New Hampshire Medicaid Fee-for-Service Program Zynteglo (betibeglogene autotemcel) Criteria

Approval Date: June 10, 2024

Medications

Brand Names	Generic Names	Indication
Zynteglo	betibeglogene autotemcel	Treatment of adult and pediatric patients with β-thalassemia who require regular red blood cell (RBC) transfusion

Criteria for Approval

1. Patient is 4 years of age or older; **AND**
2. Patient has a documented diagnosis of beta thalassemia (excludes alpha-thalassemia and hemoglobin S/β-thalassemia variants) as outlined by the following:
   - Patient diagnosis is confirmed by *HBB* sequence gene analysis showing biallelic pathogenic variants; **OR**
   - Patient has severe microcytic hypochromic anemia, anisopoikilocytosis with nucleated red blood cells on peripheral blood smear, and hemoglobin analysis that reveals decreased amounts or complete absence of hemoglobin A and increased amounts of hemoglobin F; **AND**
3. Patient has transfusion-dependent disease defined as a history of transfusions of at least 100 mL/kg/year of packed red blood cells (pRBCs) or with 8 or more transfusions of pRBCs per year in the 2 years preceding therapy; **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**
   - Advanced liver disease; **OR**
   - Patients with an MRI of the liver with results demonstrating liver iron content 15 mg/g or more (unless biopsy confirms absence of advanced disease); **AND**
5. Patient has been screened for hepatitis B virus (HBV), hepatitis C virus (HCV), human T-lymphotrophic virus 1 and 2 (HTLV-1/HTLV-2), and human immunodeficiency virus (HIV) in accordance with clinical guidelines prior to the collection of cells (leukapheresis); **AND**
6. Patient has not used prophylactic HIV anti-retroviral medication or hydroxyurea within 30 days of mobilization (or for the expected duration for elimination of those medications) and until all cycles of apheresis are completed (**Note:** if a patient requires anti-retrovirals for HIV prophylaxis, confirm a negative test for HIV before beginning mobilization); **AND**
7. Iron chelation therapy has been discontinued for 7 days or more prior to initiating myeloablative conditioning therapy; **AND**

8. Females of reproductive potential have a negative pregnancy test prior to start of mobilization and re-confirmed prior to conditioning procedures and again before administration of betibeglogene autotemcel; **AND**
9. Used as single agent therapy (not applicable to lymphodepleting or bridging therapy while awaiting manufacture); **AND**
10. Patient will receive periodic life-long monitoring for hematological malignancies; **AND**
11. Patient is eligible to undergo hematopoietic stem cell transplant (HSCT) and has **not** had prior HSCT or other gene-therapy; **AND**
12. Coverage will be provided for one treatment course (1 dose of Zynteglo) and may not be renewed.

Criteria for Denial

Above criteria are not met.

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	12/13/2022
Commissioner	Approval	01/26/2023
DUR Board	Revision	05/07/2024
Commissioner designee	Approval	06/10/2024