European Commission Approves KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in Combination With Ivacaftor to Treat Children With Cystic Fibrosis Ages 6 to 11 Years

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- More than 1,500 children now eligible to receive a treatment targeting the underlying cause of cystic fibrosis for the first time -

LONDON--(BUSINESS WIRE)--Jan. 11, 2022-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the European Commission has granted approval for the label extension of KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor for the treatment of cystic fibrosis (CF) in patients ages 6 through 11 years old who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

“We are delighted that KAFTRIO (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor is now approved for these young patients in the European Union. It provides a new treatment option for physicians to help treat the underlying cause of this devastating disease early in life,” said Reshma Kewalramani, M.D., Chief Executive Officer and President at Vertex. “This important milestone brings us one step closer to our ultimate goal of developing treatments for all patients living with CF.”

“Ivacaftor/tezacaftor/elexacaftor plus ivacaftor has shown clinical benefit since its availability last year for people with CF ages 12 and above,” said Professor Marcus A. Mall, M.D., Head of the Department of Pediatric Respiratory Medicine, Immunology and Critical Care Medicine at Charité University Medical Center Berlin. “CF is a progressive disease, in which symptoms and organ damage manifest very early in life. As a physician, I welcome the approval of this medicine for this younger age group, as it will help us treat eligible children with CF as early as 6 years old.”

As a result of long-term reimbursement agreements in Austria, Northern Ireland and Denmark, and provisions for access in health care systems such as Germany, 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 bodies across the European Union to ensure access for all eligible patients.

This medicine has also been approved by regulatory authorities in New Zealand and in Switzerland, where it is known as TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor), for people with CF ages 6 and above, and we continue to work closely with reimbursement bodies in these countries to ensure access for all eligible patients.

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 83,000 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 the 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 KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in A Combination Regimen 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.

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. 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 disease. In addition, Vertex has a rapidly expanding pipeline of cell and genetic 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 12 consecutive years on Science magazine's Top Employers list, one of the 2021 Seramount (formerly Working Mother Media) 100 Best Companies, 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 https://global.vrtx.com/ or follow us on

Twitter and LinkedIn.

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 Reshma Kewalramani, M.D., Chief Executive Officer and President at Vertex, and Professor Marcus A. Mall, M.D., Head of the Department of Pediatric Respiratory Medicine, Immunology and Critical Care Medicine at Charité University Medical Center Berlin, in this press release and statements regarding the estimated number of children eligible for a medicine that can treat the underlying cause of their disease for the first time, our beliefs regarding the benefits of our medicines, and the anticipated patient access to KAFTRIO® in combination with ivacaftor. 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 https://global.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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