



Vertex Announces FDA Approvals of TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor), SYMDEKO® (tezacaftor/ivacaftor and ivacaftor) and KALYDECO® (ivacaftor) for Use in People With CF With Certain Rare Mutations

December 21, 2020

- More than 600 people with certain rare CF mutations are now eligible for TRIKAFTA, SYMDEKO or KALYDECO -

BOSTON--(BUSINESS WIRE)--Dec. 21, 2020-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) today announced the U.S. Food and Drug Administration (FDA) expanded the eligibility for TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor) to include people with cystic fibrosis (CF) ages 12 years and older with certain mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene that are responsive to TRIKAFTA based on *in vitro* data. SYMDEKO® (tezacaftor/ivacaftor and ivacaftor) and KALYDECO® (ivacaftor) also received approvals to include additional responsive mutations in people with CF ages 6 years and older and age 4 months and older, respectively. These approvals allow more than 600 people with CF not previously eligible for these medicines an opportunity to potentially benefit from treatment that targets the underlying cause of their disease.

"The approval for expanded use of three of our CF medicines based on our well-established *in vitro* model is a testament to the relentless commitment of our scientists to reach our goal of developing treatments for all people with CF," said Reshma Kewalramani, M.D., Chief Executive Officer and President, Vertex. "We remain as committed today to reaching every patient who might benefit from our medicines as when we first started out on this journey 20 years ago, and this important milestone now enables hundreds of people with CF access to a treatment option to address the underlying cause of their disease — many for the first time."

TRIKAFTA was previously approved for people with at least one *F508del* mutation and is now approved for 177 additional mutations; SYMDEKO is now approved for 127 additional mutations, for a total of 154 SYMDEKO-responsive mutations; and KALYDECO is now approved for an additional 59 mutations, for a total of 97 KALYDECO-responsive mutations. In addition, for certain people with CF who are currently eligible for KALYDECO, this approval allows them to also be eligible for SYMDEKO or TRIKAFTA; and similarly, for those who are currently eligible for SYMDEKO, this approval allows them to also be eligible for TRIKAFTA.

The full list of mutations for TRIKAFTA, SYMDEKO and KALYDECO can be found within the updated full Prescribing Information for each respective product. In addition, people with CF and their families can search eligibility for Vertex CF medicines through [vertextreatments.com](#).

About Cystic Fibrosis

Cystic Fibrosis (CF) is a rare, life-shortening genetic disease affecting approximately 75,000 people worldwide. 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 KALYDECO® (ivacaftor), SYMDEKO® (tezacaftor/ivacaftor and ivacaftor), and TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor)

What is KALYDECO?

KALYDECO is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients

age 4 months and older who have at least one mutation in their CF 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 4 months of age.

What is SYMDEKO?

SYMDEKO is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients age 6 years and older who have two copies of the *F508del* mutation, or who have at least one mutation in the CF gene that is responsive to treatment with SYMDEKO. Patients should talk to their doctor to learn if they have an indicated CF gene mutation. It is not known if SYMDEKO is safe and effective in children under 6 years of age.

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 KALYDECO, SYMDEKO, or 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 KALYDECO, SYMDEKO, or 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 KALYDECO, SYMDEKO, or TRIKAFTA will harm an unborn baby; or are breastfeeding or planning to breastfeed because it is not known if KALYDECO, SYMDEKO, or TRIKAFTA passes into breast milk. Before taking KALYDECO, patients should tell their doctor if they drink grapefruit juice or eat grapefruit.

KALYDECO, SYMDEKO, or TRIKAFTA may affect the way other medicines work, and other medicines may affect how KALYDECO, SYMDEKO, or TRIKAFTA work. Therefore, the dose of KALYDECO, SYMDEKO, or 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.

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

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

KALYDECO, SYMDEKO, and TRIKAFTA can cause serious side effects, such as:

High liver enzymes in the blood have been reported in patients receiving KALYDECO, SYMDEKO, or TRIKAFTA. The patient's doctor will do blood tests to check their liver before starting treatment with KALYDECO, SYMDEKO, or TRIKAFTA, every 3 months during the first year of treatment, and every year while on treatment. 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) in some children and adolescents treated with KALYDECO, SYMDEKO, or TRIKAFTA. If the patient is a child or adolescent, their doctor should perform eye examinations before and during treatment with KALYDECO, SYMDEKO, or TRIKAFTA to look for cataracts.

The most common side effects of KALYDECO 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.

The most common side effects of SYMDEKO include headache, nausea, sinus congestion, and dizziness.

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 KALYDECO, SYMDEKO, or TRIKAFTA. Please click product link to see the full Prescribing Information for [KALYDECO](#), [SYMDEKO](#) or [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. Reshma Kewalramani in this press release, statements regarding the eligible patient population for TRIKAFTA, SYMDEKO and KALYDECO, our expectations regarding the number of patients newly eligible for TRIKAFTA, SYMDEKO and KALYDECO, and statements regarding the potential benefits of TRIKAFTA, SYMDEKO and 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 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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Source: Vertex Pharmaceuticals Incorporated