Harvard Pilgrim Health Care | TUFTS Health Plan

Medical Necessity Guidelines
Medical Benefit Drugs

Gene Therapies for Sickle Cell Disease for RITogether

Effective: January 1, 2026

Prior Authorization Required If REQUIRED, submit supporting clinical documentation pertinent to service request.	Yes ☒ No ☐

Applies to:

Commercial Products

☐ Harvard Pilgrim Health Care Commercial products; Fax 617-673-0988
☐ Tufts Health Plan Commercial products; Fax 617-673-0988

Public Plans Products

☐ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax 617-673-0988
☐ Tufts Health Together – MassHealth Accountable Care Partnership Plans; Fax 617-673-0939
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan; Fax 617-673-0939
☐ Tufts Health One Care-- A dual-eligible product; Fax 617-673-0956

Senior Products

☐ Tufts Health Plan Senior Care Options (SCO), (a dual-eligible product); Fax 617-673-0956
☐ Tufts Medicare Preferred HMO, (a Medicare Advantage product); Fax 617-673-0956
☐ Tufts Medicare Preferred PPO, (a Medicare Advantage product); Fax 617-673-0956

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Sickle cell disease (SCD) Sickle cell disease (SCD) is a group of inherited debilitating blood disorders caused by a mutation in the β-globin gene that leads to the production of abnormal sickle shaped hemoglobin (HbS). In SCD, the sickled RBCs become rigid, undergo premature hemolysis leading to anemia and become unable to transport oxygen to critical organs. The condition affects more than 100,000 people in the United States with an incidence of about 1 in every 365 Black births and 1 in every 16,300 Hispanic-American births. Severely affected patients may experience diverse complications such as severe anemia, repeated acute painful vaso-occlusive events (VOEs) due to small-vessel obstruction (vaso-occlusive crises [VOCs]; sickle cell crises), acute chest syndrome (ACS; acute event with pneumonia-like symptoms), cerebral vasculopathy, chronic organ damage that may involve e.g., the bones, kidneys, heart, liver, and lungs or result in severe infectious complications such as functional hyposplenism and premature death. Treatment includes measures to control complications, relieve pain, prevent infections, and minimize organ damage. Standard pharmacologic treatment has included medications, such as hydroxyurea (Hydrea), analgesics and blood transfusions. Hematopoietic stem cell transplantation for patients with an appropriate donor, until the development of gene therapy, has been an option for cure. Gene therapies now offer a treatment option for members with severe sickle cell disease who do not have a willing HLA matched family donor.

Effective January 1, 2026, RITogether will be joining 32 other states in a cell and gene therapy access model. The access model aims to improve the lives of people living with rare and severe diseases by increasing access to potentially transformative treatments. The initial focus of the model is access to gene therapy treatment for people living with sickle cell disease. The cell and gene therapy access model supports outcome based agreements between states and manufactures that will provide treatment within a framework that lowers prices for states and ties payment to outcomes[1].

Food and Drug Administration (FDA) Approved Indications:

CASGEVY is an autologous genome edited hematopoietic stem cell-based gene therapy indicated for the treatment of patients aged 12 years and older with:

- sickle cell disease (SCD) with recurrent vaso-occlusive crises (VOCs);

- transfusion-dependent beta thalassemia (TDT) **(see Casgevy Medical Drug Medical Necessity Guideline for coverage)**

Casgevy is the first FDA-approved gene therapy that uses CRISPER technology for genetic modification, which has been shown to reduce or eliminate VOCs for patients with SCD. CRISPR/Cas9-editing technology allows for modification of defective genes by editing, removing, or replacing DNA from cells. 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. The edited blood stem cells are transplanted back into the patient via a hematopoietic stem cell transplant, engraft (attach and multiply) within the bone marrow, and increase the production of fetal hemoglobin (HbF) in red blood cells. Hemoglobin F reduces intracellular hemoglobin S (Hbs) concentration, preventing the red blood cells from sickling and addressing the underlying cause of the disease. HbF is naturally present during fetal development and is a type of hemoglobin that facilitates oxygen delivery

NOTE: Casgevy can only be administered at a Casgevy Authorized Treatment Center (ATC). Each Casgevy ATC has been carefully selected based on their expertise in areas such as transplant, cell and gene therapy. For information on locating an Authorized Treatment Center, please go to https://www.casgevy.com/sickle-cell-disease/find-an-ATC

Food and Drug Administration (FDA) Approved Indications:

- Lyfgenia is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of patients 12 years of age or older with sickle cell disease and a history of vaso-occlusive events.

Lyfgenia uses an ex vivo lentiviral vector gene therapy which adds a functional copy of a modified βA-globin gene (threonine [T] into patients' HSCs through transduction of autologous CD34+ cells with BB305 LVV. After Lyfgenia infusion, the transduced CD34+ HSCs engraft in the bone marrow and differentiate to produce red blood cells containing biologically active βA-T87Q-globin that will combine with α-globin to produce functional Hb containing βA-T87Q-globin (HbAT87Q). HbAT87Q has similar oxygen-Lyfgenia (lovotibeglogene autotemcel) 2 binding affinity and oxygen hemoglobin dissociation curve to wild-type HbA, reduces intracellular and total hemoglobin S (HbS) levels, and is designed to inhibit polymerization of HbS thereby limiting the sickling of red blood cells.

NOTE: Lyfgenia can only be administered at a Lyfgenia Qualified Treatment Center (QTC). Each Lyfgenia QTC has been carefully selected based on their expertise in areas such as transplant, cell, and gene therapy. For information on locating a Qualified Treatment Center, please go to https://www.lyfgenia.com/find-a-qualified-treatment-center

NOTE: Please be aware that the Rhode Island Executive Office of Health and Human Services (EOHHS) has developed a prior authorization form for gene therapies for sickle cell disease. When requesting authorization for a gene therapy to treat a Tufts Health RITogether member with sickle cell disease, please submit this form directly to Point32Health.

Clinical Guideline Coverage Criteria

Casgevy

The Plan may cover Casgevy when all the following clinical criteria is met:

1. Documented diagnosis of SCD with confirmatory genetic testing; **and**
2. Age twelve (12) years and older; **and**
3. Prior use of or intolerance to hydroxyurea (per health care professional judgement) at any point in the past; and
4. Clinically stable and fit for transplantation; **and**
5. Prescribed in consultation with a board-certified hematologist with SCD experience; **and**
6. Experienced recurrent VOCs (defined as more than or equal to two (2) document VOCs per year in the previous twenty-four (24) months, based on provider attestation; **and**
7. No contraindication to the prescribed medication.

Lyfgenia

The Plan may cover Lyfgenia when all the following clinical criteria is met:

1. Documented diagnosis of SCD with confirmatory genetic testing; **and**
2. Age twelve (12) years and older at the expected time of gene therapy administration; **and**
3. Failure or intolerance to hydroxyurea (defined as being unable to take hydroxyurea per health care professional judgement) at any point in the past; **and**
4. Clinically stable and fit for transplantation; **and**
5. Prescribed Manufacturer CGT Product by or in consultation with a board-certified hematologist with SCD expertise.
6. Eligible Beneficiary's Treatment Center has a Sickle Cell Center; **and**
7. Either a or b (based on provider attestation):
   a. Currently receiving chronic transfusion therapy for recurrent Vaso-Occlusive Events (VOEs); **or**

b. Experienced four (4) or more VOEs in previous twenty-four (24) months as determined by the Eligible Beneficiary's treating clinician; **and**

8. No contradiction to the prescribed medication.

Any prior authorization, once approved, will be valid for at least twelve (12) months.

Codes

The following code(s) require prior authorization:

Table 1: HCPCS Codes

HCPCS Codes	Description
J3392	Injection, exagamglogene autotemcel, per treatment
J3394	Injection, lovotibeglogene autotemcel, per treatment

References:

1. Cell and Gene Therapy Access Model. Cell and Gene Therapy Access Model | Executive Office of Health and Human Services. Accessed December 12, 2025. https://eohhs.ri.gov/initiatives/medicaid-initiatives/cell-gene-therapy-access-model
2. Casgevy (exagamglogene autotemcel). [package insert]. Boston, MA: Vertex Pharmaceuticals Inc; Jan 2024.
3. Lyfgenia (lovotibeglogene autotemcel). [package insert]. Somerville, MA: bluebird bio Inc; Dec 2023.

Approval And Revision History

December 17, 2025: Reviewed by the Medical Policy Approval Committee (MPAC) effective January 1, 2026

Subsequent endorsement date(s) and changes made:

- March 16, 2026: Administrative update, added link to RI EOHHS prior authorization form for gene therapies for sickle cell disease

Background, Product and Disclaimer Information

Medical Necessity Guidelines are developed to determine coverage for benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. We make coverage decisions using these guidelines, along with the Member's benefit document, and in coordination with the Member's physician(s) on a case-by-case basis considering the individual Member's health care needs.

Medical Necessity Guidelines are developed for selected therapeutic or diagnostic services found to be safe and proven effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in our service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. We revise and update Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern. For Tufts Health Together (Medicaid), coverage may be available beyond these guidelines for pediatric members under age 21 under the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefits of the plan in accordance with 130 CMR 450.140 and 130 CMR 447.000, and with prior authorization.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this guideline is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to eligibility and benefits on the date of service, coordination of benefits, referral/authorization, utilization management guidelines when applicable, and adherence to plan policies, plan procedures, and claims editing logic.