



Vertex Announces European Commission Approval for KALYDECO® (ivacaftor) as First and Only CFTR Modulator to Treat Eligible Infants With Cystic Fibrosis as Early as Four Months of Age

November 5, 2020

- Approval provides opportunity to treat the underlying cause of cystic fibrosis earlier than ever before in Europe -

LONDON--(BUSINESS WIRE)--Nov. 5, 2020-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the European Commission has granted approval of the label extension for KALYDECO® (ivacaftor) granules to include the treatment of infants with cystic fibrosis (CF) ages 4 months and older and weighing at least 5 kg who have the *R117H* mutation or one of the following gating (class III) mutations in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *S1251N*, *S1255P*, *S549N* or *S549R*.

"Our very first CFTR modulator, KALYDECO, was first approved eight years ago, for certain CF patients ages 6 years and older. With today's approval, babies as young as 4 months are eligible and we believe early treatment is important in managing CF," said Reshma Kewalramani, M.D., Chief Executive Officer and President, Vertex. "Today's approval is a testament to our commitment to keep going until all people with CF have a treatment option."

The label update is based on data from a cohort in the 24-week Phase 3 open-label safety study (ARRIVAL) consisting of six children with CF ages four months to less than six months who have eligible gating mutations.

KALYDECO® (ivacaftor) will be now 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.

KALYDECO® (ivacaftor) is already approved in Europe for people with CF ages 6 months and older weighing at least 5 kg who have one of the following mutations in the *CFTR* gene: *G551D*, *G1244E*, *G1349D*, *G178R*, *G551S*, *R117H*, *S1251N*, *S1255P*, *S549N* or *S549R*.

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.

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 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, and statements regarding the eligible patient population in Europe, our expectations regarding the timing of access to KALYDECO for eligible patients four months of age and older across countries in Europe, and our plans to secure access to KALYDECO for additional eligible patients four months of age and older 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 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)

View source version on [businesswire.com](https://www.businesswire.com/news/home/20201105005062/en/): <https://www.businesswire.com/news/home/20201105005062/en/>

Vertex Pharmaceuticals Incorporated

Investors:

InvestorInfo@vrtx.com

or

617-961-7163

Media:

mediainfo@vrtx.com

or

International: +44 20 3204 5275

or

U.S.: 617-341-6992

Source: Vertex Pharmaceuticals Incorporated