Prior Authorization Review Panel

CHC-MCO Policy Submission

A separate copy of this form must accompany each policy submitted for review.
Policies submitted without this form will not be considered for review.

Plan: PA Health & Wellness	**Submission Date: 07/2024**
Policy Number: PA.CP.PHAR.545	**Effective Date: 11/2022** **Revision Date: 07/2024**
Policy Name: Betibeglogene Autotemcel (Zynteglo)	

Type of Submission – Check all that apply:

- ☐ **New Policy**
- ✓ **Revised Policy***
- ☐ **Annual Review - No Revisions**
- ☐ **Statewide PDL -** *Select this box when submitting policies for Statewide PDL implementation and when submitting policies for drug classes included on the Statewide PDL.*

***All revisions to the policy must be highlighted using track changes throughout the document.**

Please provide any changes or clarifying information for the policy below:

Updated to match DHS criteria, effective 07/15/2024.

Name of Authorized Individual (Please type or print): **Craig A. Butler, MD MBA**	**Signature of Authorized Individual:** 

Clinical Policy: Betibeglogene Autotemcel (Zynteglo)

Reference Number: PA.CP.PHAR.545
Effective Date: 11/2022
Last Review Date: 07/2024

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness® that Betibeglogene Autotemcel (Zynteglo) is **medically necessary** when the following criteria are met:

I. Requirements for Prior Authorization of Zynteglo (betibeglogene autotemcel)

A. Prescriptions That Require Prior Authorization

All prescriptions for Zynteglo (betibeglogene autotemcel) must be prior authorized.

B. Review of Documentation for Medical Necessity

In evaluating a request for prior authorization of a prescription for Zynteglo (betibeglogene autotemcel), the determination of whether the requested prescription is medically necessary will take into account whether the member:

1. Is prescribed Zynteglo (betibeglogene autotemcel) for an indication that is included in the U.S. Food and Drug Administration (FDA)-approved package labeling; AND
2. Is age-appropriate according to FDA-approved package labeling; AND
3. Is prescribed a dose and number of treatments that are consistent with FDA-approved package labeling; AND
4. Is prescribed Zynteglo (betibeglogene autotemcel) by a specialist at an authorized treatment center for Zynteglo (betibeglogene autotemcel); AND
5. Does not have a contraindication to the prescribed medication; AND
6. Is not a prior recipient of gene therapy or an allogeneic hematopoietic stem cell transplant; AND
7. For treatment of transfusion-dependent β-thalassemia, both of the following:
    i. Has genetic testing confirming diagnosis of β-thalassemia

ii. Has a history of at least 100 mL/kg/year or 8 transfusion episodes/year of packed red blood cell transfusions in the prior 2 years.

NOTE: If the member does not meet the clinical review guidelines above but, in the professional judgement of the physician reviewer, the services are medically necessary to meet the medical needs of the member, the request for prior authorization will be approved.

C. Clinical Review Process

Prior authorization personnel will review the request for prior authorization and apply the clinical guidelines in Section B. above to assess the medical necessity of a prescription for a Zynteglo (betibeglogene autotemcel). If the guidelines in Section B. are met, the reviewer will prior authorize the prescription. If the guidelines are not met, the prior authorization request will be referred to a physician reviewer for a medical necessity determination. Such a request for prior authorization will be approved when, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the member

D. Dose and Duration of Therapy

Requests for prior authorization of Zynteglo (betibeglogene autotemcel) will be approved for 18 months for 1 infusion.

Appendix C: Contraindications/Boxed Warnings

- None reported

Appendix D: General Information

- Conversion of RBC units from mL: 1 RBC unit in these criteria refers to a quantity of pRBC approximately 200-350 mL.
  - Sites who use transfusion bags within this range, or ≥ 350 mL, the conversion in units should be done by dividing the volume transfused to the patient by 350 mL.
  - Sites who use transfusion bags < 200 mL, the conversion in units should be done by dividing the volume transfused to the patient by 200 mL.
- Examples of advanced liver disease include, but are not limited to, the following:
  - Cirrhosis
  - Active hepatitis
  - Bridging fibrosis
  - Fatty liver disease

Appendix E: Genetic Confirmation of β-Thalassemia

Beta Thalassemia Genotype Examples
β^0/β^0
β^0/β^+
β^+/β^+
β^E/β^0

Beta Thalassemia Genotype Examples
β^+ IVS1-110/β^+ IVS1-110
β^0 /β^+ IVS1-110

I. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
β-thalassemia	Minimum dose: 5×10^6 CD34+ cells/kg	No maximum dose

II. Product Availability

Single-dose cell suspension: up to four infusion bags of transduced CD34+ cells in cryopreservation solution labeled for the specific recipient

III. References

1. Zynteglo [prescribing information]. Somerville, MA: bluebird bio, Inc.; August 2022.
2. Cappellini MD, Farmakis D, Porter J, Taher A, eds. 2021 Guidelines for the Management of Transfusion Dependent Thalassaemia (TDT). 4th ed. Thalassaemia International Federation (TIF). Available at: https://thalassaemia.org.cy/. Accessed March 2024.
3. Connor RF, Fosmarin AG, Tirnauer JS. What's new in hematology. UpToDate [internet database]. Waltham, MA: UpToDate Inc. Updated February 29, 2024. Accessed March 18, 2024.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J3590	Unclassified biologics
C9399	Unclassified drugs or biologicals

Reviews, Revisions, and Approvals	Date
Policy created	10/2022
3Q 2023 annual review: no significant changes; added additional TDT genotype examples to appendix E (β^+/β^+ and β^0 /β^+ IVS1-110); references reviewed and updated.	07/2023
Updated to match DHS criteria, effective 07/15/2024.	07/2024