Vertex Announces European Commission Approval for SYMKEVI® (tezacaftor/ivacaftor) With KALYDECO® (ivacaftor) for Eligible Children With Cystic Fibrosis Ages 6-11 Years

November 27, 2020

- The combination therapy is a new treatment option for CF patients who are homozygous for F508del -

- The only medicine to treat the underlying cause of CF in this age group with one F508del mutation and one of 14 residual function mutations -

LONDON--(BUSINESS WIRE)--Nov. 27, 2020-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the European Commission has granted approval of the label extension for SYMKEVI® (tezacaftor/ivacaftor) with KALYDECO® (ivacaftor), to include the treatment of cystic fibrosis (CF) in patients ages 6 years and older who have two copies of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene or one copy of the *F508del* mutation and one copy of one of 14 mutations in the *CFTR* gene that result in residual CFTR activity: *P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G*, and *3849+10kbC→T*.

"With this approval, children with CF in Europe ages 6 to 11 years with the most common mutation, F508del, have a new treatment option and children with certain residual function mutations will, for the first time, have a treatment option available that addresses the underlying cause of their CF," said Reshma Kewalramani, M.D., Chief Executive Officer and President at Vertex. "Today's approval brings us closer to our ultimate goal of providing medicines for all people with CF."

SYMKEVI® (tezacaftor/ivacaftor) with KALYDECO® (ivacaftor) will be immediately available to additional eligible patients in Germany and will be available shortly in countries that have entered into innovative long-term reimbursement agreements with Vertex, including the UK, Denmark and the Republic of Ireland. In all other countries, Vertex will work closely with relevant authorities in Europe to secure access for eligible patients.

In Europe, SYMKEVI® (tezacaftor/ivacaftor) with KALYDECO® (ivacaftor) is already approved for the treatment of people with CF ages 12 years and older who have either two copies of the *F508del* mutation in the *CFTR* gene, or one copy of the *F508del* mutation and a copy of one of the following 14 mutations in which the *CFTR* gene results in residual CFTR protein activity: *P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G,* and *3849+10kbC→T.*

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 SYMKEVI® in combination with KALYDECO®

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 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. 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. 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 Dr. Reshma Kewalramani in this press release, statements regarding the eligible patient population in Europe, our expectations regarding the timing of access to SYMKEVI in combination with KALYDECO for eligible patients ages 6-11 years across countries in Europe, and our plans to secure access to SYMKEVI in combination with KALYDECO for additional eligible patients ages 6-11 years 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 SYMKEVI in combination with KALYDECO in Europe, and other risks listed under Risk Factors in Vertex's most recent annual report and subsequent quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. 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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Source: Vertex Pharmaceuticals Incorporated