betibeglogene autotemcel (Zynteglo)

Commercial Medical Benefit Drug Policy

Place of Service
Hospital Administration

Drug Details

USP Category: GENETIC OR ENZYME OR PROTEIN DISORDER: REPLACEMENT, MODIFIERS, TREATMENT
Mechanism of Action: Autologous hematopoietic stem cell-based gene therapy containing transgene that encodes for βA-T787Q-globin.
HCPCS:
J3393:Injection, betibeglogene autotemcel, per treatment
How Supplied:

- Provided as a single dose for infusion containing a suspension of CD34+ cells in one or more (up to 4) infusion bags. The minimum recommended dose of ZYNTEGLO is 5.0×10^6 CD34+ cells/kg.
- Zynteglo is shipped from the manufacturing facility to the treatment center storage facility in a cryoshipper, which may contain multiple metal cassettes intended for a single patient.

Condition(s) listed in policy *(see coverage criteria for details)*

- Transfusion-Dependent Beta-Thalassemia (TDT)

Any condition not listed in this policy requires a review to confirm it is medically necessary. For conditions that have not been approved for intended use by the Food and Drug Administration (i.e., off-label use), the criteria outlined in the Health and Safety Code section 1367.21 must be met.

Special Instructions and Pertinent Information

Provider must submit documentation (such as office chart notes, lab results or other clinical information) to ensure the member has met all medical necessity requirements.

The member's specific benefit may impact drug coverage. Other utilization management processes, and/or legal restrictions may take precedence over the application of this clinical criteria.

For billing purposes, drugs must be submitted with the drug's assigned HCPCS code (as listed in the drug policy) and the corresponding NDC (national drug code). An unlisted, unspecified, or miscellaneous code should not be used if there is a specific code assigned to the drug.

Coverage Criteria
The following condition(s) require Prior Authorization/Preservice.

Transfusion-Dependent Beta-Thalassemia (TDT)
Meets medical necessity if all the following are met:

1. Transfusion dependence, defined as at least 8 transfusions of packed red blood cell (pRBC) in previous 12 months
2. Both of the following:
   a. Patient ≤50 years of age
   b. If <5 years of age: minimum weight of 6 kg, and able to provide minimum number of cells to perform apheresis (e.g., needed to manufacture product)

3. Patient is clinically stable and eligible to undergo myeloablative conditioning before infusion of Zynteglo
4. White blood cell (WBC) count ≥3 x 10^9 /L
5. Platelet count ≥100 x 10^9 /L (unless related to hypersplenism)
6. Patient is negative for HIV-1, HIV-2, hepatitis B virus (HBV), and hepatitis C virus (HCV)
7. Patient does not have any of the following:
   a. Uncorrected bleeding disorder
   b. Any prior or current malignancy (with the exception of adequately treated cone-biopsied in situ carcinoma of the cervix uteri and basal or squamous cell carcinoma of the skin)
   c. History of major organ damage (e.g., advanced liver disease, cardiac T2 <10 ms by MRI)
   d. Severe iron overload that warrants exclusion in the provider's opinion

Covered Doses:
Up to four infusions bags, which contain 2.0 to 20 x 10^6 cells/mL, as a single-dose intravenous (IV) infusion

Coverage Period:
One-time treatment per lifetime

ICD-10:
D56.1

References

1. AHFS. Available by subscription at http://www.lexi.com
2. DrugDex. Available by subscription at http://www.micromedexsolutions.com/home/dispatch
3. Zynteglo (betibeglogene autotemcel) Prescribing Information. bluebird bio, Inc., Somerville, MA: 08/2022.

Review History

Date of Last Annual Review: 4Q2025
Changes from previous policy version:

- No clinical change following annual review.

Blue Shield of California Medication Policy to Determine Medical Necessity
Reviewed by P&T Committee