ALASKA MEDICAID
Prior Authorization Criteria

Zynteglo®
(betibeglogene-autoemcel)

FDA INDICATIONS AND USAGE[1]

Zynteglo® is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of adult and pediatric patients with β-thalassemia who require regular red blood cell (RBC) transfusions.

APPROVAL CRITERIA[1,2,3]

1. Patient is 4 years of age or older **AND**;
2. Prescribed by or in consultation with a hematologist **AND**;
3. Patient has the diagnosis of β-thalassemia confirmed by genetic testing **AND**;
4. Patient weighs a minimum of 6 kg **AND**;
5. Patient is able to provide an adequate number of cells to meet the minimum recommended dose of 5.0x10^6 CD34+ cells/kg **AND**;
6. Patient has obtained a negative test result for hepatitis B virus (HBV), hepatitis C virus (HCV), human immunodeficiency virus (HIV), and human T-lymphotropic virus 1&2 (HTLV1&HTLV2) prior to collection of cells **AND;**
7. Patient has a documented history of one of the following
    a. Documented history of ≥100mL/kg/year of pRBCs in the immediately preceding two years **OR**
    b. Documentation of ≥8 transfusions of pRBCs in the immediately preceding two years

DENIAL CRITERIA [1]

1. Failure to meet approval criteria **OR**;
2. Patient has previously received gene therapy for treatment of transfusion dependent thalassemia (TDT) **OR**;
3. Patient has previously received an allogeneic hematopoietic stem cell transplant (HSCT) for treatment of TDT

CAUTIONS[1]

- Due to the risk of delayed or failed engraftment, monitor platelet and neutrophil counts following infusion.
- Patients must be monitored for hematologic malignancy at least annually for a minimum of 15 years following infusion.
- Avoid use of anti-retroviral medications or hydroxyurea beginning a minimum of one month, or for the expected duration of elimination of all such medications, prior to mobilization until all cycles of apheresis are completed.
- Discontinue use of iron chelators seven days prior to initiation of myeloablative conditioning and avoid use until six months after Zynteglo infusion.
- Zynteglo® contains dimethyl sulfoxide (DMSO) which may cause hypersensitivity reactions, including anaphylaxis.

DURATION OF APPROVAL

- Initial Approval: up to 6 months
- Reauthorization will not be approved

QUANTITY LIMIT

- One infusion per lifetime
- HCPCS: J3393

REFERENCES / FOOTNOTES:

1. Zynteglo (betibeglogene autotemcel) [prescribing information]. Sommerville, MA: Bluebird Bio; August 2022
2. Locatelli F., Thompson A., Kwiatkowski J., et al. Betibeglogene Autotemcel Gene Therapy for Non-β(0)/β(0) Genotype β-Thalassemia. N Engl J Med. 2022;386(5):415-427. DOI: 10.1056/NEJMoa2113206.
3. Institute for Clinical and Economic Review: Draft Evidence Report – Betibeglogene Autotemcel for Beta Thalassemia: Effectiveness and Value . July 19, 2022. Available at: https://icer.org/wp-content/uploads/2023/09/ICER_Beta-Thalassemia_Final-Report_12-Month_Update.pdf