

PA DEPARTMENT OF HUMAN SERVICES
MAAC BRIEFING DOCUMENT
ZYNTGLO (BETIBEGLOGENE AUTOTEMCEL)

Proposed Effective Date: July 15, 2024

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 beneficiary:

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 a qualified 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 beneficiary does not meet the clinical review guidelines listed above but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary, 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 Zynteglo (betibeglogene autotemcel). If the guidelines in Section B. are met, the reviewer will

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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 beneficiary.

D. Dose and Duration of Therapy

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

E. 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.