

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

Approval Date: June 10, 2024

### **Medications**

Brand Names	Generic Names	Indication
Zynteglo	betibeglogene	Treatment of adult and pediatric patients with $\beta$ -thalassemia
	autotemcel	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 Tlymphotrophic 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**

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- 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