Vertex Announces CASGEVY® Reimbursement Agreement for the Treatment of Sickle Cell Disease in England

January 31, 2025

- Agreement means eligible sickle cell disease (SCD) patients in England now have access to CASGEVY -

- Agreement for CASGEVY in transfusion-dependent beta thalassemia (TDT) was previously reached in August 2024 -

LONDON--(BUSINESS WIRE)--Jan. 31, 2025-- Vertex Pharmaceuticals (Nasdaq: VRTX) announced today a reimbursement agreement with NHS England for eligible sickle cell disease (SCD) patients to access the CRISPR/Cas9 gene-edited therapy, CASGEVY® (exagamglogene autotemcel).

The reimbursement agreement comes as the National Institute for Health and Care Excellence (NICE) issues positive guidance recommending CASGEVY's use in the NHS. It means that eligible SCD patients in England now have access to the therapy following the prior agreement for transfusion-dependent beta thalassemia (TDT) patients announced last August.

"Today is an important day for the sickle cell community who have gone too long without treatments that address the underlying cause of their devastating disease," said Ludovic Fenaux, Senior Vice President, Vertex International. "We are pleased to have reached this new agreement that ensures both eligible SCD and TDT patients can now be treated with CASGEVY, recognizing the value a one-time treatment can provide to patients, their families and the healthcare system."

The administration of the therapy requires experience in stem cell transplantation and the management of hemoglobinopathies; therefore, Vertex is continuing to engage with experienced hospitals throughout England to establish a network of independently operated authorized treatment centers (ATCs).

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. Stem cell transplant from a matched donor is a potentially curative option but is only available to a small fraction of people living with SCD because of the lack of available donors.

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. The Conditional Marketing Authorization in Great Britain for CASGEVY is for the treatment of patients 12 years of age and older with either TDT or SCD (with recurrent VOCs who have the *βS*/*βS, βS*/*β+* or *βS*/*β0* genotype), for whom hematopoietic stem cell transplantation is appropriate and a human leukocyte antigen matched related hematopoietic stem cell donor is not available.

For full details about access eligibility please refer to the NICE final draft guidance issued today.

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 serious diseases and conditions — cystic fibrosis, sickle cell disease, transfusion-dependent beta thalassemia and acute pain — and continues tc

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.

(VRTX-GEN)

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 SCD patients in England, and Vertex's plans to continue to engage with experienced hospitals throughout England to establish an ATC network. 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, 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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