Vertex Announces European Commission Approval for KALYDECO® (ivacaftor) in Infants With Cystic Fibrosis Ages 6 Months to Less Than 12 Months With Certain Mutations in the CFTR Gene

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- Ivacaftor is the first and only approved medicine in Europe to treat the underlying cause of cystic fibrosis in children this young -

LONDON--(BUSINESS WIRE)--Dec. 10, 2019-- Vertex Pharmaceuticals (Europe) Limited today announced that the European Commission has granted approval of the label extension for KALYDECO® (ivacaftor) to include the treatment of infants with cystic fibrosis (CF) ages 6 months to less than 12 months weighing 5 kg and more who have at least one of the following nine mutations in their cystic fibrosis transmembrane conductance regulator (*CFTR*) gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R*.

"Today's approval is another milestone in our commitment to treat all people with CF as early in life as possible, given manifestations of CF are often present at birth," said Reshma Kewalramani, M.D., Executive Vice President and Chief Medical Officer at Vertex. "For the first time, children with CF in Europe as young as 6 months with certain mutations now have access to a medicine that treats the underlying cause of their disease."

The label update is based on data from the ongoing Phase 3 open-label safety study (ARRIVAL) of children with CF who are less than 24 months of age and have a CFTR gating mutation. The study showed a safety profile similar to that observed in previous Phase 3 studies of older children and adults, and improvements in sweat chloride, a secondary endpoint.

KALYDECO® (ivacaftor) is already approved in Europe for the treatment of CF in patients ages 12 months and older who have one of the following mutations in the *CFTR* gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R*. It is also approved for the treatment of CF in patients 18 years and older who have the *R117H* mutation in the *CFTR* gene.

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.

People with CF who have specific mutations in the *CFTR* gene are currently benefiting from ivacaftor in countries across North America, Europe and in Australia.

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 four approved medicines in the U.S. and three approved medicines in Europe 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 disease. 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, the statement in the second paragraph of the press release. 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 registration or further development of its compounds due to safety, efficacy or other reasons, and other risks listed under Risk Factors in Vertex's annual report and 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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Source: Vertex Pharmaceuticals Incorporated

Vertex Pharmaceuticals Incorporated
Investors:
Michael Partridge, +1-617-341-6108
or
Zach Barber, +1-617-341-6470
or
Leah Gibson, +1-617-961-1507 or

Media: mediainfo@vrtx.com
or
North America:
Sarah D'Souza, +1-617-341-6992
or
Europe & Australia:
Patricia Dessert, +44 7543 237825