Vertex Announces Expansion of Reimbursement Agreement With NHS England to Include KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in Combination With KALYDECO® (ivacaftor)

June 30, 2020

-CF patients in England will be among the first in Europe to benefit from access to KAFTRIO®, if the medicine is approved by the European Commission-

LONDON--(BUSINESS WIRE)--Jun. 30, 2020-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that it has expanded its reimbursement agreement with NHS England for Vertex's cystic fibrosis medicines to include KAFTRIO ® (ivacaftor/tezacaftor/elexacaftor), in a combination regimen with KALYDECO® (ivacaftor) 150 mg, ahead of the medicine's anticipated approval by the European Commission.

The new expanded agreement includes reimbursed access to Vertex's currently licensed medicines — KALYDECO® (ivacaftor), ORKAMBI® (lumacaftor/ivacaftor) and SYMKEVI® (tezacaftor/ivacaftor), as well as the triple combination therapy if approved — and any future additional licensed indications for all of these medicines.

Reshma Kewalramani, M.D., Chief Executive Officer and President at Vertex, said, "I'm pleased that NHS England has recognized the value of KAFTRIO, and that Vertex and NHS England have been able to work quickly, collaboratively and flexibly to expand the existing reimbursement agreement to include the triple combination therapy in advance of the medicine being licenced. This will ensure that eligible patients in England will be among the first in Europe to benefit from access to this innovative medicine upon approval."

The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) recently adopted a positive opinion for KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with KALYDECO® (ivacaftor) 150 mg to treat people with cystic fibrosis (CF) ages 12 and older with one *F508del* mutation and one minimal function mutation (F/MF) or two *F508del* mutations (F/F) in the *cystic fibrosis transmembrane conductance regulator* (*CFTR*) gene.

As part of the agreement with NHS England, Vertex has committed to submit ORKAMBI®, SYMKEVI® and KAFTRIO® to the National Institute for Health and Care Excellence (NICE) within an agreed upon timetable, allowing for a period of real-world data collection on the medicines.

Vertex will be working closely with the authorities in Northern Ireland, Wales and Scotland with the aim of securing an equivalent agreement in those countries as soon as possible.

Vertex's CF medicines are reimbursed in more than 20 countries around the world including Australia, France, Germany, the Republic of Ireland, Italy, Switzerland, Spain, Denmark, the UK and the U.S.

About Cystic Fibrosis
Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting approximately 75,000 people worldwide. 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 Regimen With KALYDECO® (ivacaftor)

KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in a combination regimen with KALYDECO® (ivacaftor) is an investigational medicine 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. It is designed to increase the quantity and function of the *F508del*-CFTR protein at the cell surface. It recently received a CHMP positive opinion, with the EU license expected over the next few months.

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 genetic and cell 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, UK. 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 10 consecutive years on Science magazine's Top Employers list and top five on the 2019 Best Employers for Diversity list by Forbes. For company updates and to learn more about Vertex's history of innovation, visit https://www.vrtx.com

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, the statements by Dr. Kewalramani in this press release and statements regarding our expectations for the patient population that will be able to access Vertex's medicines, the timing of such access, our commitment to submit ORKAMBI, SYMKEVI and KAFTRIO to NICE for a period of real-world data collection and our plans to secure additional agreements with other countries. 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, risks related to obtaining approval for and commercializing our medicines in Europe, and other risks listed under Risk Factors in Vertex's annual report and subsequent quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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