Vertex Announces European Commission Approval of ALYFTREK®, a New Once-Daily CFTR Modulator for the Treatment of Cystic Fibrosis

July 1, 2025

- ALYFTREK® (deutivacaftor/tezacaftor/vanzacaftor) approved in the EU for people with CF 6 years and older with at least one non-class I mutation in the CFTR gene, making it the broadest label for this medicine in the world -

- In head-to-head clinical trials, deutivacaftor/tezacaftor/vanzacaftor was non-inferior on $ppFEV_1$ and superior compared to KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) at reducing sweat chloride, demonstrating greater improvement in CFTR function -

- Approximately 31,000 people with CF in the EU are now eligible for this new highly effective modulator therapy -

LONDON--(BUSINESS WIRE)--Jul. 1, 2025-- Vertex Pharmaceuticals (Nasdaq: VRTX) today announced that the European Commission has granted approval for ALYFTREK® (deutivacaftor/tezacaftor/vanzacaftor) for the treatment of people with cystic fibrosis (CF) ages 6 years and older who have at least one non-class I mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

“Thousands of people with CF across the EU may now benefit from this new, once-daily medicine, which has demonstrated further improvement in CFTR protein function versus KAFTRIO,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “With this approval, we are one step closer to our ultimate goal of restoring normal levels of CFTR function in people living with CF.”

In two head-to-head pivotal clinical trials, deutivacaftor/tezacaftor/vanzacaftor was non-inferior to KAFTRIO® (ivacaftor/tezacaftor/elexacaftor) in combination with ivacaftor on $ppFEV_1$ and superior at reducing sweat chloride, demonstrating greater improvement in CFTR function.

“CF care has been transformed by the advent of highly effective CFTR modulators, and I am very pleased that we now have a new treatment option to even better address this multi-systemic disease,” said Professor Marcus A. Mall, M.D., Professor and Chair of the Department of Pediatric Respiratory Medicine, Immunology and Critical Care Medicine and Cystic Fibrosis Center at Charité Universitätsmedizin Berlin. “Deutivacaftor/tezacaftor /vanzacaftor has shown it can deliver greater reductions in sweat chloride compared to standard of care. By bringing more people closer to normal level of CFTR function, this new medicine has the potential to further improve outcomes for patients.”

As a result of reimbursement agreements in Ireland and Denmark and provisions for access in health care systems such as Germany, eligible patients in these countries will have access to deutivacaftor/tezacaftor/vanzacaftor shortly following regulatory approval by the European Commission. Vertex will continue to work with reimbursement bodies across the European Union member states to ensure access for all eligible patients as quickly as possible.

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 109,000 people, including 94,000 people in North America, Europe and Australia. 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.

Today Vertex CF medicines are treating over 75,000 people with CF in more than 60 countries on six continents. This represents 2/3 of the diagnosed people with CF eligible for CFTR modulator therapy.

Sweat chloride is used to diagnose CF, which measures CFTR function. 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 a *CFTR* gene mutation but do not have any manifestation of disease (carriers). At a population level, higher levels of SwCl are associated with more severe disease. Restoring CFTR function leads to lower levels of SwCl. SwCl levels below 60 mmol/L are associated with improved outcomes such as better and more stable lung function, fewer pulmonary exacerbations, better quality of life and improved survival. Restoring SwCl levels below 30 mmol/L has long been the ultimate treatment goal for Vertex, as levels below 30 mmol/L are considered normal and are typical of CF carriers who do not have disease.

About ALYFTREK® (deutivacaftor/tezacaftor/vanzacaftor)

In people with CF, mutations in the *CFTR* gene lead to decreased quantity and/or function of the CFTR protein channel at the cell surface. Vanzacaftor and tezacaftor are designed to increase the amount of CFTR protein at the cell surface by facilitating the processing and trafficking of the CFTR protein. Deutivacaftor is a potentiator designed to increase the channel open probability of the CFTR protein delivered to the cell surface to improve the flow of salt and water across the cell membrane.

ALYFTREK is approved in the EU for the treatment of people with cystic fibrosis (CF) ages 6 years and older who have at least one non-class I mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene.

For complete product information, please see the Summary of Product Characteristics (SmPC) that can be found at www.ema.europa.eu.

ALYFTREK is currently licensed in the U.S., the UK and the European Union and is under regulatory review in Canada, Switzerland, Australia and New Zealand.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases and conditions. The company has approved therapies for cystic fibrosis, sickle cell disease, transfusion-dependent beta thalassemia and acute pain, and it continues to advance clinical and research programs in these areas. 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 neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, primary membranous nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes and myotonic dystrophy type 1.

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 15 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/en-global.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements made by Reshma Kewalramani, M.D., and Professor Marcus A. Mall, M.D., in this press release and statements regarding the expectations for the potential benefits of ALYFTREK, expectations for the eligible patient population in the EU, expectations for patient access to deutivacaftor/tezacaftor/vanzacaftor shortly following regulatory approval by the European Commission, and Vertex's continued work with reimbursement bodies across the EU member states to ensure access for all eligible patients as quickly as possible. 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, 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.

(VRTX-GEN)

View source version on businesswire.com: https://www.businesswire.com/news/home/20250626924350/en/

Vertex Pharmaceuticals Incorporated
Investors:
InvestorInfo@vrtx.com

Media:
mediainfo@vrtx.com
or
International: +44 20 3204 5275

Source: Vertex Pharmaceuticals Incorporated