



STATE OF WEST VIRGINIA
DEPARTMENT OF HUMAN SERVICES
BUREAU FOR MEDICAL SERVICES



Office of Pharmacy Services
Prior Authorization Criteria

ZYNTGLO® (betibeglogene autotemcel)
Billed under: J3590
Restricted to ICD-10 D56.1

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 RBC transfusions.

Beta-thalassemia (β -thalassemia), or beta-thal, is a genetic disease characterized by anemia, a condition which results from a shortage of healthy red blood cells (RBCs). Without sufficient healthy RBCs, cells and tissues throughout the body do not get the oxygen they need to function. Anemia causes people to feel tired, weak or short of breath. In the most severe form of beta-thal, also referred to as transfusion dependent thalassemia (TDT), patients require lifelong regular red blood cell transfusions to survive. If left untreated, the disease can damage organs and potentially lead to death.

Patients with TDT require lifelong blood transfusions, often every 2-5 weeks to suppress their body's production of abnormal hemoglobin. Frequent transfusions often lead to iron overload, which is the main source of morbidity and mortality in TDT. Hematopoietic stem cell transplantation (HSCT) is currently the only curative treatment for TDT; however, the lack of compatible donors is a limiting factor as it is estimated that only 25% of patients have access to a compatible related or unrelated donor. Emerging gene therapies (such as Zynteglo) offer a potentially curative alternative.

Initial authorization requires review by the Medical Director and may be approved when all of the following criteria is met:

1. Must be prescribed by a Hematologist; **AND**
2. Patient has not received Zynteglo or any other related gene therapy previously; **AND**
3. Patient must be at least 4 years of age; **AND**
4. Patient must weigh at least 6 kilograms; **AND**



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5. Patient must have a confirmed diagnosis of transfusion dependent beta-thalassemia with a non- β^0/β^0 or β^0/β^0 genotype confirmed via genetic testing (genetic test results must be supplied) ; **AND**
6. Patient is transfusion dependent defined by the patient meeting one of the following conditions within the previous two (2) years:
 - a. Having received at least 100 ml/kg packed red blood cells (pRBC's) per year;
OR
 - b. Having received at least 8 transfusion events of pRBC's per year; **AND**
7. Patient is eligible for, but has not received a prior hematopoietic stem cell transplant (HSCT); **AND**
8. Females of reproductive potential must have documentation of a negative pregnancy test prior to the start of therapy; **AND**
9. Patient has been screened and not tested positive for human immunodeficiency virus (HIV), hepatitis B virus (HBV) or hepatitis C virus (HCV); **AND**
10. Patient must not have any prior or current malignancy or advanced liver disease (e.g. bridging fibrosis, cirrhosis, or active hepatitis); **AND**
11. Patient must not have severely elevated cardiac iron (i.e. cardiac T2* less than 10 milliseconds by magnetic resonance imaging (MRI)).

All criteria requirements must be acknowledged and documented prior to approval of Zynteglo. Supporting diagnostic documentation MUST be supplied. If any of the above criteria are not met or not documented in the prior authorization request, coverage will be denied.



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References:

Government Agency, Medical Society, and Other Authoritative Publications:

1. CPB 1016 – Betibeglogene Autotemcel (Zynteglo). Aetna. (2023, November 7). https://www.aetna.com/cpb/medical/data/1000_1099/1016.html; (Accessed 6/27/2024)
2. Zynteglo [package insert]. Somerville, MA: bluebird bio, Inc.; 2022. https://www.bluebirdbio.com/-/media/bluebirdbio/Corporate%20COM/Files/Zynteglo/ZYNTEGLO_prescribing_information.pdf (Accessed 6/24/2024)
3. UpToDate clinical article “Management of thalassemia” – Revised 5/02/2024; (Accessed 6/19/2024)
4. UpToDate Lexidrug Monograph “Betibeglogene Autotemcel” – Revised 2/24/2024; (Accessed 6/19/2024)
5. ICER Evidence Report on Gene Therapy for Beta Thalassemia: “Betibeglogene Autotemcel for Beta Thalassemia: Effectiveness and Value” – Published 6/2/2022; (Accessed 6/19/2024)
6. Mol Genet Genomic Med. 2021 Dec; 9(12): e1788. Published online 2021 Nov 5. doi: 10.1002/mgg3.1788 “Current status of beta-thalassemia and its treatment strategies”
7. Our focus: Transfusion-dependent beta-thalassemia (TDT): Bluebird bio. Bluebird bio| Pioneering Gene Therapies. (2023, July). <https://www.bluebirdbio.com/our-focus/beta-thalassemia>

Please refer to the member’s contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member according to BMS coverage and policy guidelines.