Vertex Announces European Commission Approval for KALYDECO® to Treat Infants With Cystic Fibrosis Ages 1 Month and Older

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- KALYDECO ® is the first and only medicine approved in the EU in this age group to treat the underlying cause of cystic fibrosis for specific mutations in the CFTR gene -

LONDON--(BUSINESS WIRE)--Apr. 26, 2024-- Vertex Pharmaceuticals (Nasdaq: VRTX) today announced that the European Commission has granted approval for the label expansion of KALYDECO® (ivacaftor) for the treatment of infants down to 1 month of age with cystic fibrosis (CF) who have one of the following mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene: *R117H*, *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R*.

"Today's approval is an important milestone for the cystic fibrosis community. Treating CF early in life can potentially slow the progression of the disease, which is why it is so important to start treatment from a very young age," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer, Vertex.

As a result of existing access agreements in Austria, Czech Republic, Denmark, Ireland, Norway, Sweden, and The Netherlands, eligible patients will have access to the expanded indication of KALYDECO® (ivacaftor) shortly following regulatory approval by the European Commission. Vertex will continue to work with reimbursement authorities across the European Union to ensure access for all other eligible patients. In the U.K., following MHRA approval at the end of 2023, and as a result of the existing reimbursement agreement between Vertex and the National Health Service, eligible infants ages 1 month and older in the U.K. have access to this expanded indication for KALYDECO® (ivacaftor).

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 92,000 people globally. CF is a progressive, multi-organ disease that affects the lungs, liver, pancreas, GI tract, sinuses, sweat glands 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, and these mutations can be identified by a genetic test. While there are many different types of *CFTR* mutations that can cause the disease, the vast majority of people with CF have at least one *F508del* mutation. *CFTR* mutations lead to CF by causing CFTR protein to be defective or by leading to a shortage or absence of CFTR protein 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, chronic lung infections and progressive lung damage that eventually leads to death for many patients. The median age of death is in the 30s, but with treatment, projected survival is improving.

About KALYDECO® (ivacaftor)

In people with certain types of mutations in the *CFTR* gene, the CFTR protein at the cell surface does not function properly. Known as a CFTR potentiator, ivacaftor is an oral medicine designed to facilitate the ability of CFTR proteins to transport salt and water across the cell membrane, which helps hydrate and clear mucus from the airways. KALYDECO® (ivacaftor) was the first medicine to treat the underlying cause of cystic fibrosis in people with specific mutations in the *CFTR* gene.

KALYDECO® (ivacaftor) is a prescription medicine for the treatment of people with CF aged at least 1 month and weighing at least 3 kg who have one of the following mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene: *R117H*, *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R*.

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 approved medicines that treat the underlying causes of multiple chronic, life-shortening genetic diseases — cystic fibrosis, sickle cell disease and transfusion-dependent beta thalassemia — and continues to advance clinical and research programs in these diseases.Vertex also has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including acute and neuropathic pain, APOL1-mediated kidney disease, autosomal dominant polycystic kidney disease, type 1 diabetes, myotonic dystrophy type 1 and alpha-1 antitrypsin deficiency.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia, Latin America and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 14 consecutive years on Science magazine's Top Employers list and one of Fortune's 100 Best Companies to Work For. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on LinkedIn, YouTube and Twitter/X.

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 Carmen Bozic, M.D., in this press release, statements regarding the eligible patient population for KALYDECO, expectations for access to KALYDECO for eligible patients, including Vertex's plans to continue to work with reimbursement authorities across the European Union to

ensure access for eligible patients, and statements regarding the potential benefits of KALYDECO. 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 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.

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