European Commission Approves First CRISPR/Cas9 Gene-Edited Therapy, CASGEVY™ (exagamglogene autotemcel), for the Treatment of Sickle Cell Disease and Transfusion-Dependent Beta Thalassemia

February 13, 2024

- Over 8,000 patients 12 years of age and older with severe sickle cell disease or transfusion-dependent beta thalassemia may be eligible for treatment -

LONDON--(BUSINESS WIRE)--Feb. 13, 2024-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) announced today that the European Commission has granted conditional marketing authorization to CASGEVY ™ (exagamglogene autotemcel [exa-cel]), a CRISPR/Cas9 gene-edited therapy. CASGEVY is approved for the treatment of patients who are 12 years of age and older with severe sickle cell disease (SCD) characterized by recurrent vaso-occlusive crises (VOCs) or transfusion-dependent beta thalassemia (TDT), for whom hematopoietic stem cell (HSC) transplantation is appropriate and a human leukocyte antigen matched related HSC donor is not available.

CASGEVY is the only genetic therapy approved for SCD and TDT patients in the European Union (EU) and with this approval, there are now more than 8,000 patients potentially eligible for treatment.

“With this approval, CASGEVY is now approved for sickle cell disease and transfusion-dependent beta thalassemia in multiple geographies making tens of thousands of patients eligible for this potentially transformative therapy,” said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. “Now our goal shifts to translating these approvals into real-world patient benefit and ensuring access and reimbursement across the globe.”

“Sickle cell disease and transfusion-dependent beta thalassemia are debilitating, life-shortening diseases associated with significant burden on patients, families and health care systems,” said Franco Locatelli, M.D., Ph.D., Principal investigator in the CLIMB-111 and CLIMB-121 studies, Professor of Pediatrics at the Catholic University of the Sacred Heart, Rome, and Director of the Department of Pediatric Hematology and Oncology at the Bambino Gesù Children’s Hospital. “CASGEVY offers the potential of a functional cure, and it will be important to offer this therapeutic option to eligible patients as soon as possible.”

Vertex is already working closely with national health authorities to secure access for eligible patients as quickly as possible. Through this work, Vertex has secured early access for eligible TDT patients in France ahead of the national reimbursement process. Vertex continues to engage with hospitals experienced in stem cell transplantation to establish a network of independently operated authorized treatment centers (ATCs) for the administration of CASGEVY. There are currently three activated ATCs in the EU and Vertex plans to activate a total of approximately 25 centers across Europe.

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 curative option but is only available to a small fraction of people living with SCD because of the lack of available donors.

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 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 VOCs for patients with SCD and transfusion requirements for patients with TDT.

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

About Conditional Marketing Authorizations (CMAs)

CMAs are for medicines that fulfil a significant unmet medical need such as being for serious and life-threatening diseases, where no satisfactory treatment methods are available or where the medicine offers a major therapeutic advantage. A CMA is granted where comprehensive clinical data is not yet complete, but the benefit of the medicine to address a significant unmet need outweighs the need for data that will become available in the future. CMAs are valid for one year and renewable annually with ongoing regulatory review of data.

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 APOL1-mediated kidney disease, acute and neuropathic pain, 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 Reshma Kewalramani, M.D., and Franco Locatelli, M.D., Ph.D., in this press release, and statements regarding our expectations for and the anticipated benefits of CASGEVY, the estimated eligible patient population, our efforts to secure access for eligible patients as quickly as possible, our continued engagement with hospitals to establish a network of ATCs and our plans to activate a total of approximately 25 centers across 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, that commercializing CASGEVY in multiple geographies may not occur on the anticipated timeline, or at all, that adequate pricing and reimbursement for CASGEVY may not be achieved on the anticipated timeline, or at all, that the conditional marketing authorization may not be renewed annually, or at all, 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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Source: Vertex Pharmaceuticals Incorporated