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/ß-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