Vertex Announces CASGEVY™ Reimbursement Agreement for the Treatment of Transfusion-Dependent Beta Thalassemia in England

August 7, 2024

- Eligible transfusion-dependent beta thalassemia (TDT) patients in England will be able to access the therapy from today -

- CASGEVY is one of the first medicines funded by NHS England's Innovative Medicines Fund -

LONDON--(BUSINESS WIRE)--Aug. 7, 2024-- Vertex Pharmaceuticals (Nasdaq: VRTX) announced today a reimbursement agreement with NHS England for eligible transfusion-dependent beta thalassemia (TDT) patients to access the CRISPR/Cas9 gene-edited therapy, CASGEVY™ (exagamglogene autotemcel), from today.

The reimbursement agreement comes as the National Institute for Health and Care Excellence (NICE) issues positive guidance recommending CASGEVY's use in the NHS.

The United Kingdom (U.K.) Medicines and Healthcare products Regulatory Agency (MHRA) granted CASGEVY the first authorization in the world for a CRISPR-based gene-editing therapy on November 15, 2023.

"Securing access to CASGEVY is a historic moment for people living with transfusion-dependent beta thalassemia who, for too long, have had limited options for this life-shortening disease," said Ludovic Fenaux, Senior Vice President, Vertex International. "Through collaboration with NHS England and NICE, we have reached this landmark agreement that recognizes the value a one-time treatment can provide to patients, their families and the health care system."

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

Vertex continues to work collaboratively with NICE and NHS England to ensure eligible sickle cell disease (SCD) patients in England can also access this treatment as soon as possible. In the European Union, Vertex is working closely with reimbursement authorities to bring this innovative therapy to eligible SCD and TDT patients rapidly as the company has done in the United States, the Kingdom of Saudi Arabia and Bahrain.

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

About CASGEVY™ (exagamglogene autotemcel [exa-cel])

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 vaso-occlusive crises (VOCs) for patients with SCD and transfusion requirements for patients with TDT.

CASGEVY is approved for certain indications in multiple jurisdictions for eligible patients.

In Great Britain, CASGEVY was granted Conditional Marketing Authorization for the treatment of patients 12 years of age and older with either TDT or SCD with recurrent vaso-occlusive crises 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.

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, YouTube and Twitter/X.

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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 our expectations for and the anticipated benefits of CASGEVY, our expectations for the anticipated timeline for eligible TDT patients in England to have access to CASGEVY, our plans to engage with experienced hospitals throughout England to establish an ATC network, our plans to continue working with NICE and NHS England to ensure eligible SCD patients in England can access CASGEVY as soon as possible, and our plans to work with reimbursement authorities in the European Union to bring CASGEVY to eligible SCD and TDT patients. 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 eligible patient access to CASGEVY may not be achieved on the anticipated timeline, or at all, 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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