Drug Coverage Policy

Effective Date 5/1/2026
Coverage Policy Number.....IP0441
Policy Title: ..... Cerdelga

Gaucher Disease – Substrate Reduction Therapy – Cerdelga

- Cerdelga® (eliglustat capsules - Genzyme)

INSTRUCTIONS FOR USE

The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Each coverage request should be reviewed on its own merits. Medical directors are expected to exercise clinical judgment where appropriate and have discretion in making individual coverage determinations. Where coverage for care or services does not depend on specific circumstances, reimbursement will only be provided if a requested service(s) is submitted in accordance with the relevant criteria outlined in the applicable Coverage Policy, including covered diagnosis and/or procedure code(s). Reimbursement is not allowed for services when billed for conditions or diagnoses that are not covered under this Coverage Policy (see "Coding Information" below). When billing, providers must use the most appropriate codes as of the effective date of the submission. Claims submitted for services that are not accompanied by covered code(s) under the applicable Coverage Policy will be denied as not covered. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

OVERVIEW

Cerdelga, a glucosylceramide synthase inhibitor, is indicated for the long-term treatment of **Gaucher disease type 1**, in adults who are cytochrome P450 2D6 extensive metabolizers, intermediate metabolizers, or poor metabolizers as detected by an FDA-cleared test.[1]

Disease Overview

Gaucher disease is caused by a deficiency in the lysosomal enzyme beta-glucocerebrosidase.[2] This enzyme is responsible for the breakdown of glucosylceramide into glucose and ceramide. In Gaucher disease, deficiency of the enzyme beta-glucocerebrosidase results in the accumulation of glucosylceramide substrate in the lysosomal compartment of macrophages, giving rise to foam cells or "Gaucher cells." Cerdelga is a specific inhibitor of the enzyme glycosylceramide synthase, which is responsible for producing the substrate glucosylceramide; hence Cerdelga functions as a substrate reduction therapy.

Coverage Policy

POLICY STATEMENT

Prior Authorization is required for prescription benefit coverage of Cerdelga. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Cerdelga as well as the monitoring required for adverse events and long-term efficacy, approval requires Cerdelga to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Documentation: Documentation is required where noted in the criteria as **[documentation required]**. Documentation may include, but is not limited to, chart notes, laboratory tests, claims records, prescription receipts and/or other information. All documentation must include patient-specific identifying information.

Cerdelga® is considered medically necessary when the following are met:

1. **Gaucher Disease Type 1.** Approve for 1 year if the patient meets **ALL** of the following criteria (A, B, and C):
    A. Patient is a cytochrome P450 2D6 extensive metabolizer, intermediate metabolizer, or poor metabolizer as detected by an approved test; AND
    B. The diagnosis is established by ONE of the following (i or ii):
        i. Demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts **[documentation required]**; OR
        ii. Molecular genetic test documenting biallelic pathogenic glucocerebrosidase *(GBA)* gene variants **[documentation required]**; AND
    C. The medication is prescribed by, or in consultation with a geneticist, endocrinologist, metabolic disorder subspecialist, or a physician who specializes in the treatment of Gaucher disease or related disorders.

Conditions Not Covered

Eliglustat for any other use is considered not medically necessary, including the following (this list may not be all inclusive; criteria will be updated as new published data are available):

1. **Concomitant Use with Other Approved Therapies for Gaucher Disease.** Concomitant use with other treatments approved for Gaucher disease has not been evaluated. Of note, examples of medications approved for Gaucher disease include Cerezyme (imiglucerase intravenous infusion), Elelyso (taliglucerase alfa intravenous infusion), Vpriv (velaglucerase alfa intravenous infusion), and Zavesca (miglustat capsules).

References

1. Cerdelga® capsules [prescribing information]. Waterford, Ireland: Genzyme; January 2024.
2. Stirnemann J, Belmatoug N, Camou F, et al. A review of Gaucher disease pathophysiology, clinical presentation and treatments. *Int J Mol Sci*. 2017;18:441.

Revision Details

Type of Revision	Summary of Changes	Date
Selected Revision	**Gaucher Disease Type 1:** **Removed** criterion "Individual is age 18 years or older" **Updated** criterion **from** "deficiency of glucosylceramidase [also known as acid β-glucosidase or glucocerebrosidase] in peripheral blood leukocytes or other nucleated cells" **to** "demonstration of deficient beta-glucocerebrosidase activity in leukocytes or fibroblasts." **Updated** criterion **from** "Confirmation of biallelic pathogenic variants in the GBA gene" **to** "Confirmation of molecular genetic test documenting biallelic pathogenic glucocerebrosidase (GBA) gene variants." **Updated** criterion **from** "Individual is ONE of the following: CYP2D6 extensive metabolizer (EM), CYP2D6 intermediate metabolizer (IM) or CYP2D6 poor metabolizer (PM)" **to** "Individual is ONE of the following as detected by an approved test: CYP2D6 extensive metabolizer (EM), CYP2D6 intermediate metabolizer (IM) or CYP2D6 poor metabolizer (PM)" **Reauthorization Criteria:** **Updated** criterion **from** "Eliglustat (Cerdelga) is considered medically necessary for continued use when initial criteria are met AND there is documentation of beneficial response" **to** "Continuation of Eliglustat (Cerdelga) is considered medically necessary for Gaucher Disease Type 1 when the above medical necessity criteria are met AND there is documentation of beneficial response." **Conditions Not Covered:** Concomitant use with other approved therapies for Gaucher disease was added.	12/1/2024
Annual Revision	No criteria changes	07/15/2025
Early Annual Revision	**Policy Title:** **Updated** from "Eliglustat" to "Gaucher Disease – Substrate Reduction Therapy – Cerdelga". **Gaucher Disease Type 1:** **Clarified** medical necessity criteria for eliglustat (Cerdelga) in Gaucher disease type 1. **Added** an explicit approval duration of 1 year when all criteria are met. **Updated** diagnostic criteria to clarify that Gaucher disease must be established by one qualifying method (enzyme activity testing *or* molecular	5/1/2026

	genetic testing), with documentation requirements specified. **Updated** CYP2D6 metabolizer requirements by simplifying and clarifying eligibility language and removing redundant subtype listings. Editorial updates made to improve clarity, consistency, and alignment with current prescribing and documentation standards; no change to overall intent of coverage.	

The policy effective date is in force until updated or retired.