

Clinical Policy: Eliglustat (Cerdelga)
Reference Number: HIM.PA.SP9
Effective Date: 05/17
Last Review Date:
Line of Business: Health Insurance Marketplace

[Coding Implications](#)
[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

Eliglustat (Cerdelga®) is a glucosylceramide synthase inhibitor.

FDA approved indication

Cerdelga is indicated for long-term treatment of adult patients with Gaucher disease type 1 who are CYP2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test.

Limitation of use:

- CYP2D6 ultra-rapid metabolizers may not achieve adequate concentrations of Cerdelga to achieve a therapeutic effect.
- A specific dosage cannot be recommended for CYP2D6 indeterminate metabolizers.

Policy/Criteria

Provider must submit documentation (including office chart notes and lab results) supporting that member has met all approval criteria

I. Initial Approval Criteria

A. Type 1 Gaucher Disease (must meet all):

1. Diagnosis of type 1 Gaucher disease confirmed by one of the following (a or b):
 - a. Enzyme assay demonstrating a deficiency of β -glucocerebrosidase activity;
 - b. DNA testing;
2. Member has CYP2D6 genotype EM, IM, or PM as detected by an FDA-cleared test;
3. Dose does not exceed:
 - a. CYP2D6 EMs and IMs: 168 mg per day (2 capsules per day);
 - b. CYP2D6 PMs: 84 mg per day (1 capsule per day).

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to HIM.PHAR.21 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

A. Type 1 Gaucher Disease (must meet all):

1. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
2. Documentation of positive response to therapy (e.g., stabilized/improved spleen volume, liver volume hemoglobin level, or platelet count);

3. If request is for a dose increase, new dose does not exceed:
 - a. CYP2D6 EMs and IMs: 168 mg per day (2 capsules per day);
 - b. CYP2D6 PMs: 84 mg per day (1 capsule per day).

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy; or
2. Refer to HIM.PHAR.21 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

Approval duration: 12 months

III. Diagnoses/Indications for which coverage is NOT authorized:

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policy – HIM.PHAR.21 or evidence of coverage documents

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

EM: extensive metabolizer

FDA: Food and Drug Administration

IM: intermediate metabolizer

PM: poor metabolizer

V. References

1. Cerdelga Prescribing Information. Waterford, Ireland: Genzyme Ireland, Ltd.; August 2014. Available at <http://www.cerdelga.com>. Accessed January 19, 2017.
2. Charrow J, Andersson HC, Kaplan P. Enzyme replacement therapy and monitoring for children with Type 1 Gaucher disease: consensus recommendations. *J Pediatr.* 2004; 144: 112-20.
3. Hollak CEM, Weinreb NJ. The attenuated/late onset lysosomal storage disorders: therapeutic goals and indications for enzyme replacement treatment in Gaucher and Fabry disease. *Best Pract Res Clin Endocrinol Metab.* 2015; 29: 205-218.
4. 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. *Molecular Genetics and Metabolism.* 2016; 117(2): 95-103.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created.	01/17	05/17

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted

standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note: For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence.

Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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