



European Commission Approves ORKAMBI® (lumacaftor/ivacaftor) for the Treatment of Children With Cystic Fibrosis Ages 1 to <2 Years Old

July 5, 2023

-Nearly 300 children with cystic fibrosis and two copies of the F508del mutation are now eligible for the first time for a medicine that can treat the underlying cause of their disease-

LONDON--(BUSINESS WIRE)--Jul. 5, 2023-- [Vertex Pharmaceuticals](#) today announced that the European Commission has granted approval for the label extension of ORKAMBI® (lumacaftor/ivacaftor) for the treatment of children with cystic fibrosis (CF) ages 1 to <2 years old who have two copies of the *F508del* mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene, the most common form of the disease.

"This approval will offer some of the youngest children with cystic fibrosis the chance of improved outcomes, by treating their disease at a young age," said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer, Vertex. "With this important milestone, we move ever closer to our goal of providing medicines that treat the underlying cause of CF to all people living with the disease."

"CF symptoms and organ damage can manifest very early in life, so it is crucial to start treatment as early as possible," said Silvia Gartner, M.D., specialist in Pediatrics and Pneumology, Coordinator of the Pediatric Cystic Fibrosis Center, Barcelona. "Today's approval provides us with a medicine that gives a window of opportunity to possibly delay the onset of CF for these very young eligible children."

ORKAMBI® has also been approved by regulatory authorities in the U.S., in Great Britain, Australia and Canada, for people with CF and two copies of the *F508del* mutation in the *CFTR* gene, ages 1 and above.

As a result of long-term reimbursement agreements in Austria, Denmark, the Republic of Ireland and Sweden, and provisions for access in health care systems such as Germany, eligible patients in these countries will have access to the expanded indication for ORKAMBI® shortly following regulatory approval by the European Commission. As a result of the long-term reimbursement agreement in the UK, children ages 1 to <2 years old in the UK have had access to this expanded indication for ORKAMBI® since MHRA approval in March 2023. Vertex will continue to work with reimbursement bodies across the European Union, Australia and Canada to ensure access for all eligible patients.

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 88,000 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 early 30s.

About ORKAMBI® (lumacaftor/ivacaftor)

In people with two copies of the *F508del* mutation, the *CFTR* protein is not processed and trafficked normally within the cell, resulting in little to no *CFTR* protein at the cell surface.

ORKAMBI® (lumacaftor/ivacaftor) is an oral medicine that is a combination of lumacaftor and ivacaftor. Lumacaftor is designed to increase the amount of mature protein at the cell surface by targeting the processing and trafficking defect of the *F508del*-*CFTR* protein. Ivacaftor, which is known as a *CFTR* potentiator, is designed to facilitate the ability of *CFTR* proteins to transport salt and water across the cell membrane. The combined actions of lumacaftor and ivacaftor help hydrate and clear mucus from the airways.

ORKAMBI® (lumacaftor/ivacaftor) is a prescription medicine used for the treatment of cystic fibrosis (CF) in patients aged 1 year and older who have two copies of the *F508del* mutation (*F508del/F508del*) in their *CFTR* gene.

The complete product information will be available 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, we have a robust clinical pipeline of investigational small molecule, mRNA, cell and genetic therapies (including gene editing) in other serious diseases where we have deep insight into causal human biology, including sickle cell disease, beta thalassemia, APOL1-mediated kidney disease, acute and neuropathic pain, type 1 diabetes and alpha-1 antitrypsin deficiency.

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 13 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 Twitter, LinkedIn and YouTube.

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. and Silvia Gartner, M.D., in this press release and statements regarding the eligible patient population for ORKAMBI, including patients newly eligible for ORKAMBI, our expectations for patient access to ORKAMBI, including statements about our efforts to ensure patient access to ORKAMBI, and statements regarding the potential benefits of ORKAMBI. 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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