RX.PA.071.MPC Zynteglo (betibeglogene autotemcel)

The purpose of this policy is to define the prior authorization process for Zynteglo® (betibeglogene autotemcel)

Zynteglo® (betibeglogene autotemcel) is an autologous hematopoietic stem cell-based gene therapy indicated for the treatment of adult and pediatric patients with β-thalassemia who require regular red blood cell (RBC) transfusions.

Note: This review criteria is only applicable to the medication. All additional supportive services required for the administration of this medication will be reviewed separately.

PROCEDURE

A. Initial Authorization Criteria

1. Must meet ALL of the criteria listed below:

- Member is ≥ 4 years of age and < 65 years of age
- Prescribed by or in consultation with a hematologist or transplant specialist
- Diagnosis of β-thalassemia confirmed by:
  - HBB sequence gene analysis showing biallelic pathogenic variants
- Documentation confirming member does not have hemoglobin S/β-thalassemia or α-thalassemia
- Prescriber attests to the following:
  - Member is clinically stable and eligible to undergo hematopoietic stem cell transplant (HSCT)
  - Member has not received prior gene therapy or hematopoietic stem cell transplant
- Member must be transfusion dependent as evidenced by:
  - Transfusions of at least 100 mL/kg/year of packed red blood cells (pRBCs) in the last 2 years preceding therapy
    OR
  - 8 or more transfusions of pRBCs per year in the last 2 years preceding therapy
- Provider attestation and documentation of **ALL** of the following:
  - Iron chelation therapy will be discontinued at least 7 days prior to initiating myeloablative conditioning therapy
  - Anti-retroviral medications or hydroxyurea will be discontinued at least 30-days prior to mobilization and until all cycles of apheresis have been completed
  - For female members:
    a. Negative pregnancy test will be confirmed prior to starting mobilization, prior to conditioning procedures and before Zynteglo administration
- Documentation that the member has the following:

    - MRI scan of the liver (scan must be obtained within 180 days of starting the treatment request process)
    - Liver biopsy is required to rule out advanced liver disease if at least **ONE** of the following is true from the MRI scan:
        a. Iron content ≥ 15 mg/g. (Note: biopsy must be obtained within 180 days of starting treatment request process.)
        b. Cardiac T2* less than 10 msec via MRI. (Note: biopsy must be obtained within 180 days of starting treatment request process.)
- Documentation confirming that the member has ALL of the following (lab work must be obtained within 30 days of starting the treatment request process):
    - Negative HIV-1 or HIV-2 infection
    - No Hepatitis B or C infection
    - Negative Human T-lymphotropic virus Type (HTLV) 1 or 2
    - No advanced liver disease with absence of all:
        a. Cirrhosis
        b. Bridging fibrosis
        c. Active hepatitis
    - No prior or current malignancies

- Provider attests to that the member will be monitored for hematological malignancies
- Member does not have a white blood cell count < 3 X 10^9/L and/or a platelet count < 100 X10^9/L (obtained within 30 days of starting the treatment process)
- Member does not have a history of an uncorrected bleeding disorder

B. Must be prescribed at a dose within the manufacturer's dosing guidelines (based on diagnosis, weight, etc) listed in the FDA approved labeling.

C. Zynteglo will be considered investigational or experimental for any other use and will not be covered.

D. <u>Reauthorization Criteria:</u>

Zynteglo is not eligible for reauthorization.

<u>Limitations:</u>

Length of Authorization (if above criteria met)	
Initial Authorization	1 month
Reauthorization	N/A

Codes:

Code	Description
J3590	Injection, Unclassified biologics

REFERENCES

1. Zynteglo [package insert]. Somerville, MA: bluebird bio, Inc.; August 2022.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Update to MRI scan/biopsy requirements – must be obtained within 180 days of treatment request process	*01/2026*
Selected Review *Removal of GFR requirement*	*11/2024*
Annual Review	*02/2024*
Clarified 30-day lab requirement and updated prior authorization approval duration	*05/2023*
New Policy	*12/2022*