Vertex Announces US FDA Approval for Expanded Use of CASGEVY® for the Treatment of People Ages 2 Years and Older With Sickle Cell Disease or Transfusion-Dependent Beta Thalassemia

July 1, 2026

- First and only approved genetic therapy to treat children as young as 2 years for both severe sickle cell disease and transfusion-dependent beta thalassemia -

- Approximately 5,500 additional children in the U.S. are now eligible for this established one-time therapy, expanding upon the prior FDA approval in people 12 years and older -

- Regulatory review for label expansion underway in the Kingdom of Saudi Arabia and United Kingdom -

BOSTON--(BUSINESS WIRE)--Jul. 1, 2026-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) announced today that the U.S. Food and Drug Administration (FDA) has approved expanded use of CASGEVY® (exagamglogene autotemcel) for the treatment of people ages 2 years and older with either sickle cell disease (SCD) with recurrent vaso-occlusive crises (VOCs) or transfusion-dependent beta thalassemia (TDT). CASGEVY is the first approved genetic therapy indicated for children as young as 2 years for both SCD and TDT.

“Just as we redefined what is possible in cystic fibrosis, our ambition is to transform the future for people living with sickle cell disease and transfusion-dependent beta thalassemia. The remarkable consistency of results across age groups reinforces the potential of CASGEVY to deliver durable, transformative benefits to those who have historically had limited options,” said Reshma Kewalramani, M.D., Chief Executive Officer and President, Vertex. “We’re deeply grateful to the patients, families and investigators who participated in the clinical trials that led to this historic approval, and we are ready to bring CASGEVY to children and their families across the U.S.”

“Today’s approval offers renewed hope for children living with sickle cell disease or transfusion -dependent beta thalassemia,” said Haydar Frangoul, M.D., M.S., Medical Director of HCA Healthcare’s Sarah Cannon Transplant and Cellular Therapy Program at TriStar Centennial Children’s Hospital, investigator with Sarah Cannon Research Institute (SCRI) and Member of Vertex’s SCD Program Steering Committee. “Earlier access to the transformative potential of this therapy will allow clinicians and families to consider treatment before years of cumulative damage from these life-shortening diseases take hold.”

Vertex has established a network of independently operated, authorized treatment centers (ATCs) throughout the U.S. to offer CASGEVY to eligible patients through existing access and reimbursement pathways. Today, there are more than 75 activated ATCs in the U.S. The full list can be accessed at CASGEVY.com.

About Sickle Cell Disease (SCD)

Sickle cell disease (SCD) is a rare serious, inherited blood disease that is progressive and life-shortening. The disease causes red blood cells to become rigid and misshapen, restricting blood flow and oxygen delivery to vital organs. Recurrent vaso-occlusive crises (VOCs), unpredictable episodes of severe pain caused by blocked blood vessels, are a defining feature of SCD and frequently require hospitalization. Many patients experience these complications early in life, and over time, repeated VOCs and chronic anemia lead to progressive and irreversible organ damage, including damage to the brain, lungs, kidneys and heart. SCD places a substantial burden on patients and their families, who must manage frequent medical visits, hospitalizations, school and work disruptions, and the emotional toll of chronic pain and life-threatening complications. Despite lifelong treatment, people with SCD and recurrent VOCs in the U.S. face shortened life expectancy, with a median age of death of approximately 45 years, report quality-of-life scores far below the general population and the estimated lifetime healthcare costs of managing the disease are $4–6 million.

About Transfusion-Dependent Beta Thalassemia (TDT)

Transfusion-dependent beta thalassemia (TDT) is a rare serious, inherited blood disease that is progressive and life-shortening. The disease impairs the body’s ability to produce sufficient hemoglobin, limiting oxygen delivery to tissues and organs. People with TDT do not have enough functional hemoglobin in their red blood cells and require regular, lifelong blood transfusions, often beginning early in childhood, along with ongoing iron chelation therapy. While transfusions are necessary for survival, many of the long-term complications of TDT are exacerbated by chronic transfusion therapy and iron overload and cumulative damage to the heart, liver and endocrine system, as well as bone abnormalities and delayed growth and puberty. TDT places a significant and

ongoing burden on patients and their families, requiring frequent medical visits and complex lifelong treatment. Despite lifelong treatment, people with TDT in the U.S. face shortened life expectancy, with a median age of death of approximately 37 years, reduced quality of life and productivity, and the estimated lifetime healthcare costs of managing the disease are $5–5.7 million.

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 in clinical trials to reduce or eliminate VOCs for patients with SCD and transfusion requirements for patients with TDT.

In the United States, CASGEVY was approved using the Commissioner's National Priority Voucher. Vertex has also recently completed regulatory submissions in the Kingdom of Saudi Arabia and United Kingdom to expand the use of CASGEVY to children as young as five.

About the CLIMB Studies

The completed Phase 1/2/3 open-label studies, CLIMB-111 and CLIMB-121, were designed to assess the safety and efficacy of a single dose of CASGEVY in patients ages 12-35 years with TDT or with SCD and recurrent VOCs. Patients were followed for approximately two years after CASGEVY infusion in these studies. CLIMB-141 and CLIMB-151 are ongoing Phase 3 open-label studies, designed to assess the safety and efficacy of a single dose of exagamglogene autotemcel in patients ages 2-11 years with TDT or with SCD and recurrent VOCs. Enrollment and dosing are complete for the 5–11-year-old cohort in both studies.

Each patient in these studies is asked to participate in the ongoing long-term, open-label study, CLIMB-131. CLIMB-131 is designed to evaluate the long-term safety and efficacy of CASGEVY in patients with up to 15 years of follow up after CASGEVY infusion.

U.S. INDICATIONS AND IMPORTANT SAFETY INFORMATION FOR CASGEVY

INDICATION

CASGEVY is indicated for the treatment of patients aged 2 years and older with:

- sickle cell disease (SCD) with recurrent vaso-occlusive crises (VOCs)
- transfusion-dependent β-thalassemia (TDT)

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Neutrophil Engraftment Failure

There is potential risk of neutrophil engraftment failure after treatment with CASGEVY. In the clinical trials, all treated patients achieved neutrophil engraftment and no patients received rescue $CD34^+$ cells.

Monitor absolute neutrophil counts (ANC) and manage infections according to standard guidelines and medical judgement. In the event of neutrophil engraftment failure, patients should be infused with rescue $CD34^+$ cells.

Granulocyte colony-stimulating factor (G-CSF) is not recommended for 21 days after CASGEVY infusion.

Delayed Platelet Engraftment

Delayed platelet engraftment has been observed with CASGEVY treatment. There is an increased risk of bleeding until platelet engraftment is achieved.

Monitor patients for bleeding according to standard guidelines and medical judgement. Conduct frequent platelet counts until platelet engraftment and platelet recovery are achieved. Perform blood cell count determination and other appropriate testing whenever clinical symptoms suggestive of bleeding arise.

Hypersensitivity Reactions

Hypersensitivity reactions, including anaphylaxis can occur due to dimethyl sulfoxide (DMSO) or dextran 40 in the cryopreservation solution. Monitor patients for hypersensitivity reactions during and after infusion.

Off-Target Genome Editing Risk

The risk of unintended, off-target editing in an individual's $CD34^+$ cells cannot be ruled out due to genetic variants. The clinical

significance of potential off-target editing is unknown.

ADVERSE REACTIONS

The most common Grade 3 or 4 non-laboratory adverse reactions (occurring in ≥ 25%) were mucositis and febrile neutropenia in patients with SCD and patients with TDT, and decreased appetite in patients with SCD.

All (100%) of the patients with TDT and SCD experienced Grade 3 or 4 neutropenia and thrombocytopenia. Other common Grade 3 or 4 laboratory abnormalities (≥ 50%) include leukopenia, anemia, and lymphopenia.

DRUG INTERACTIONS

No formal drug interaction studies have been performed. CASGEVY is not expected to interact with the hepatic cytochrome P450 family of enzymes or drug transporters.

Use of Granulocyte-Colony Stimulating Factor (G-CSF): G-CSF must not be used for $CD34^+$ HSC mobilization of patients with SCD.

Use of Hydroxyurea: Discontinue the use of hydroxyurea at least 8 weeks prior to start of each mobilization cycle and conditioning. There is no experience of the use of hydroxyurea after CASGEVY infusion.

Use of Crizanlizumab: Discontinue the use of crizanlizumab at least 8 weeks prior to start of mobilization and conditioning, as their interaction potential with mobilization and myeloablative conditioning agents is not known.

Use of Iron Chelators: Discontinue the use of iron chelators at least 7 days prior to initiation of myeloablative conditioning, due to potential interaction with the conditioning agent. Some iron chelators are myelosuppressive. If iron chelation is required, avoid the use of non-myelosuppressive iron chelators for at least 3 months and use of myelosuppressive iron chelators for at least 6 months after CASGEVY infusion. Phlebotomy can be used instead of iron chelation, when appropriate.

USE IN SPECIFIC POPULATIONS

Pregnancy/Lactation: CASGEVY must not be administered during pregnancy and breastfeeding should be discontinued during conditioning because of the risks associated with myeloablative conditioning. Pregnancy and breastfeeding after CASGEVY infusion should be discussed with the treating physician.

Females and Males of Reproductive Potential: A negative serum pregnancy test must be confirmed prior to the start of each mobilization cycle and reconfirmed prior to myeloablative conditioning.

Women of childbearing potential and men capable of fathering a child should use effective methods of contraception from start of mobilization through at least 6 months after administration of CASGEVY. Advise patients of the risks associated with conditioning agents.

Infertility has been observed with myeloablative conditioning, therefore, advise patients of fertility preservation options before treatment, if appropriate.

Please see full Prescribing Information for CASGEVY.

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 IgA nephropathy, neuropathic pain, APOL1-mediated kidney disease, primary membranous nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes, generalized myasthenia gravis, 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 16 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 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, including, without limitation, statements made by Reshma Kewalramani, M.D., and Haydar Frangoul, M.D., M.S., in this press release, statements regarding the expected clinical benefits of CASGEVY, expectations regarding the eligible patient population, and expectations for patient access to CASGEVY. 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 factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include the risks listed under the heading “Risk Factors” in Vertex's annual report and in subsequent filings 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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Vertex Pharmaceuticals Incorporated

Investors:
InvestorInfo@vrtx.com or
+1 617-341-6108

Media:
mediainfo@vrtx.com or
617-341-6992

Source: Vertex Pharmaceuticals Incorporated