Vertex Receives CHMP Positive Opinion for KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in Combination With Ivacaftor in Children With Cystic Fibrosis Ages 6 Through 11

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- If approved, more than 1,500 children would be eligible for a medicine that can treat the underlying cause of their disease for the first time -

LONDON--(BUSINESS WIRE)--Nov. 12, 2021-- Vertex Pharmaceuticals Incorporated (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 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.

"Today marks an important milestone for the treatment of children with CF in Europe. If approved, KAFTRIO (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor will offer physicians a new treatment option for these young patients to help combat this life-shortening condition as early as possible," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex.

"The clinical data for ivacaftor/tezacaftor/elexacaftor plus ivacaftor in people with CF ages 6 through 11 with eligible CF genotypes demonstrated improvements in lung function, sweat chloride and respiratory symptoms and a safety and tolerability profile consistent with that observed in patients ages 12 years and older," 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. "This medicine has already made a big impact on the lives of eligible people ages 12 years and above in Europe. The CF community is now looking forward to it being available for younger patients too, to enable treatment as early as possible in life."

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.

KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in combination with ivacaftor is approved in the European Union for the treatment of cystic fibrosis (CF) in patients ages 12 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. 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 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 Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer 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 our expectations for regulatory approval and a label extension for KAFTRIO in combination with ivacaftor, the estimated number of children eligible for a medicine that can treat the underlying cause of their disease for the first time and our beliefs regarding the benefits of our medicines. 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 extension for KAFTRIO in combination with ivacaftor, the European Commission may not approve the company's post marketing applications for KAFTRIO in combination with ivacaftor 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 https://global.vrtx.com/ and 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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