Vertex Announces European Commission Approval for KALYDECO® (ivacaftor) for Children and Adolescents With Cystic Fibrosis Between the Ages of 6 Months and 18 Years With the R117H Mutation in the CFTR Gene

June 10, 2020

- KALYDECO® (ivacaftor) is the first and only approved medicine in Europe to treat the underlying cause of cystic fibrosis in patients with the R117H mutation, the most common residual function mutation, in children as young as 6 months of age -

LONDON--(BUSINESS WIRE)--Jun. 10, 2020-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the European Commission has granted approval of the label extension for KALYDECO® (ivacaftor) to include the treatment of children and adolescents with cystic fibrosis (CF), ages 6 months and older and weighing at least 5 kg, who have the *R117H* mutation in the *cystic fibrosis transmembrane conductance regulator* (*CFTR*) gene – the most common residual function mutation underlying CF.

“A little over eight years ago, KALYDECO® was approved as the first and only medicine to treat the underlying cause of cystic fibrosis in patients with specific mutations,” said Reshma Kewalramani, M.D., Chief Executive Officer and President at Vertex. “Since then, it’s been our goal to ensure that as many people with CF as possible are able to benefit from our treatments, and today’s label extension means that approximately 500 young patients in Europe, who have long awaited a treatment option, are now eligible for KALYDECO®.”

Now approved, KALYDECO® (ivacaftor) will be immediately available to additional eligible patients in Germany and shortly in countries where respective long-term reimbursement agreements have been previously secured. Vertex will work closely with all other relevant government authorities to secure access for eligible patients as quickly as possible.

In Europe, KALYDECO® (ivacaftor) is already approved for the treatment of people with CF ages 18 and older with the *R117H* mutation, and in infants ages 6 months and older weighing at least 5 kg who have one of the following mutations in the *CFTR* gene: *G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N* or *S549R.*

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 KALYDECO® (ivacaftor)

Ivacaftor is the first medicine to treat the underlying cause of CF in people with specific mutations in the *CFTR* gene. Known as a CFTR potentiator, ivacaftor is an oral medicine designed to keep CFTR proteins at the cell surface open longer to improve the transport of salt and water across the cell membrane, which helps hydrate and clear mucus from the airways.

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.

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. Kewalramani in this press release, and statements regarding our expectations for the availability of KALYDECO in Europe, and our plans for securing access to KALYDECO for eligible patients 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 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 commercializing KALYDECO 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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