



## Vertex Receives European CHMP Positive Opinion for KALYDECO® (ivacaftor) to Treat Eligible Infants with Cystic Fibrosis as Early as 6 Months of Age

October 18, 2019

*- If approved, ivacaftor will be the first and only medicine to treat the underlying cause of cystic fibrosis for children this young -*

LONDON--(BUSINESS WIRE)--Oct. 18, 2019-- Vertex Pharmaceuticals (Europe) Limited today announces that the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion for KALYDECO® (ivacaftor), to include use in infants with cystic fibrosis (CF) ages 6 months to less than 12 months who have one of the following mutations in their *cystic fibrosis transmembrane conductance regulator (CFTR)* gene: *G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N* or *S549R*.

If the European Commission issues a favorable adoption of the EMA CHMP opinion for the extension of indication, ivacaftor will be the first and only medicine approved in Europe to treat the underlying cause of CF in patients ages 6 months to less than 12 months, who have specific mutations in the CFTR gene.

"Cystic fibrosis is present from birth and symptoms frequently manifest in infancy, which is why it is so important to treat the condition as early as possible" said Reshma Kewalramani, M.D., Executive Vice President and Chief Medical Officer at Vertex. "We are committed to treating every person with this life-limiting disease and today brings us one step closer to providing a medicine for very young children with cystic fibrosis."

The submission was supported by data from the ongoing Phase 3 open-label safety study (ARRIVAL) of children with CF ages 6 months to less than 12 months who have certain mutations in the CFTR gene. 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 key secondary efficacy endpoint.

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, sinus, sweat gland, 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)

KALYDECO® (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 three 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 medicines in other serious diseases where it has deep insight into causal human biology, such as sickle cell disease, beta thalassemia, pain, alpha-1 antitrypsin deficiency, Duchenne muscular dystrophy and APOL1-mediated kidney diseases.

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 nine 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 statements in the second and third paragraphs 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](http://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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