mall, M.D., Head of the Department of Pediatric Respiratory Medicine, Immunology and Critical Care Medicine at Charité Universitätsmedizin Berlin.

As a result of existing reimbursement agreements in Austria, Denmark, Ireland, Norway, Latvia, and Sweden, eligible patients in these countries will have access to the expanded indication for KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor shortly following regulatory approval by the European Commission. Vertex will continue to work with reimbursement authorities across the European Union to ensure access for all eligible patients. In the U.K., following MHRA approval on November 15, 2023, and as a result of the existing reimbursement agreement between Vertex and the NHS, children ages 2 years and above in the U.K. have access to this expanded indication for KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor.

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 KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in Combination With 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. KAFTRIO® (ivacaftor/tezacaftor /elexacaftor) in combination with 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 ivacaftor, tezacaftor and elexacaftor help hydrate and clear mucus from the airways.

KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in combination with ivacaftor is approved in the European Union 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 *CFTR* gene.

For complete product information, please see the Summary of Product Characteristics that can be found on www.ema.europa.eu.

U.S. INDICATION AND IMPORTANT SAFETY INFORMATION FOR 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 approved medicines that treat the underlying causes of multiple chronic, life-shortening genetic diseases — cystic fibrosis, sickle cell disease and transfusion-dependent beta thalassemia — and continues to advance clinical and research programs in these diseases. Vertex also 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 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, including, without limitation, statements made by Carmen Bozic, M.D., and Professor Marcus A. Mall, M.D., in this press release and statements regarding the number of children newly eligible for KAFTRIO, our expectations for patient access to KAFTRIO, including statements about our efforts to ensure patient access in the EU, and statements regarding the anticipated benefits of KAFTRIO. 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 risks listed under the heading “Risk Factors” in Vertex's annual report and in subsequent filings filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com and 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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