# Vertex Announces FDA Acceptance of New Drug Application for Vanzacaftor/Tezacaftor /Deutivacaftor, a Next-In-Class Triple Combination Treatment for Cystic Fibrosis

July 2, 2024

*- Vanza triple granted priority review with Prescription Drug User Fee Act (PDUFA) target action date of January 2, 2025 –*

*- EU Marketing Authorization Application (MAA) submission also validated by European Medicines Agency (EMA)*

BOSTON--(BUSINESS WIRE)--Jul. 2, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that the U.S. Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) for investigational once-daily vanzacaftor/tezacaftor/deutivacaftor triple combination therapy (vanza triple) for people living with cystic fibrosis (CF) ages 6 years and older who have at least one *F508del* mutation or another responsive mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene responsive to the vanza triple. Vertex used a priority review voucher for this submission reducing the review time from 10 months to 6 months, resulting in a Prescription Drug User Fee Act (PDUFA) target action date of January 2, 2025.

"The FDA acceptance of our vanza triple application and the MAA validation by the EMA represent important milestones in the decades-long development of CFTR modulators and another example of our track record of serial innovation in CF," said Nia Tatsis, Ph.D., Executive Vice President, Chief Regulatory and Quality Officer at Vertex. "Vanzacaftor raises the high bar set by TRIKAFTA® and gives more people with CF the chance to get to levels of sweat chloride below the diagnostic threshold for CF, and even to levels of sweat chloride seen in those without CF."

Vertex also received validation of its Marketing Authorization Application (MAA) submission by the European Medicines Agency (EMA) in the EU for patients ages 6 years and older. The company has also submitted in Canada, Australia, Switzerland and the U.K.

**About Cystic Fibrosis**

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 92,000 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 30s, but with treatment, projected survival is improving.

Today Vertex CF medicines are treating over 65,000 people with CF across 60 countries on six continents. This represents 2/3 of the diagnosed people with CF eligible for CFTR modulator therapy.

Diagnosis of CF is often made by genetic testing and is confirmed by testing sweat chloride (SwCl), which measures CFTR protein dysfunction. 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). 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 vanzacaftor/tezacaftor/deutivacaftor (the "vanza triple")**

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.

Investigational vanzacaftor/tezacaftor/deutivacaftor was granted Fast Track and Orphan Drug Designations from the U.S. Food and Drug Administration for the treatment of cystic fibrosis.

The vanza triple will be subject to a meaningfully lower single-digit royalty obligation, compared to the rate payable on Vertex's current CF portfolio.

**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 approved medicines that treat the underlying causes of multiple chronic, life-shortening genetic diseases — cystic fibrosis, sickle cell disease and transfusion-dependent beta thalassemia — and continues to advance clinical and research programs in these diseases. 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 acute and neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes, myotonic dystrophy type 1 and alpha-1 antitrypsin deficiency.

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 14 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 LinkedIn, Facebook, Instagram, YouTube and Twitter/X.

**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, the statements by Nia Tatsis, Ph.D., in this press release, and statements regarding our expectations for the benefits of and potential for the vanza triple, and our expectations that the vanza triple will be subject to a lower single-digit royalty obligation. 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, and other reasons, that future competitive or other market factors may adversely affect the commercial potential for the vanza triple, and other risks listed under the heading "Risk Factors" in Vertex's most recent annual report and subsequent quarterly reports filed with the Securities and Exchange Commission at www.sec.gov 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.

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