Vertex Announces National Reimbursement Agreement in France for KAFTRIO® (ivacaftor/tezacaftor /elexacaftor) and SYMKEVI® (tezacaftor/ivacaftor) for Eligible Cystic Fibrosis Patients

June 28, 2021

- With this reimbursement agreement more than 1,500 patients now have access to a CFTR modulator therapy for the first time -

LONDON--(BUSINESS WIRE)--Jun. 28, 2021-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced a national reimbursement agreement with the French Health Authorities for the cystic fibrosis (CF) medicines KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with ivacaftor and SYMKEVI® (tezacaftor/ivacaftor) in combination with ivacaftor. Both medicines will be available for all eligible patients once the agreement has been published in the French Official Journal.

"Today's announcement represents a major milestone for CF patients in France. Through this national reimbursement agreement, eligible patients 12 years and older now have access to KAFTRIO and SYMKEVI. For those living with CF, we are delighted to have reached this agreement so quickly and that the French Health Authorities have recognized the value of both medicines," said Ludovic Fenaux, Senior Vice President, Vertex International.

The reimbursement agreement enables broad access to KAFTRIO® for people with CF ages 12 years and older with one *F508del* mutation and one minimal function mutation. The triple combination therapy will also be reimbursed for patients who are homozygous for the *F508del* mutation in the *CFTR* gene, representing a new therapeutic option for treating physicians. In November 2020, the Transparency Commission (TC) of the French National Authority for Health (HAS) granted KAFTRIO® an ASMR 2 rating, which indicates "a significant improvement in medical service rendered." Of the 250 first-time listing medicines issued in 2019 by the TC, only two received such a rating.

Under the terms of the new reimbursement agreement, SYMKEVI® will be reimbursed for people with CF ages 12 years and older with one *F508del* mutation and one of the mutations resulting in residual activity (F/RF) of the CFTR protein as listed in the Summary of Product Characteristics (SmPC). It will also be funded for patients who are homozygous for the *F508del* mutation in the *CFTR* gene.

About Cystic Fibrosis

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

For complete product information, please see the Summary of Product Characteristics that can be found on www.ema.europa.eu.

About SYMKEVI® (tezacaftor/ivacaftor) in Combination With Ivacaftor

Some mutations result in CFTR protein that is not processed or folded normally within the cell, and that generally does not reach the cell surface. Tezacaftor is designed to address the trafficking and processing defect of the CFTR protein to enable it to reach the cell surface and ivacaftor is designed to enhance the function of the CFTR protein once it reaches the cell surface.

For complete product information including dosing guidance, 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 Ludovic Fenaux, Senior Vice President, Vertex International in this press release and statements regarding the reimbursement of, availability of and access to KAFTRIO® and SYMKEVI® for certain patients, the estimated number of patients eligible for a CFTR modulator therapy 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 expressed or implied 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, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20210628005507/en/

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

Source: Vertex Pharmaceuticals Incorporated