Pharmacy Management Drug Policy

SUBJECT: Casgevy-exagamglogene autotemcel
POLICY NUMBER: PHARMACY-120
EFFECTIVE DATE: 06/2024
LAST REVIEW DATE: 11/19/2025

If the member's subscriber contract excludes coverage for a specific service or prescription drug, it is not covered under that contract. In such cases, medical or drug policy criteria are not applied. This drug policy applies to the following line/s of business:

Policy Application

Category:	☒ Commercial Group (e.g., EPO, HMO, POS, PPO)	☒ Medicare Advantage
	☒ On Exchange Qualified Health Plans (QHP)	☐ Medicare Part D
	☒ Off Exchange Direct Pay	☒ Essential Plan (EP)
	☒ Medicaid & Health and Recovery Plans (MMC/HARP)	☒ Child Health Plus (CHP)
	☐ Federal Employee Program (FEP)	☐ Ancillary Services
	☒ Dual Eligible Special Needs Plan (D-SNP)	

DESCRIPTION:

Sickle Cell Disease

Sickle Cell Disease (SCD) represents a group of genetic disorders characterized by structural abnormalities in hemoglobin (Hb). A single amino acid substitution is responsible for the production of sickle hemoglobin (HbS). There are several variant genotypes of the normal adult hemoglobin (Hb AA) that cause SCD, with the most prevalent including HbSS, HbSC, HbS/β+ thalassemia, and HbS/β0 thalassemia.[1] SCD affects millions worldwide, including an estimated 100,000 Americans.[2]

The main driver of sickle cell pathogenesis is polymerization of deoxygenated HbS, creating a distorted sickle shaped red blood cell (RBC). These rigid and inflexible RBCs have a higher propensity for hemolysis and adhering to vascular endothelial cells causing vaso-occlusion.[3] The repeated sickling and hemolysis causes a variety of complications including vaso-occlusive crisis (VOCs), hemolytic anemia, acute chest syndrome, stroke, pulmonary hypertension, deep vein thrombosis, infection, and splenic sequestration. VOCs are one of the main reasons for healthcare encounters.[4]

The main goal of SCD treatment is preventing and managing disease complications such as acute and chronic pain, and cerebrovascular, cardiopulmonary, and kidney disease. Lifestyle modifications, health screenings and other prevention strategies are recommended to optimize care. Disease modifying therapies (e.g., hydroxyurea, L-glutamine, voxelotor, and crizanlizumab) target various pathways involved in SCD to prevent sickling, reduce comorbid complications, and decrease VOCs. Allogenic hematopoietic stem cell transplantation (HSCT) is a potentially curative therapy but carries many risks (e.g., graft failure, infections, graft-versus-host disease, death) and <20% of individuals have a suitable matched donor.[1,5]

Beta-Thalassemia

Beta-Thalassemia is caused by a mutation in the beta-globulin gene resulting in reduced (β+) or absent (β0) beta-globulin production. The imbalanced ratio of alpha to beta-globulin chains leads to reduced Hb and dysfunctional erythropoiesis. The most severe form of beta-thalassemia is transfusion-dependent thalassemia (TDT) which requires lifelong transfusions to treat anemia. Approximately 3,000 individuals in the US have beta-thalassemia, half of which are transfusion-dependent.[6] Symptom presentation typically occurs within the first two years of life and include

severe anemia (e.g., dizziness, weakness, shortness of breath, fatigue), slowed growth, and organ damage due to iron overload. Frequent transfusions (every 2-5 weeks) and iron chelation therapy are considered standard treatment. mainstays of standard treatment. The erythroid maturation agent, luspatercept (Reblozyl), was FDA approved for use in adults with beta thalassemia requiring regular red blood cell (RBC) transfusions. Allogenic HSCT is potentially curative, though only 25% of patients have suitable matched donors. From an outcome perspective, children and young adolescents who receive a HSCT tend to have more favorable thalassemia-free survival than adults.[6]

Casgevy (exagamglogene autotemcel)
Casgevy *(exagamglogene autotemcel)* is an autologous genome edited hematopoietic stem cell-based gene therapy approved for use in patients 12 years and older with sickle cell disease (SCD) with recurrent vaso-occlusive crises (VOCs) and transfusion-dependent β-thalassemia (TDT). Casgevy uses clustered regularly interspaced palindromic repeats (CRISPR)/CRISPR-associated protein 9 (Cas9)–based gene editing. This technology allows for targeted manipulation of specific genes. In the case of SCD, Casgevy targets the BCL11A gene to reduce its expression and ultimately increase fetal hemoglobin (HbF) and γ-globin production. Elevated levels of fetal hemoglobin (HbF) have been associated with improved morbidity and mortality in patients with SCD and TDT. Typically, infants and neonates are asymptomatic due to elevated fetal hemoglobin levels and begin to show symptoms after HbF synthesis declines within the first year of life. [7-9] In TDT, the increase in γ-globin alleviates the imbalance between α-globin and non-α-globin chains resulting in improved erythropoiesis and hemoglobin levels and ultimately transfusion independence.

Casgevy is administered as a single dose (once per lifetime) via intravenous infusion with a single dose containing at minimum 3 x 10^6 CD34+ cells/kg of body weight. Prior to Casgevy administration, it must be confirmed that HSCT is appropriate for the patient. This includes screening for HIV-1, HIV-2, HBV, HCV, and any other infectious agents in accordance with local guidelines before collection of cells for manufacturing. Casgevy should not be used in patients with active HIV-1, HIV-2, HBV, or HCV. Disease modifying therapies (e.g., hydroxyurea, crizanlizumab, voxelotor) must be discontinued 8 weeks before the planned start of mobilization and conditioning. Patients must then undergo mobilization and apheresis procedures, followed by myeloablative conditioning before receiving Casgevy.[11]

FDA approval in SCD was based on results from CLIMB SCD-121 (NCT03745287), an ongoing, single-arm, multicenter trial. Patients were included between the ages of 12 to 35 years with a diagnosis of sickle cell disease with either βS/βS or βS/β0 or βS/β+ genotype and clinically stable Karnofsky performance status of ≥ 80% (for patients ≥ 16 years of age) or a Lansky performance status of ≥ 80% (for patients < 16 years of age. Additionally, patient had to have a history of at least 2 protocol-defined severe vaso-occlusive crisis (VOC) events during each of the 2 years prior to screening. Individuals were excluded from the trial if they had prior HSCT, availability of a 10/10 human leukocyte antigen matched related hematopoietic stem cell donor, advanced liver disease, clinically significant and active infection, significant bleeding disorder, history of Moyamoya disease, and contraindications to mobilization and myeloablative conditioning products.[11-13]

The primary efficacy outcome was proportion of V12 responders, defined as patients who did not experience severe VOCs for 12 consecutive months within the first 24 months after Casgevy infusion. A key secondary endpoint was proportion of patients who were free from hospitalization due to VOCs for at least 12 consecutive months within the 24-month evaluation period. An interim analysis included 31 patients eligible in the primary efficacy set (PES). The V12 response rate was 29/31 (93.5%; 98% one-sided CI: 77.9%, 100.0%). The HF response rate was 30/30 (100%; 98% one-sided CI: 87.8%, 100.0%).[11.]

Safety and efficacy in TDT were based on results from the ongoing open-label, multicenter, single arm CLIMB THAL-111 (NCT03655678). The study included adult and adolescent patients with TDT and a history of requiring at least 100 mL/kg/year or 10 units/year of RBC transfusions in the 2 years prior to enrollment. Key exclusions consisted of advanced liver disease, severely elevated iron in the heart (i.e., cardiac T2* less than 10 msec by magnetic resonance imaging [MRI] or left ventricular ejection fraction [LVEF] < 45% by echocardiogram), and available 10/10 human leukocyte antigen matched related hematopoietic stem cell donor. A total of 35 patients were included in the primary efficacy set (PES) at the time of interim analysis. The primary efficacy outcome was proportion of patients achieving transfusion independence for 12 consecutive months (TI12). A TI12 responder rate of 32/35 (91.4%, 98.3% one-sided CI: 75.7%, 100%) was achieved. The three patients who did not achieve TI12 had reduced annualized RBC transfusion volume requirements and annualized transfusion frequency, when compared to baseline.[11,12]

From a safety perspective, Casgevy carries warnings and precautions regarding neutrophil engraftment failure, delayed platelet engraftment, hypersensitivity reactions, and off-target genome editing risk. Common adverse reactions include mucositis and febrile neutropenia for both TDT and SCD patients, and decreased appetite in SCD patients. Laboratory abnormalities reported include neutropenia, thrombocytopenia, leukopenia, anemia, and lymphopenia.[11]

Other Gene Therapies in SCD and TDT

Lyfgenia is an autologous HSC-based gene therapy indicated for the treatment of sickle disease in patients ≥ 12 years of age with a history of vaso-occlusive events **[See Lyfgenia (lovotibeglogene autotemcel) (Pharmacy-119)].**[14] In August 2023, The Institute for Clinical and Economic Review (ICER) published a Final Evidence Report on sickle cell disease gene therapies. Majority of the panelists found the current evidence adequate to demonstrate a net health benefit for Lyfgenia and Casgevy when compared to standard of care. When comparing Lyfgenia to Casgevy, ICER noted there was inadequate evidence to distinguish a benefit of one therapy over another. [15] Casgevy and Lyfgenia have not been directly compared in clinical trials.

Zynteglo (betibeglogene autotemcel), a hematopoietic stem cell-based gene therapy, is indicated for treatment of adult and pediatric patients with β-thalassemia who require regular red blood cell (RBC) transfusions **[See Rare Diseases CRPA (Pharmacy-98)].**[16] Casgevy and Zynteglo have not been directly compared in clinical trials.

POLICY:

Casgevy (exagamglogene autotemcel) - Medical
Casgevy is considered medically necessary for treatment of sickle cell disease (SCD) with recurrent vaso-occlusive crises (VOCs) when the following are met: 1. Must be prescribed by a Hematologist or prescriber who specializes in management of sickle cell disease (SCD) **AND** a. Must be ≥ 12 years of age **AND** b. Must have documentation of all of the following: i. Patient has a confirmed diagnosis of sickle-cell disease with one of the following βS/βS or βS/β0 or βS/β+ genotype. Additional genotypes will be considered on a case-by-case basis based on disease severity **AND** ii. History of at least 2 severe vaso-occlusive events or crises (VOCs/VOEs) per year over the last 2 years. Severe VOC/VOE is defined as at least ONE of the following (1,2, 3, or 4):

1. Acute pain episode that requires a visit to a medical facility and administration of pain medications (opioids or IV NSAIDs) or RBC transfusions **OR**
2. Acute chest syndrome (ACS), as indicated by presence of new pulmonary infiltrate associated with pneumonia-like symptoms, pain, or fever **OR**
3. Hepatic sequestration **OR**
4. Priapism lasting more than 2 hours and leading to a medical-facility visit **AND**

iii. Must have experienced ONE of the following pertaining to hydroxyurea (1, 2, or 3):
1. A therapeutic failure (ex. continued frequent and/or severe VOCs or ongoing, frequent transfusion requirements) to a ≥ 6 consecutive month trial of hydroxyurea at maximum tolerated dosing. Adherence will be assess based on:
   a. Pharmacy refill history. If the patient does not have pharmacy benefits through this health plan, a recent pharmacy profile will be requested. Progress notes documenting usage of sample medication may also be requested **OR**
2. A contraindication to hydroxyurea defined as a hypersensitivity to hydroxyurea or any component of the formulation **OR**
3. Experienced two hematologic toxicity reactions with hydroxyurea that resulted in discontinuation of therapy
   a. Hematologic toxicity with hydroxyurea is defined by neutrophil, platelet, hemoglobin and/or reticulocyte count abnormalities concurrent with hydroxyurea use suggestive of hematologic toxicity. After the first hematologic toxicity, hydroxyurea therapy should be stopped and can be restarted with a dose reduction upon hematologic recovery. If a second hematologic toxicity is experienced, treatment should be discontinued **AND**

iv. Must have Karnofsky performance status of ≥80% for patients ≥16 years of age or Lansky performance status of ≥80% for patients < 16 years of age **AND**

c. Must be used as a single agent therapy (not applicable to myeloablative conditioning therapy) **AND**
d. The patient must be eligible to undergo hematopoietic stem cell transplant (HSCT) **AND**
e. Patient has been screened and found negative for active infections of the following: hepatitis B virus (HBV), hepatitis C virus (HCV), and human immunodeficiency virus 1 &2 (HIV-1/HIV-2) in accordance with clinical guidelines prior to collection of cells (documentation of laboratory results taken within the past 3 months is required) **AND**
f. Patient will not receive concomitant therapy with any of the following:
   i. Iron chelators for 7-days prior to mobilization and 6 months post-treatment (3-months post-treatment for non-myelosuppressive iron chelators) **AND**
   ii. Disease-modifying agents (e.g., voxelotor, crizanlizumab, L- glutamine) for at least 8-weeks prior to mobilization **AND**
   iii. Hydroxyurea for at least 2 months prior to mobilization **AND**
g. Prescriber must attest that the patient will not be re-started on hydroxyurea or other disease modifying therapies for SCD after receipt of Casgevy **AND**
h. Patients with any of the following will not be eligible for coverage:
   i. An accessible and willing 10/10 human leukocyte antigen (HLA)-matched related donor
   ii. Prior receipt of allogeneic or autologous HSCT
   iii. Prior treatment with gene therapy/editing product
   iv. Clinically significant and active bacterial, viral, fungal, or parasitic infection
   v. History of untreated Moyamoya disease or presence of Moyamoya disease
   vi. Advanced liver disease, defined as any of the following:
      1. Alanine transaminase (ALT) >3 × the upper limit of normal (ULN) or direct bilirubin value >2.5 × ULN
      2. Baseline prothrombin time (PT) (international normalized ratio [INR]) >1.5 × ULN

3. History of cirrhosis or any evidence of bridging fibrosis, or active hepatitis

vii. Baseline estimated glomerular filtration rate < 60 mL/min/1.73 m^2 (documentation of laboratory results taken within the past 3 months is required)

viii. Any contraindications to the use of plerixafor during the mobilization of hematopoietic stem cells and any contraindications to the use of busulfan and any other medicinal products required during the myeloablative conditioning, including hypersensitivity to the active substances or to any of the excipients.

ix. Any prior or current malignancy or myeloproliferative disorder or a significant immunodeficiency disorder.

x. History of familial cancer syndrome

i. Casgevy is indicated for one-time single one-time single-dose intravenous use only and therefore will not be authorized for retreatment. Retreatment will be considered Experimental/Investigational when any FDA approved gene therapy, or any other gene therapy under investigation, has been previously administered.

j. The minimum recommended dose of Casgevy is 3 × 10^6 CD34+ cells/kg and will be approved as a one-time dose

i. Please refer to Casgevy FDA-approve prescribing information for complete dosage and administration instructions

ii. Casgevy is for autologous use only **OR**

Casgevy is considered medically necessary for treatment of <u>transfusion-dependent β-thalassemia (TDT)</u> when the following are met:

2. Must be prescribed by a Hematologist or provider who specializes in management of beta thalassemia **AND**
   a. Must be ≥ 12 years of age **AND**
   b. Must have documented diagnosis of transfusion-dependent β-thalassemia (TDT) as defined by:
      i. Homozygous β-thalassemia or compound heterozygous β-thalassemia including β-thalassemia/hemoglobin E (HbE) confirmed on genetic testing **AND**
      ii. History of at least 100 mL/kg/year or ≥10 units/year of packed RBC transfusions in the prior 2 years **AND**
   c. Must have a Karnofsky performance status of ≥80% for subjects ≥16 years of age or a Lansky performance status of ≥80% for subjects <16 years of age **AND**
   d. Patient must be eligible to undergo hematopoietic stem cell transplant (HSCT) **AND**
   e. Patient has been screened and found negative for active infections of the following: hepatitis B virus (HBV), hepatitis C virus (HCV), and human immunodeficiency virus 1 &2 (HIV-1/HIV-2) in accordance with clinical guidelines prior to collection of cells (documentation of laboratory results taken within the past 3 months is required) **AND**
   f. Patient will not receive therapy concomitantly with any of the following:
      i. Iron chelators for 7-days prior to mobilization and 6 months post-treatment (3-months post-treatment for non-myelosuppressive iron chelators) **AND**
      ii. Disease-modifying agents (e.g., voxelotor, crizanlizumab, gluatmine) for at least 8-weeks prior to mobilization **AND**
      iii. Hydroxyurea for at least 2 months prior to mobilization **AND**
   g. Patients with any of the following will not be eligible for coverage:
      i. An accessible and willing 10/10 human leukocyte antigen (HLA)-matched related donor
      ii. Prior receipt of allogeneic or autologous HSCT
      iii. Prior treatment with gene therapy/editing product
      iv. Significant and active bacterial, viral, fungal, or parasitic infection
      v. α-thalassemia and >1 alpha chain deletion, or alpha multiplications

vi. White blood cell (WBC) count <3 × 10^9/L or platelet count <50 × 10^9/L not related to hypersplenism (documentation of laboratory results taken within the past 3 months is required)
1. NOTE: If patient has a white blood cell count less than 3 X 10^9/L, and/or platelet count less than 100 X 10^9/L, then documentation of hypersplenism (i.e., ultrasound) is required

vii. Any prior or current malignancy or myeloproliferative disorder or a significant immunodeficiency disorder

viii. A cardiac T2* <10 ms by MRI or left ventricular ejection fraction (LVEF) <45% by echocardiogram

ix. Baseline estimated glomerular filtration rate <60 mL/min/1.73 m2 (documentation of laboratory results taken within the past 3 months is required)

x. Any contraindications to the use of plerixafor or granulocyte colony stimulating factor (G-CSF) during the mobilization of hematopoietic stem cells and any contraindications to the use of busulfan and any other medicinal products required during the myeloablative conditioning, including hypersensitivity to the active substances or to any of the excipients.

xi. Advanced liver disease, defined as any of the following:
A. Aspartate transaminase (AST), alanine transaminase (ALT) >3 x the upper limit of normal (ULN), or direct bilirubin value >2.5 x the ULN
B. Baseline prothrombin time (International Normalized Ratio; INR) >1.5 x ULN
C. History of cirrhosis or any evidence of bridging fibrosis, or active hepatitis on previous liver biopsy.
D. Liver iron content (LIC) ≥15 mg/g on R2* MRI of liver **AND**

h. Casgevy is indicated for one-time single one-time single-dose intravenous use only and therefore will not be authorized for retreatment. Retreatment will be considered Experimental/Investigational when any FDA approved gene therapy, or any other gene therapy under investigation, has been previously administered.

i. The minimum recommended dose of Casgevy is 3 × 10^6 CD34+ cells/kg and will be approved as a one-time dose
i. Please refer to Casgevy FDA-approve prescribing information for complete dosage and administration instructions
ii. Casgevy is for autologous use only

3. Casgevy (exagamglogene autotemcel) is considered **investigational** when the above criteria are not met.
4. Casgevy (exagamglogene autotemcel) is considered **investigational** for all other indications.
5. Authorization will be for 6 months to allow sufficient time for administration.

POLICY GUIDELINES:

1. Prior-authorization is contract dependent.
2. Dose and frequency should be in accordance with the FDA label or recognized compendia (for off-label uses). When services are performed in excess of established parameters, they may be subject to review for medical necessity.
3. This policy does not apply to Medicare Part D and D-SNP pharmacy benefits. The drugs in this policy may apply to all other lines of business including Medicare Advantage.
4. For members with Medicare Advantage, medications with a National Coverage Determination (NCD) and/or Local Coverage Determination (LCD) will be covered pursuant to the criteria outlined by the NCD and/or LCD. NCDs/LCDs for applicable medications can be found on the CMS website at https://www.cms.gov/medicare-coverage-database/search.aspx. Indications that have not been addressed by the applicable medication's LCD/NCD will be covered in accordance with criteria determined by the Health Plan (which may include review per the Health Plan's Off-

Label Use of FDA Approved Drugs policy). Step therapy requirements may be imposed in addition to LCD/NCD requirements.
5. Not all contracts cover all Medical Infusible drugs. Refer to specific contract/benefit plan language for exclusions of Injectable Medications.
6. Clinical documentation must be submitted for each request (initial and recertification) unless otherwise specified (e.g., provider attestation required). Supporting documentation includes, but is not limited to, progress notes documenting previous treatments/treatment history, diagnostic testing, laboratory test results, genetic testing/biomarker results, and imaging.
7. All requests will be reviewed to ensure they are being used for an appropriate indication and may be subject to an off-label review in accordance with our Off-Label Use of FDA Approved Drugs Policy (Pharmacy-32).
8. All utilization management requirements outlined in this policy are compliant with applicable New York State insurance laws and regulations. Policies will be reviewed and updated as necessary to ensure ongoing compliance with all state and federally mandated coverage requirements.
9. Manufacturers may either discontinue participation in, or may not participate in, the Medicaid Drug Rebate Program (MDRP). Under New York State Medicaid requirements, physician-administered drugs must be produced by manufacturers that participate in the MDRP. Products made by manufacturers that do not participate in the MDRP will not be covered under Medicaid Managed Care/HARP lines of business. Drug coverage will not be available for any product from a non-participating manufacturer. For a complete list of New/Reinstated & Terminated Labelers please visit: https://www.medicaid.gov/medicaid/prescriptiondrugs/medicaid-drug-rebate-program/newreinstated-terminated-labeler-information/index.html

CODES:

Eligibility for reimbursement is based upon the benefits set forth in the member's subscriber contract.

CODES MAY NOT BE COVERED UNDER ALL CIRCUMSTANCES. PLEASE READ THE POLICY AND GUIDELINES STATEMENTS CAREFULLY.

Codes may not be all inclusive as the AMA and CMS code updates may occur more frequently than policy updates.

Code Key:

Experimental/Investigational = (E/I),
Not medically necessary/ appropriate = (NMN).

HCPCS: J3392

UPDATES:

Date	Revision
11/19/2025	Revised
10/16/2025	Revised
05/08/2025	Reviewed / P&T Committee Approval
03/06/2025	Revised
01/01/2025	Revised
09/13/2024	Revised
06/20/2024	Revised

06/17/2024	Created and Implemented
05/09/2024	P&T Committee Approval

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