Vertex Announces European Commission Approval for KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in Combination With Ivacaftor to Treat Cystic Fibrosis Patients 12 Years and Older With At Least One F508del Mutation in the CFTR gene

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- New indication includes people ages 12 years and older who have one copy of the F508del mutation regardless of the other mutation type -

- People with gating (F/G) or residual function (F/RF) mutations now eligible for the triple combination therapy -

LONDON--(BUSINESS WIRE)--Apr. 28, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the European Commission has granted approval of the label extension for KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor for the treatment of cystic fibrosis (CF) in all patients ages 12 years and older who have at least one *F508del* mutation in the cystic fibrosis transmembrane conductance regulator *(CFTR)* gene. With this extension of the indication, CF patients 12 years and older who are heterozygous for the *F508del-CFTR* mutation and a residual function (F/RF) or gating mutation (F/G) are eligible for the triple combination therapy for the first time, and the majority of people ages 12 years and older with CF in Europe are now eligible for KAFTRIO.

“This indication extension is important as CF patients in Europe with gating and residual function mutations will have access to KAFTRIO for the first time,” said Reshma Kewalramani, M.D., Chief Executive Officer and President at Vertex. “We look forward to working with health authorities and governments to ensure that all eligible patients who can benefit from this medicine have access as soon as possible.”

In both the United States and Australia, where the triple combination therapy is known as TRIKAFTA® (elexacaftor/tezacaftor/ivacaftor and ivacaftor), it is already licensed for the treatment of people with CF ages 12 years and older who have at least one copy of the *F508del* mutation in the *CFTR* gene**,** regardless of the other mutation type.

“In clinical trials ivacaftor/tezacaftor/elexacaftor plus ivacaftor showed positive significant results in people with cystic fibrosis ages 12 years and older who have at least one copy of the most common gene mutation (*F508del*) with the latest results showing clinical benefits in individuals who have an additional ‘gating’ (F/G) or ‘residual function’ (F/RF) gene mutation. It is welcome news for the CF community that the European regulatory authority has extended the licensed indications for this therapy based on the latest trial results which means that additional patients will gain access to this medication,” said Dr Peter Barry, Honorary Senior Lecturer at The University of Manchester.

As a result of long-term reimbursement agreements in the Republic of Ireland, Northern Ireland, Denmark and Luxembourg, and provisions for access in health care systems such as Germany, eligible patients in these countries will have access to the expanded indication for the triple combination regimen shortly following regulatory approval by the European Commission.

About Cystic Fibrosis

Cystic Fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 80,000 people globally.[1] CF is a progressive, multi-system disease that affects the lungs, liver, gastrointestinal (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.

About KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a Combination With Ivacaftor

KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor 150 mg was developed 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 cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. Ivacaftor/tezacaftor/elexacaftor is designed to increase the quantity and function of the F508del-CFTR protein at the cell surface. The latest approved EU licensed indication for ivacaftor/tezacaftor/elexacaftor was supported by positive results of three global Phase 3 studies in people ages 12 years and older with CF: a 24-week Phase 3 study (Study 445-102) in 403 people with one *F508del* mutation and one minimal function mutation (*F*/MF), a four-week Phase 3 study (Study 445-103) in 107 people with two *F508del* mutations (*F/F*), and a Phase 3 study (Study 445-104) in 258 people heterozygous for the *F508del-CFTR* mutation and a *CFTR* gating mutation (F/G) or a residual function mutation (F/RF).[2]

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 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign.

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 and Dr. Peter Barry in this press release, statements regarding the eligible patient population in Europe and our expectations regarding the timing of access to KAFTRIO® in combination with ivacaftor for eligible patients ages 12 years and older in Europe. 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 registration or further development of its compounds due to safety, efficacy or other reasons, risks related to commercializing KAFTRIO® in combination with ivacaftor in Europe, and other risks listed under the heading “Risk Factors” in Vertex's annual report filed with the Securities and Exchange Commission 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 or the scientific data presented. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)

[1] Vertex. Data on File. Global CF EPI. 2021.

[2] EMA. Kaftrio Summary of product characteristics. Vertex Pharmaceuticals (Ireland) Limited. Available at: https://www.ema.europa.eu/en/documents/product-information/kaftrio-epar-product-information_en.pdf
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Vertex Pharmaceuticals Incorporated
Investors:
InvestorInfo@vrtx.com
or
617-961-7163

Media:
mediainfo@vrtx.com
or
International: +44 20 3204 5275
or
U.S.: 617-341-6992

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