New Hampshire Medicaid Fee-for-Service Program Casgevy™ (exagamglogene autotemcel) Criteria

Approval Date: November 21, 2024

Medications

Brand Names	Generic Names	Indication
Casgevy™	exagamglogene autotemcel	• Treatment of patients 12 years of age and older with sickle cell disease with recurrent vaso-occlusive crises (VOCs) • Treatment of patients 12 years of age and older with transfusion-dependent β-thalassemia (TDT)

Criteria for Approval

All indications

1. Patient is 12 years of age or older; **AND**
2. Provider has considered use of prophylaxis therapy for seizures prior to initiating myeloablative conditioning; **AND**
3. Patient has been screened and found negative for hepatitis B virus (HBV), hepatitis C virus (HCV), and human immunodeficiency virus 1 and 2 (HIV-1/HIV-2) in accordance with clinical guidelines prior to collection of cells (leukapheresis); **AND**
4. Patient does **not** have a history of hypersensitivity to dimethyl sulfoxide (DMSO) or dextran 40; **AND**
5. Patient has **not** received other gene therapy (e.g., Lyfgenia® [lovotibeglogene autotemcel], Zynteglo® [betibeglogene autotemcel])*; **AND**
6. Patient will **not** receive therapy concomitantly with any of the following:
   - Iron chelators for 7 days or more prior to myeloablative conditioning and 6 months post-treatment for myelosuppressive iron chelators (3 months post-treatment for non-myelosuppressive iron chelators); **AND**
   - Disease-modifying agents (e.g., hydroxyurea, voxelotor, crizanlizumab) for 8 weeks or more prior to mobilization and conditioning; **AND**
7. Patient is a candidate for autologous hematopoietic stem cell transplant (HSCT) and has **not** had prior HSCT; **AND**
8. Patient does **not** have a known 10/10 human leukocyte antigen (HLA) matched related donor willing to participate in an allogeneic HSCT; **AND**
9. Casgevy must not be administered concurrently with live vaccines while the patient is immunosuppressed.

Sickle cell disease (SCD) criteria

1. Patient has a confirmed diagnosis of sickle-cell disease (includes genotypes βS/βS or βS/β0 or βS/β+) as determined by one of the following:
   - Identification of significant quantities of HbS with or without an additional abnormal β-globin chain variant by hemoglobin assay; **OR**
   - Identification of biallelic HBB pathogenic variants where 1 allele or more is the p.Glu6Val pathogenic variant based on molecular genetic testing; **AND**
2. Patient has symptomatic disease despite treatment with hydroxyurea or add-on therapy (e.g., crizanlizumab); **AND**
3. Patient experienced 2 or more vaso-occlusive events/crises (VOE/VOC)† in the previous year while adhering to the above therapy; **AND**
4. Patient will be transfused prior to apheresis to a total Hb less than or equal to 11 g/dL and a HbS level less than 30% and patient will be transfused 8 weeks or more prior to initiation of myeloablative conditioning (with aforementioned Hb and HbS goals); **AND**
5. Patient will **not** receive granulocyte-colony stimulating factor (G-CSF) for the mobilization of hematopoietic stem cells (HSC).

Transfusion-dependent beta-thalassemia (TDT) criteria

1. Patient has a documented diagnosis of homozygous beta thalassemia or compound heterozygous beta thalassemia including β-thalassemia/hemoglobin E (HbE) as outlined by the following:
   - Patient diagnosis is confirmed by *HBB* sequence gene analysis showing biallelic pathogenic variants; **OR**
   - Patient has a severe microcytic hypochromic anemia, absence of iron deficiency, anisopoikilocytosis with nucleated red blood cells on peripheral blood smear, and hemoglobin analysis that reveals decreased amounts or complete absence of hemoglobin A (HbA) and increased HbA2 with or without increased amounts of hemoglobin F (HbF); **AND**
2. Patient has transfusion-dependent disease defined as a history of transfusions of 100 mL/kg/year or more or 10 units/years or more of packed red blood cells (pRBCs) in the 2 years preceding therapy; **AND**
3. Patient will be transfused prior to achieving a total Hb 11 g/dL or more for 60 days prior to myeloablative conditioning; **AND**
4. Patient does **not** have any of the following:
   - Severely elevated iron in the heart (e.g., patients with cardiac T2* less than 10 msec by magnetic resonance imaging [MRI] or left ventricular ejection fraction [LVEF] less than 45% by echocardiogram); **OR**
   - Advanced liver disease (e.g., aspartate transaminase [AST] or alanine transaminase [ALT] more than 3 times the upper limit of normal [ULN], or direct bilirubin value more than 2.5 times the ULN, or if a liver biopsy demonstrated bridging fibrosis or cirrhosis).

**Requests for subsequent use of exagamglogene autotemcel after receipt of other gene therapies (e.g., lovotibeglogene autotemcel, betibeglogene autotemcel) will be evaluated on a case-by-case basis.*

†VOE/VOC is defined as an event requiring a visit to a medical facility for evaluation which results in a diagnosis of such being documented due to one (or more) of the following: acute pain, acute chest syndrome, acute splenic sequestration, acute hepatic sequestration, priapism lasting > 2 hours ***and*** *necessitating subsequent interventions such as opioid pain management, non-steroidal anti-inflammatory drugs, RBC transfusion, etc.*

Criteria for Denial

Above criteria are not met.

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	05/07/2024
Commissioner designee	Approval	06/10/2024
DUR Board	Revision	10/15/2024
Commissioner designee	Approval	11/21/2024