Vertex Receives CHMP Positive Opinion for Expanded Label for KAFTRIO® in Combination With Ivacaftor for People With Cystic Fibrosis to Include Rare Mutations

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-If approved, approximately 4,000 people with cystic fibrosis in the European Union will be eligible for a medicine that treats the underlying cause of their disease for the first time-

LONDON--(BUSINESS WIRE)--Feb. 28, 2025-- Vertex Pharmaceuticals (Nasdaq: VRTX) today announced that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion for the label expansion of KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in combination with ivacaftor for the treatment of people with cystic fibrosis (CF) aged 2 years and older who have at least one non-class I mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

"We are thrilled by the positive CHMP opinion in support of expanding the KAFTRIO in combination with ivacaftor indication to include all CF patients 2 years and older who make CFTR protein," said Fosca De Iorio, M.D., Vice President, International Medical Affairs at Vertex. "If approved, thousands of additional patients across Europe will be eligible for a CFTR modulator, bringing us closer than ever to our goal of getting treatments that address the underlying cause of the disease to all people living with CF."

In the European Union, ivacaftor/tezacaftor/elexacaftor in combination with ivacaftor is currently approved for the treatment of people with CF aged 2 years and older who have at least one copy of the *F508del* mutation in the *CFTR* gene.

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 94,000 people in North America, Europe and Australia. 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.

Today Vertex's CF medicines are treating over 68,000 people with CF across more than 60 countries on six continents. This represents 2/3 of the diagnosed people with CF eligible for CFTR modulator therapy.

Diagnosis of CF is often made by genetic testing and is confirmed by testing sweat chloride (SwCl), which measures CFTR protein dysfunction. The diagnostic threshold for CF is SwCl ≥60 mmol/L, while levels between 30-59 indicate CF is possible and more testing may be needed to make the diagnosis of CF. A SwCl level of <30 mmol/L is seen in people who carry one copy of a *CFTR* gene mutation but do not have any manifestation of disease (carriers). Higher levels of SwCl are associated with more severe disease. Restoring CFTR function leads to lower levels of SwCl. SwCl levels below 60 mmol/L are associated with improved outcomes such as better and more stable lung function, fewer pulmonary exacerbations, better quality of life and improved survival. Restoring SwCl levels below 30 mmol/L has long been the ultimate treatment goal for Vertex, as levels below 30 mmol/L are considered normal and are typical of CF carriers who do not have disease.

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.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases and conditions. The company has approved therapies for cystic fibrosis, sickle cell disease, transfusion-dependent beta thalassemia and acute pain, and it continues to advance clinical and research programs in these areas. 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 neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, primary membranous nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes and myotonic dystrophy type 1.

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, Latin America and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 15 consecutive years on Science magazine's Top Employers list and one of Fortune’s 100 Best Companies to Work For.

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 Fosca De Iorio, M.D., in this press release, statements regarding the expected eligible patient population for KAFTRIO, if approved, and statements regarding the potential 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, among other things, that data from the company’s development programs may not support a label expansion for KAFTRIO, that regulatory authorities in the EU may not approve a label expansion for KAFTRIO on a timely basis or at all, and other 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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