



Vertex Announces US FDA Approval of CASGEVY™ (exagamglogene autotemcel) for the Treatment of Transfusion-Dependent Beta Thalassemia

January 16, 2024

- Approximately 1,000 patients in the U.S. 12 years of age and older are now eligible for this one-time treatment -

BOSTON--(BUSINESS WIRE)--Jan. 16, 2024-- [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) announced today that the U.S. Food and Drug Administration (FDA) has approved CASGEVY™ (exagamglogene autotemcel [exa-cel]), a CRISPR/Cas9 gene-edited cell therapy, for the treatment of transfusion-dependent beta thalassemia (TDT) in patients 12 years and older.

"On the heels of the historic FDA approval of CASGEVY for sickle cell disease, it is exciting to now secure approval for TDT well ahead of the PDUFA date," said Reshma Kewalramani, M.D., Chief Executive Officer and President of Vertex. "TDT patients deserve new, potentially curative treatment options, and we look forward to bringing CASGEVY to eligible patients who are waiting."

The administration of CASGEVY requires experience in stem cell transplantation; therefore, Vertex is engaging with experienced hospitals to establish a network of independently operated, authorized treatment centers (ATCs) throughout the U.S. to offer CASGEVY to patients. All nine ATCs activated in the U.S. are able to offer CASGEVY to eligible patients with TDT and sickle cell disease (SCD). Additional ATCs will be activated in the coming weeks and a complete list of ATCs can be accessed at [CASGEVY.com](#).

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 the lifetime health care costs in the U.S. of managing TDT are estimated between \$5 and \$5.7 million. 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 the U.S., the median age of death for patients living with TDT is 37 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.

Helping Patients on the CASGEVY Journey

Vertex Connects™ is a program for eligible patients in the U.S. who have been prescribed CASGEVY. Through Vertex Connects, Care Managers are available to provide educational resources, communications and support to navigate the treatment journey, and additional assistance is available for eligible patients.

For more information on ATCs, the CASGEVY treatment journey, and Vertex Connects, call 1-833-VERTEX-5 (1-833-837-8395) — and learn more about our patient support program at [VertexConnects.com](#).

U.S. INDICATIONS AND IMPORTANT SAFETY INFORMATION FOR CASGEVY (exagamglogene autotemcel)

What is CASGEVY?

CASGEVY is a one-time therapy used to treat people aged 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](#) and [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. 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](#), [Facebook](#), [Instagram](#), [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, statements made by Reshma Kewalramani, M.D., in this press release and statements regarding the expectations for the potential benefits of CASGEVY, expectations for the eligible patient population, plans to engage with hospitals to establish a network of ATCs throughout the U.S. and expectations that additional ATCs will be activated in the coming weeks. 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 development programs may not support registration or further development of its compounds due to safety, efficacy or other reasons, that obtaining additional approvals and/or 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, and other risks listed under the heading "Risk Factors" in Vertex's annual report and in subsequent filings filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com and www.sec.gov. 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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