Vertex Announces U.S. FDA Approval for KALYDECO® (ivacaftor) to Treat Eligible Infants With CF Ages 1 Month and Older

May 3, 2023

-First and only CFTR modulator approved for this age group-

BOSTON--(BUSINESS WIRE)--May 3, 2023-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced the U.S. Food and Drug Administration (FDA) approved KALYDECO® (ivacaftor) for use in children with cystic fibrosis (CF) ages 1 month to less than four months old who have at least one mutation in their cystic fibrosis transmembrane conductance regulator (*CFTR*) gene that is responsive to KALYDECO® based on clinical and/or in vitro assay data. KALYDECO® is already approved in the U.S. and EU for the treatment of CF in patients ages four months and older.

“Treating the underlying cause of cystic fibrosis as early as possible is important, and this approval, the first for a CFTR modulator in this age group, means families will now have a medicine for eligible infants,” said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer, Vertex.

“As a physician caring for infants and children with cystic fibrosis, I see the importance of initiating therapies early in life that may slow disease progression,” said Margaret Rosenfeld, M.D., M.P.H., Seattle Children’s Research Institute and Department of Pediatrics, University of Washington School of Medicine and one of the Principal Investigators for the KALYDECO® study in less than 24-month-olds. “Today's approval provides many families and caregivers comfort in knowing that there is a highly effective modulator therapy available for their babies with CF.”

The approval was supported by a cohort in the Phase 3, 24-week, open-label study to evaluate the safety, pharmacokinetics and pharmacodynamics of ivacaftor in subjects with CF who are less than 24 months of age and have an ivacaftor-responsive *CFTR* mutation. This cohort demonstrated a safety profile similar to that observed in older children and adults.

KALYDECO® was first approved in 2012 in the U.S. and is now available in more than 30 countries. For more information on KALYDECO®, prescribing information, or patient assistance programs, visit Kalydeco.com or VertexGPS.com.

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 early 30s.

About KALYDECO® (ivacaftor)

In people with certain types of mutations in the *CFTR* gene, the CFTR protein at the cell surface does not function properly. Known as a CFTR potentiator, ivacaftor is an oral medicine designed to facilitate the ability of CFTR proteins to transport salt and water across the cell membrane, which helps hydrate and clear mucus from the airways. KALYDECO® (ivacaftor) was the first medicine to treat the underlying cause of cystic fibrosis in people with specific mutations in the *CFTR* gene.

INDICATIONS AND USAGE

KALYDECO (ivacaftor) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 1 month and older who have at least one mutation in the cystic fibrosis transmembrane conductance regulator (CFTR gene) that is responsive to KALYDECO. Patients should talk to their doctor to learn if they have an indicated CF gene mutation. It is not known if KALYDECO is safe and effective in children under 1 month of age.

IMPORTANT SAFETY INFORMATION

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

Tell your doctor about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. KALYDECO may affect the way other medicines work**,** and other medicines may affect how KALYDECO works. 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 the antibiotics rifampin or rifabutin; seizure medications such as phenobarbital, carbamazepine, or phenytoin; St. John’s wort; antifungal medicines such as ketoconazole, itraconazole, posaconazole, voriconazole, or fluconazole; or antibiotics such as telithromycin, clarithromycin, or erythromycin.

KALYDECO can cause dizziness in some people who take it. If patients experience dizziness, they should not drive or operate machines until symptoms improve.

Patients should avoid food or drink containing grapefruit while taking KALYDECO.

KALYDECO can cause serious side effects including:

High liver enzymes in the blood, which have happened in patients receiving KALYDECO. The patient's doctor will do blood tests to check their liver before starting KALYDECO, every 3 months during the first year of taking KALYDECO, and every year while taking KALYDECO. For patients who have had high liver enzymes in the past, the doctor may do blood tests to check the liver more often.

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 their skin or the white part of their eyes; loss of appetite; nausea or vomiting; or dark, amber-colored urine.

Abnormality of the eye lens (cataract), which has happened in some children and adolescents receiving KALYDECO. The patient's doctor should perform eye examinations before and during treatment with KALYDECO to look for cataracts.

The most common side effects include headache; upper respiratory tract infection (common cold), which includes sore throat, nasal or sinus congestion, and runny nose; stomach (abdominal) pain; diarrhea; rash; nausea; and dizziness.

These are not all the possible side effects of KALYDECO.

Use of KALYDECO in patients aged 1 month to less than 6 months born from a pregnancy lasting (gestational age) less than 37 weeks has not been evaluated.

Please click here to see the full Prescribing Information for KALYDECO.

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 small molecule, mRNA, cell and genetic therapies (including gene editing) 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.

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 13 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 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 and Dr. Margaret Rosenfeld in this press release, statements regarding the eligible patient population for KALYDECO®, and statements regarding the potential benefits of KALYDECO®. 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 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 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 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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