Vertex Receives CHMP Positive Opinion for KALYDECO® for the Treatment of Infants With Cystic Fibrosis Ages 1 Month and Older

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- If approved, KALYDECO® will be the first and only medicine approved in Europe to treat the underlying cause of cystic fibrosis in babies as young as 1 month with specific mutations in the CFTR gene -

LONDON--(BUSINESS WIRE)--Feb. 23, 2024-- Vertex Pharmaceuticals (Nasdaq: VRTX) today announced that the European Medicines Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion for the label expansion of KALYDECO ® (ivacaftor) for the treatment of infants with cystic fibrosis (CF) ages 1 month to less than 4 months old 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*.

"Cystic fibrosis is a chronic, progressive disease that is present from birth, which is why it is important to treat the underlying cause of this disease as early as possible to potentially slow disease progression," said Fosca De Iorio, Vice President, International Medical Affairs at Vertex. "If the label expansion for KALYDECO is approved, this would allow eligible infants with CF to receive a treatment that targets the underlying cause of their disease at the very start of their lives."

In the European Union, KALYDECO® is already approved for the treatment of people with CF ages 4 months old and above with specific mutations in the *CFTR* gene.

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.

Diagnosis of CF is often made by genetic testing and is confirmed by testing sweat chloride (SwCl), which measures CFTR protein dysfunction. The diagnostic threshold for CF is SwCl ≥60 mmol/L, while levels between 30-59 indicate CF is possible and more testing may be needed to make the diagnosis of CF. A SwCl level of <30 mmol/L is seen in people who carry one copy of the CF gene but do not have any manifestation of disease (carriers). Higher levels of SwCl are associated with more severe disease. Restoring CFTR function leads to lower levels of SwCl.

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 used for the treatment of cystic fibrosis (CF) in patients aged at least 4 months and older who have 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 APOL1-mediated kidney disease, acute and neuropathic pain, 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 Fosca De Iorio, Vice President, International Medical Affairs at Vertex, in this press release, statements regarding the eligible

patient population for KALYDECO, 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, among other things, that data from the company's development programs may not support a label expansion for KALYDECO, that regulatory authorities in the E.U. may not approve a label expansion for KALYDECO on a timely basis or at all, 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.

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