Vertex Announces Reimbursement Agreement in Spain for KAFTRIO® (ivacaftor/tezacaftor /elexacaftor) in Combination With Ivacaftor to Treat People With Cystic Fibrosis 12 Years and Older With At Least One F508del Mutation in the CFTR Gene

November 19, 2021

– With this reimbursement agreement approximately 700 people with cystic fibrosis now have access to a CFTR modulator therapy for the first time –

LONDON--(BUSINESS WIRE)--Nov. 19, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the Spanish government has approved terms for the national reimbursement of KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor for the treatment of cystic fibrosis (CF) for eligible patients. The agreement covers people with CF ages 12 years and older who have at least one copy of the *F508del* mutation, regardless of the other mutation type in the *CFTR* gene. KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor can be prescribed to eligible patients by treating physicians once the medicine is listed in the national Official Gazette *Nomenclator.*

"The formalized agreement is an important milestone for people living with cystic fibrosis in Spain. We are pleased that the Ministry of Health recognized the value of KAFTRIO (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor. Today's announcement means that the medicine can be prescribed to eligible patients in Spain when listed," said Ludovic Fenaux, Senior Vice President, Vertex International. "We would like to thank the Ministry of Health for its collaborative approach, as well as the CF community for their important input during this process."

With this agreement, Spain joins the group of over 25 countries — such as Ireland, Germany, Austria, Slovenia, Croatia, Luxembourg, Denmark, Finland, France, Portugal, Italy, Switzerland and the U.K. — where eligible patients with CF have access to the triple combination therapy.

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 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 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 Ludovic Fenaux, Senior Vice President, Vertex International, in this press release and statements regarding our beliefs about the

eligible patient population that will have access to KAFTRIO® in combination with ivacaftor, including patients that will now have access to a CFTR modulator therapy for the first time in Spain 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 risks and uncertainties that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks are 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.

(VRTX-GEN)

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