Vertex Announces CASGEVY® Reimbursement Agreement for the Treatment of Transfusion-Dependent Beta Thalassemia and Sickle Cell Disease in Italy

September 18, 2025

- CASGEVY is the first, and only, gene editing therapy approved for the treatment of transfusion-dependent beta thalassemia (*TDT) and* sickle cell disease *(SCD) in Europe -*

- Italy has the largest population of people living with TDT in Europe -

LONDON--(BUSINESS WIRE)--Sep. 18, 2025-- Vertex Pharmaceuticals (Nasdaq: VRTX) announced today a reimbursement agreement with the Italian Medicines Agency (AIFA) for eligible transfusion-dependent beta thalassemia (TDT) and severe sickle cell disease (SCD) patients to access the CRISPR/Cas9 gene-edited therapy, CASGEVY® (exagamglogene autotemcel).

"Today is a turning point for eligible people in Italy living with transfusion-dependent beta thalassemia and sickle cell disease, two life-shortening diseases with limited treatment options," said Ludovic Fenaux, Senior Vice President, Vertex International. "Italy has the largest TDT population in Europe, which underscores the importance of this agreement. We appreciate the collaboration with AIFA to recognize the value a one-time transformative treatment provides to patients, families and the health care system."

In Italy, there are approximately 5,000 people 12 years and older living with TDT and around 2,300 with SCD. Italy joins a number of countries that have signed reimbursement agreements for CASGEVY including Austria, Bahrain, Denmark, England, the Kingdom of Saudi Arabia and the United Arab Emirates.

About Transfusion-Dependent Beta Thalassemia (TDT)

TDT is a serious, life-threatening genetic disease. TDT patients report health-related quality of life scores below the general population and significant health care resource utilization. TDT requires frequent blood transfusions and iron chelation therapy throughout a person's life. Due to anemia, patients living with TDT may experience fatigue and shortness of breath, and infants may develop failure to thrive, jaundice and feeding problems. Complications of TDT can also include an enlarged spleen, liver and/or heart, misshapen bones and delayed puberty. TDT requires lifelong treatment and significant use of health care resources, and ultimately results in reduced life expectancy, decreased quality of life and reduced lifetime earnings and productivity. In Europe, the mean age of death for patients living with TDT is 50-55 years.

About Sickle Cell Disease (SCD)

SCD is a debilitating, progressive, life-shortening genetic disease. SCD patients report health-related quality of life scores well below the general population and significant health care resource utilization. SCD affects the red blood cells, which are essential for carrying oxygen to all organs and tissues of the body. SCD causes severe pain, organ damage and shortened life span due to misshapen or "sickled" red blood cells. The clinical hallmark of SCD is vaso-occlusive crises (VOCs), which are caused by blockages of blood vessels by sickled red blood cells and result in severe and debilitating pain that can happen anywhere in the body at any time. SCD requires lifelong treatment and significant use of health care resources, and ultimately results in reduced life expectancy, decreased quality of life and reduced lifetime earnings and productivity. In Europe, the mean age of death for patients living with SCD is around 40 years.

About CASGEVY® (exagamglogene autotemcel)

CASGEVY® is a non-viral, *ex vivo* CRISPR/Cas9 gene-edited cell therapy for eligible patients with SCD or TDT, in which a patient's own hematopoietic stem and progenitor cells are edited at the erythroid specific enhancer region of the *BCL11A* gene through a precise double-strand break. This edit results in the production of high levels of fetal hemoglobin (HbF; hemoglobin F) in red blood cells. HbF is the form of the oxygen-carrying hemoglobin that is naturally present during fetal development, which then switches to the adult form of hemoglobin after birth. CASGEVY has been shown to reduce or eliminate VOCs for patients with SCD and transfusion requirements for patients with TDT.

CASGEVY is approved for eligible SCD and TDT patients 12 years and older by multiple regulatory bodies around the world. In the European Union, CASGEVY is approved for patients 12 years of age and older with either severe SCD with recurrent VOCs or TDT, for whom hematopoietic stem cell (HSC) transplantation is appropriate and a human leukocyte antigen matched related HSC donor is not available.

For complete product information, please see the Summary of Product Characteristics (SmPC) at 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 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.

Vertex 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 Ludovic Fenaux, in this press release, and statements regarding Vertex's expectations for and the anticipated benefits of CASGEVY, expectations for access to CASGEVY for eligible TDT and SCD patients in Italy, and expectations for the population of people living with TDT and SCD in Europe. While we believe 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, anticipated patient populations may be different than expected, 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.

(VRTX-GEN)

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