

Clinical Policy: Enzyme Replacements, Gaucher Disease

Reference Number: PHW.PDL.517

Effective Date: 01/01/2020

Last Review Date: 11/2025

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 Gaucher Disease Enzyme Replacements are **medically necessary** when the following criteria are met:

I. Requirements for Prior Authorization of Enzyme Replacements, Gaucher Disease

A. Prescriptions That Require Prior Authorization

All prescriptions for Enzyme Replacements, Gaucher Disease agents must be prior authorized.

B. Review of Documentation for Medical Necessity

In evaluating a request for prior authorization of a prescription for an Enzyme Replacement, Gaucher Disease agent, the determination of whether the requested prescription is medically necessary will take into account whether the member:

1. Is prescribed the Enzyme Replacements, Gaucher Disease agent for the treatment of a diagnosis that is indicated in the U.S. Food and Drug Administration (FDA)-approved package labeling OR a medically accepted indication; **AND**
2. Is age-appropriate according to FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**
3. Is prescribed a dose that is consistent with FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**
4. Does not have a history of a contraindication to the prescribed medication; **AND**
5. Is prescribed the Enzyme Replacements, Gaucher Disease agent by or in consultation with a specialist in the treatment of Gaucher disease; **AND**
6. For a non-preferred Enzyme Replacements, Gaucher Disease agent, has a history of therapeutic failure, contraindication, or intolerance of the preferred Enzyme Replacements, Gaucher Disease agents approved or medically

accepted for the member's indication; **AND**

7. For a diagnosis of Gaucher disease, has documentation of **both** of the following:
 - a. **One** of the following:
 - i. Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity
 - ii. DNA testing confirming the diagnosis
 - b. **One** of the following:
 - i. Anemia
 - ii. Bone disease
 - iii. Hepatomegaly
 - iv. Interstitial lung disease
 - v. Splenomegaly
 - vi. Thrombocytopenia

AND

8. If a prescription for an Enzyme Replacement, Gaucher Disease agent is in a quantity that exceeds the quantity limit, the determination of whether the prescription is medically necessary will also take into account the guidelines set forth in PA.CP.PMN.59 Quantity Limit Override.

NOTE: If the member does not meet the clinical review guidelines listed 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.

FOR RENEWALS OF PRIOR AUTHORIZATION FOR ENZYME REPLACEMENTS, GAUCHER DISEASE AGENTS:

The determination of medical necessity of a request for renewal of a prior authorization for an Enzyme Replacements, Gaucher Disease agent that was previously approved will take into account whether the member:

1. Is prescribed a dose that is consistent with FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**
2. Is prescribed the Enzyme Replacements, Gaucher Disease agent by or in consultation with a specialist in the treatment of Gaucher disease; **AND**
3. Has documentation of improvement in disease severity since initiating treatment with the requested Enzyme Replacements, Gaucher Disease agent; **AND**
4. If a prescription for an Enzyme Replacements, Gaucher Disease agent is in a

quantity that exceeds the quantity limit, the determination of whether the prescription is medically necessary will also take into account the guidelines set forth in PA.CP.PMN.59 Quantity Limit Override.

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 an Enzyme Replacements, Gaucher Disease agent. 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. Approval Duration:

- **New Request: 6 months**
- **Renewal Request: 12 months**

E. References

1. Wang RY, Bodamer OA, et al. ACMG Standards and Guidelines. Lysosomal storage diseases: Diagnostic confirmation and management of presymptomatic individuals. *Genetics in Medicine*;13, (5), May 2011.
2. Hughes D, Sidransky E. Gaucher disease: Pathogenesis, clinical manifestations, and diagnosis. In: UpToDate [internet database]. Hahn SH, TePas E, eds. Waltham, MA: UpToDate. Updated May 21, 2019. Accessed July 29, 2019.
3. Hughes D, Sidransky E. Gaucher disease: Treatment. In: UpToDate [internet database]. Hahn SH, TePas E, eds. Waltham, MA: UpToDate. Updated April 10, 2018. Accessed July 29, 2019

Reviews, Revisions, and Approvals	Date
Policy created	01/01/2020
Q3 2020 annual review: no changes.	07/2020
Q1 2021 annual review: no changes.	01/2021
Q1 2022 annual review: no changes.	11/2021
Q1 2023 annual review: no changes.	11/2022
Q1 2024 annual review: no changes.	11/2023
Q1 2025 annual review: no changes.	11/2024
Q1 2026 annual review: no changes.	11/2025