Vertex Presents New Data on CASGEVY®, Including First European Presentation of Data in Children Ages 5–11, at the European Hematology Association Congress and Announces Additional Global Regulatory Submissions

June 11, 2026

- Data from pivotal studies of CASGEVY in children ages 5–11 with severe sickle cell disease or transfusion-dependent beta thalassemia demonstrate transformative potential in younger patients, consistent with the durable benefits established in patients 12 years and older -

- Data simultaneously published in the New England Journal of Medicine -

- Regulatory review underway in the United States to expand the use of CASGEVY, and Vertex has recently completed submissions in the Kingdom of Saudi Arabia and United Kingdom -

BOSTON--(BUSINESS WIRE)--Jun. 11, 2026-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced data demonstrating the clinical benefits of CASGEVY® (exagamglogene autotemcel) in people ages 5 years and older living with severe sickle cell disease (SCD) or transfusion-dependent beta thalassemia (TDT). The results, from pivotal studies in children ages 5–11, show that the efficacy and safety outcomes in this age group are consistent with the transformative profile established in adult and adolescent patients. These data were presented at the European Hematology Association (EHA) Congress and simultaneously published in the *New England Journal of Medicine* (NEJM).

“The data presented at EHA and published in NEJM underscore the consistent, durable and transformative benefits CASGEVY can provide to people living with sickle cell disease or transfusion-dependent beta thalassemia from early in life,” said Carmen Bozic, M.D., Executive Vice President, Global Medicines Development and Medical Affairs, and Chief Medical Officer at Vertex.

“Despite optimized supportive therapy, children living with sickle cell disease and transfusion-dependent beta thalassemia carry a significant disease burden from a very young age, with progressive complications leading to the irreversible and life-shortening consequences of these diseases,” said Franco Locatelli, M.D., Ph.D., Professor of Pediatrics at the Catholic University of the Sacred Heart of Rome, Director of the Department of Pediatric Hematology and Oncology at Bambino Gesù Children’s Hospital, Chair of Vertex’s TDT Program Steering Committee, and presenting author of the 5–11 years old CASGEVY data at EHA. “These data represent a profoundly important step forward, and I look forward to the possibility of providing earlier intervention to prevent complications in children and for families who have had limited potentially curative options to date.”

CASGEVY clinical data for children ages 5–11 presented at EHA and published in NEJM

Data from an interim analysis of the CLIMB-151 and CLIMB-141 studies highlight the transformative potential CASGEVY can provide to children ages 5–11 and are consistent with the durable clinical profile established in adult and adolescent patients. Collectively, these findings highlight the potential benefits of addressing vaso-occlusive crises (VOCs) and transfusion burden earlier in life, which can begin in childhood and are associated with cumulative, long-term complications in SCD and TDT including organ damage.

- In the Phase 3 CLIMB-151 clinical study of children with severe SCD, all 11 patients dosed are free from VOCs and all 8 out of 8 (100%) patients with sufficient follow-up achieved the primary endpoint of being free from VOCs for at least 12 consecutive months (VF12). Of children achieving VF12, the mean (min, max) duration VOC-free was 19.0 (13.2, 30.1) months.
- In the Phase 3 CLIMB-141 clinical study of children with TDT, 15 patients have been dosed with CASGEVY, and all 8 out of 8 (100%) patients with sufficient follow-up achieved the primary endpoint of transfusion independence for at least 12 consecutive months while maintaining a weighted average hemoglobin of at least 9 g/dL (TI12). All children who achieved TI12 remained so throughout the follow-up; the mean (min, max) duration transfusion independence was 23.4 (13.3, 28.5) months.
- The safety profile of CASGEVY in younger patients is consistent with myeloablative conditioning and autologous transplant, as established in clinical studies in older patients with SCD and TDT.
    - As previously disclosed, there was one death, not related to CASGEVY, in a child with TDT who developed severe veno-occlusive disease from busulfan conditioning.
- Consistent with studies in older patients, children with severe SCD and TDT treated with CASGEVY have durable and

clinically relevant increases in fetal hemoglobin (HbF) and stable allelic editing.

Global regulatory submissions to expand use of CASGEVY

CASGEVY is currently approved for eligible people 12 years and older with SCD with recurrent VOCs or TDT in several countries around the world. In the United States the regulatory review is underway with the FDA to expand the use of CASGEVY to younger children after Vertex was awarded 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 younger children. Upon availability, there is an established network of activated authorized treatment centers in these countries prepared to support patients.

The use of CASGEVY in children ages 5–11 years is investigational.

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 Europe face shortened life expectancy, with a mean age of death of around 40 years, and report quality-of-life outcomes far below the general population.

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 face shortened life expectancy, with a mean age of death of approximately 50–55 years in Europe, reduced quality of life and productivity, and significant use of health care resources.

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.

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

WHAT IS CASGEVY?

CASGEVY is a one-time therapy used to treat people ages 12 years and older with:

- sickle cell disease (SCD) who have frequent vaso-occlusive crises or VOCs
- beta thalassemia (β-thalassemia) who need regular blood transfusions

CASGEVY is made specifically for each patient, using the patient's own edited blood stem cells, and increases the production of a

special type of hemoglobin called hemoglobin F (fetal hemoglobin or HbF). Having more HbF increases overall hemoglobin levels and has been shown to improve the production and function of red blood cells. This can eliminate VOCs in people with sickle cell disease and eliminate the need for regular blood transfusions in people with beta thalassemia.

IMPORTANT SAFETY INFORMATION

What is the most important information I should know about CASGEVY?

After treatment with CASGEVY, you will have fewer blood cells for a while until CASGEVY takes hold (engrafts) into your bone marrow. This includes low levels of platelets (cells that usually help the blood to clot) and white blood cells (cells that usually fight infections). Your doctor will monitor this and give you treatment as required. The doctor will tell you when blood cell levels return to safe levels.

- **Tell your healthcare provider right away** if you experience any of the following, which could be signs of low levels of platelet cells:
    - severe headache
    - abnormal bruising
    - prolonged bleeding
    - bleeding without injury such as nosebleeds; bleeding from gums; blood in your urine, stool, or vomit; or coughing up blood
- **Tell your healthcare provider right away** if you experience any of the following, which could be signs of low levels of white blood cells:
    - fever
    - chills
    - infections

You may experience side effects associated with other medicines administered as part of the treatment regimen for CASGEVY. Talk to your physician regarding those possible side effects. Your healthcare provider may give you other medicines to treat your side effects.

How will I receive CASGEVY?

Your healthcare provider will give you other medicines, including a conditioning medicine, as part of your treatment with CASGEVY. It's important to talk to your healthcare provider about the risks and benefits of all medicines involved in your treatment.

After receiving the conditioning medicine, it may not be possible for you to become pregnant or father a child. You should discuss options for fertility preservation with your healthcare provider before treatment.

STEP 1: Before CASGEVY treatment, a doctor will give you mobilization medicine(s). This medicine moves blood stem cells from your bone marrow into the blood stream. The blood stem cells are then collected in a machine that separates the different blood cells (this is called apheresis). This entire process may happen more than once. Each time, it can take up to one week.

During this step rescue cells are also collected and stored at the hospital. These are your existing blood stem cells and are kept untreated just in case there is a problem in the treatment process. If CASGEVY cannot be given after the conditioning medicine, or if the modified blood stem cells do not take hold (engraft) in the body, these rescue cells will be given back to you. If you are given rescue cells, you will not have any treatment benefit from CASGEVY.

STEP 2: After they are collected, your blood stem cells will be sent to the manufacturing site where they are used to make CASGEVY. It may take up to 6 months from the time your cells are collected to manufacture and test CASGEVY before it is sent back to your healthcare provider.

STEP 3: Shortly before your stem cell transplant, your healthcare provider will give you a conditioning medicine for a few days in hospital. This will prepare you for treatment by clearing cells from the bone marrow, so they can be replaced with the modified cells in CASGEVY. After you are given this medicine, your blood cell levels will fall to very low levels. You will stay in the hospital for this step and remain in the hospital until after the infusion with CASGEVY.

STEP 4: One or more vials of CASGEVY will be given into a vein (intravenous infusion) over a short period of time.

After the CASGEVY infusion, you will stay in hospital so that your healthcare provider can closely monitor your recovery. This can take 4-6 weeks, but times can vary. Your healthcare provider will decide when you can go home.

What should I avoid after receiving CASGEVY?

- Do not donate blood, organs, tissues, or cells at any time in the future

What are the possible or reasonably likely side effects of CASGEVY?

The most common side effects of CASGEVY include:

- Low levels of platelet cells, which may reduce the ability of blood to clot and may cause bleeding
- Low levels of white blood cells, which may make you more susceptible to infection

Your healthcare provider will test your blood to check for low levels of blood cells (including platelets and white blood cells). Tell your healthcare provider right away if you get any of the following symptoms:

- fever
- chills
- infections
- severe headache
- abnormal bruising
- prolonged bleeding
- bleeding without injury such as nosebleeds; bleeding from gums; blood in your urine, stool, or vomit; or coughing up blood

These are not all the possible side effects of CASGEVY. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

General information about the safe and effective use of CASGEVY

Talk to your healthcare provider about any health concerns.

Please see full Prescribing Information including Patient 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.

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, statements made by Carmen Bozic, M.D., and Franco Locatelli, M.D., Ph.D., and statements regarding expectations for the transformative potential of CASGEVY in this age group, and expectations for the global regulatory submissions for younger children. 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 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 research and development programs may not support registration or further development of its potential medicines in a timely manner, or at all, due to safety, efficacy or other reasons, that CASGEVY may not receive regulatory approval for this age range on the expected timeline, 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, or the scientific data presented. 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