Clinical Policy: Eliglustat (Cerdelga)

Reference Number: CP.PHAR.153
Effective Date: 02/16
Last Review Date: 02/17 Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description
The intent of the criteria is to ensure that patients follow selection elements established by Centene® clinical policy for eliglustat (Cerdelga®).

Policy/Criteria
It is the policy of health plans affiliated with Centene Corporation® that Cerdelga is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

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

1. Diagnosis of Type 1 Gaucher disease (GD1) confirmed by one of the following:
   a. Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity;
   b. DNA testing;
2. Positive for one of the following CYP2D6 genotypes as detected by an FDA-cleared test:
   a. Extensive metabolizer (EM);
   b. Intermediate metabolizer (IM);
   c. Poor metabolizer (PM);
3. Cerdelga is prescribed as monotherapy;
4. Prescribed dose of Cerdelga does not exceed a total daily dose of 168 mg;
5. Member does not have any of the following medical conditions:
   a. Pre-existing cardiac disease (e.g., congestive heart failure, recent acute myocardial infarction, bradycardia, heart block, ventricular arrhythmia) or long QT syndrome;
   b. Moderate to severe renal impairment, or end-stage renal disease;
   c. All stages of hepatic impairment or cirrhosis;
6. Member has none of the following contraindications:
   a. If positive for the CYP2D6 EM or IM genotype, concurrent use of a strong (e.g., paroxetine) or moderate (e.g., terbinafine) CYP2D6 inhibitor and a strong (e.g., ketoconazole) or moderate (e.g., fluconazole) CYP3A inhibitor;
   b. If positive for the CYP2D6 IM or PM genotype, concurrent use of a strong CYP3A (e.g., ketoconazole) inhibitor.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Type 1 Gaucher Disease (must meet all):
1. Currently receiving medication via Centene benefit or member has previously met all initial approval criteria;
2. Member is responding positively to therapy.

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 CP.PHAR.57 - Global Biopharm Policy**.**

Background

Description/Mechanism of Action:

Gaucher disease is caused by a deficiency of the lysosomal enzyme acid beta-glucosidase. Acid beta-glucosidase catalyzes the conversion of the sphingolipid glucocerebroside into glucose and ceramide. The enzymatic deficiency causes an accumulation of glucosylceramide (GL-1) primarily in the lysosomal compartment of macrophages, giving rise to foam cells or "Gaucher cells". Cerdelga is a specific inhibitor of glucosylceramide synthase, and acts as a substrate reduction therapy for GD1. In clinical trials Cerdelga reduced spleen and liver size, and improved anemia and thrombocytopenia. In this lysosomal storage disorder (LSD), clinical features are reflective of the accumulation of Gaucher cells in the liver, spleen, bone marrow, and other organs. The accumulation of Gaucher cells in the liver, spleen, and bone marrow leads to organomegaly and skeletal disease. Presence of Gaucher cells in the bone marrow and spleen lead to clinically significant anemia and thrombocytopenia.

Formulations:

Cerdelga (eliglustat): Capsules for oral use
- 84 mg/capsule

FDA Approved Indications:

Cerdelga is a glucosylceramide synthase inhibitor/oral capsule formulation indicated for:
- Long-term treatment of adult patients with GD1 who are CYP2D6 EMs, IMs, or PMs as detected by an FDA-cleared test.

  Limitations of use:
  - Patients who are CYP2D6 ultra-rapid metabolizers may not achieve adequate concentrations of Cerdelga to achieve a therapeutic effect;
  - A specific dosage cannot be recommended for those patients whose CYP2D6 genotype cannot be determined (IMs).

Appendices

Appendix A: Abbreviation Key

EM: Extensive metabolizer
ERT: Enzyme replacement therapy
GD1: Type 1 Gaucher disease
IM: Intermediate metabolizer
PM: Poor metabolizer

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
N/A	

Reviews, Revisions, and Approvals	Date	Approval Date
Policy split from CP.PHAR.48. Policy converted to new template.	01/16	02/16
Age restriction removed. Max dose added. Monotherapy requirement added. Conditions for which Cerdelga is not recommended are added to initial criteria per the PI. Initial approval extended to 6 months for consistency across similar policies. Positive response to therapy added. Background section converted to new template.	12/16	02/17

References

1. Cerdelga Prescribing Information. Waterford, Ireland: Genzyme Ireland, Ltd.; August 2014. Available at http://www.cerdelga.com. Accessed December 14, 2016.
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.

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.

Note: For Medicare members, to ensure consistency with the Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), all applicable NCDs, LCDs, and Medicare Coverage Articles should be reviewed prior to applying the criteria set forth in this clinical policy. Refer to the CMS website at http://www.cms.gov for additional information.