

PHARMACY POLICY STATEMENT

Marketplace

DRUG NAME	Cerdelga (eliglustat)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Cerdelga is a substrate reduction therapy, FDA approved in 2014 for the treatment of Gaucher disease type 1. Gaucher disease is a rare, inherited, lysosomal storage disorder. In Gaucher disease, mutations of the GBA gene cause deficiency of the enzyme glucocerebrosidase (acid beta-glucosidase), resulting in the accumulation of glucocerebroside (glucosylceramide [GLC]) in the lysosomes of macrophages to form “Gaucher cells,” especially in the bone marrow, spleen, and liver. Prominent symptoms include hepatosplenomegaly, anemia, thrombocytopenia, and skeletal problems. Type 1 Gaucher disease is the most common form and does not affect the central nervous system. In contrast to standard of care enzyme replacement therapy (ERT), Cerdelga reduces synthesis of the accumulating substrate to compensate for its impaired degradation.

Cerdelga (eliglustat) will be considered for coverage when the following criteria are met:

Gaucher disease type 1 (GD1)

For **initial** authorization:

1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a geneticist, hematologist, or metabolic specialist; AND
3. Member has a diagnosis of Gaucher disease type 1 confirmed by documentation of at least one of the following:
 - a) Deficiency of glucocerebrosidase activity by enzyme assay (0 to 15% of normal), and/or
 - b) Molecular genetic test documenting 2 mutations (biallelic variants) of the GBA gene; AND
4. CYP2D6 genotype analysis shows the member is an extensive, intermediate, or poor metabolizer; AND
5. Member exhibits at least one of the following as a result of Gaucher disease:
 - a) Anemia,
 - b) Thrombocytopenia,
 - c) Spleen and/or liver enlargement; AND
6. Member does NOT have any of the following:
 - a) Neurologic symptoms suggestive of type II or III Gaucher disease (i.e., supranuclear gaze palsy, cognitive decline, epilepsy, myoclonus and/or ataxia),
 - b) CYP2D6 ultra-rapid or indeterminate metabolizer status,
 - c) Pre-existing cardiac disease, long QT syndrome, or in combination with Class IA (e.g., quinidine, procainamide) or Class III (e.g., amiodarone, sotalol) antiarrhythmic medications,
 - d) Concomitant use with Zavesca (miglustat) or ERT.
7. **Dosage allowed/Quantity limit:**
Recommended dosing is based on CYP2D6 metabolizer status:

CYP2D6 Metabolizer Status	CERDELGA Dosage
EMs	84 mg twice daily
IMs	
PMs	84 mg once daily

NOTE: Dose adjustments may be needed based on other medications the member is taking. Consult the complete prescribing information from the manufacturer.

QL: 56 capsules per 28 days.

If all the above requirements are met, the medication will be approved for 12 months.

For **reauthorization**:

- Chart notes must document improvement in one or more of the following parameters compared to baseline:
 - Hemoglobin level
 - Platelet count
 - Spleen and/or liver volumes

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Cerdelga (eliglustat) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
08/04/2021	New policy for Cerdelga created.
05/11/2023	Added references. Clarified that 2 mutations of the GBA gene should be present. Removed bone outcomes from reauth section; was not measured in clinical trials and unlikely to be evident at 12 mo.
04/29/2025	Updated references.

References:

- Cerdelga [package insert]. Waterford, Ireland: Genzyme Ireland, Ltd.; 2024.
- Mistry PK, Lukina E, Ben Turkia H, et al. Effect of oral eliglustat on splenomegaly in patients with Gaucher disease type 1: the ENGAGE randomized clinical trial. *JAMA*. 2015;313(7):695-706. doi:10.1001/jama.2015.459
- Lukina E, Watman N, Arreguin EA, et al. A phase 2 study of eliglustat tartrate (Genz-112638), an oral substrate reduction therapy for Gaucher disease type 1. *Blood*. 2010;116(6):893-899. doi:10.1182/blood-2010-03-273151
- Lukina E, Watman N, Dragosky M, et al. Outcomes after 8 years of eliglustat therapy for Gaucher disease type 1: Final results from the Phase 2 trial. *Am J Hematol*. 2019;94(1):29-38. doi:10.1002/ajh.25300
- Cox TM, Drelichman G, Cravo R, et al. Eliglustat maintains long-term clinical stability in patients with Gaucher disease type 1 stabilized on enzyme therapy. *Blood*. 2017;129(17):2375-2383. doi:10.1182/blood-2016-12-758409
- Belmatoug N, Di Rocco M, Fraga C, et al. Management and monitoring recommendations for the use of eliglustat in adults with type 1 Gaucher disease in Europe. *Eur J Intern Med*. 2017;37:25-32. doi:10.1016/j.ejim.2016.07.011
- Balwani M, Burrow TA, Charrow J, et al. Recommendations for the use of eliglustat in the treatment of adults with Gaucher disease type 1 in the United States. *Mol Genet Metab*. 2016;117(2):95-103. doi:10.1016/j.ymgme.2015.09.002
- Dardis A, Michelakakis H, Rozenfeld P, et al. Patient centered guidelines for the laboratory diagnosis of Gaucher disease type 1. *Orphanet J Rare Dis*. 2022;17(1):442. Published 2022 Dec 21. doi:10.1186/s13023-022-02573-6
- Torralba-Cabeza MÁ, Morado-Arias M, Pijierro-Amador A, Fernández-Canal MC, Villarrubia-Espinosa J. Recommendations for oral treatment for adult patients with type 1 Gaucher disease [published online ahead of print, 2022 Jun 5]. *Rev Clin Esp (Barc)*. 2022;S2254-8874(22)00043-1. doi:10.1016/j.rceng.2022.02.008

10. Stone WL, Basit H, Mukkamalla SKR, et al. Gaucher Disease. [Updated 2023 Nov 12]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2025 Jan-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK448080/>

Effective date: 10/01/2025

Revised date: 04/29/2025