

CAREFIRST: ZYNTGLO

Client Requested: The intent of the criteria is to ensure that patients follow selection elements as established by CareFirst.

COVERAGE CRITERIA

The requested drug will be covered with prior authorization when the following criteria are met:

FDA-Approved Indication

Zynteglo is indicated for the treatment of adult and pediatric patients with beta-thalassemia who require regular blood cell (RBC) transfusions.

All other indications are considered experimental/investigational and not medically necessary.

I. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a hematologist.

II. CRITERIA FOR INITIAL APPROVAL

Beta-thalassemia

Authorization of 3 months for a one-time administration may be granted when all of the following criteria are met:

- A. Patient is 50 years old or younger at time of treatment decision
- B. Member is 4 years of age or older and meets both of the following criteria:
 1. Member weighs at least 6 kg
 2. Member is reasonably anticipated to provide at least the minimum number of cells required to initiate the manufacturing process
- C. Member has a diagnosis of transfusion-dependent beta-thalassemia with a non- β^0/β^0 OR β^0/β^0 genotype confirmed via genetic testing (Appendix A)
- D. Member requires regular blood cell transfusions and meets one of the following criteria within the previous two years:
 1. Member has received at least 100 milliliter per kilogram of packed red blood cells (pRBCs) per year
 2. Member has received at least 8 transfusions events of packed red blood cells (pRBCs) per year
- E. Member is clinically stable and eligible for a hematopoietic stem cell transplant (HSCT) but is unable to find a matched related donor
- F. Member has not received prior hematopoietic stem cell transplant (HSCT)
- G. Member has not received Zynteglo or any other gene therapy previously
- H. Member does not have any of the following conditions:
 1. Positive for the presence of human immunodeficiency virus type 1 or 2 (HIV-1 and HIV-2), hepatitis B virus (HBV), or hepatitis C (HCV)
 2. Any prior or current malignancy
 3. Advanced liver disease (e.g., bridging fibrosis, cirrhosis, active hepatitis)
 4. Severely elevated iron in the heart (i.e., patients with cardiac T2* less than 10 msec by MRI)
 5. Severe iron overload in physician's opinion warrants exclusion.
 6. A white blood cell (WBC) count $<3 \times 10^9/L$, and/or platelet count $<100 \times 10^9/L$ not related to hypersplenism.
 7. Uncorrected bleeding disorder.
 8. Availability of human leukocyte antigen- identical or human leukocyte antigen- matched donor.

Reference number(s)
C26815-A

Appendix A

Examples of non-beta-0 OR beta-0 genotypes:

1. Beta-0/beta-0
2. Beta-0/Beta+
3. Beta-E/Beta-0
4. Beta-0/IVS-I-110
5. IVS-I-110/IVS-1-110

DOCUMENT HISTORY

Created: Specialty Clinical Development (JS) 11/2023
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