Clinical Policy: Eliglustat (Cerdelga)

Reference Number: USS.SPMN.137
Effective Date: 10/16
Last Review Date: 09/16

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

Policy/Criteria
It is the policy of health plans affiliated with Envolve Pharmacy Solutions® that eliglustat (Cerdelga®) is medically necessary when the following criteria are met:

I. Initial Approval Criteria
   A. Type 1 Gaucher Disease (GD1) (must meet all):
      1. Age ≥ 18 years;
      2. Diagnosis of GD1 confirmed by one of the following:
         a. Enzyme assay demonstrating a deficiency of β-glucocerebrosidase activity;
         or
         b. Diagnosis confirmed by DNA testing;
      3. Genotype CYP2D6 EM, IM, or PM as detected by an FDA-cleared test.

      Approval duration: 6 months

   B. Other diagnoses/indications: Refer to ERX.SPMN.16 - Global Biopharm Policy.

II. Continued Approval
   A. Type 1 Gaucher Disease (GD1) (must meet all):
      1. Currently receiving medication via health plan benefit or member has previously met all initial approval criteria.

      Approval duration: 12 months

   B. Other diagnoses/indications (must meet 1 or 2):
      1. Currently receiving medication via health plan benefit and documentation supports positive response to therapy; or
      2. Refer to ERX.SPMN.16 - Global Biopharm Policy.
Background

**Description/Mechanism of Action:**
Eliglustat capsules contain eliglustat tartrate, which is a small molecule inhibitor of glucosylceramide synthase that resembles the ceramide substrate for the enzyme. Gacher disease is an autosomal recessive glycosphingolipid storage disorder caused by deficient lysosomal β-glucocerebrosidase. Acid β-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.

**FDA Approved Indication(s):**
Cerdelga is an enzyme inhibitor/oral capsule indicated for:
- Long-term treatment of adult patients with Gaucher disease type 1, CYP2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test.

Limitations 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.

Appendices

**Appendix A: Abbreviation Key**
EMs: extensive metabolizers
ERT: enzyme replacement therapy
GD1: type 1 Gaucher disease
IMs: intermediate metabolizers
PMs: poor metabolizers

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<th>Reviews, Revisions, and Approvals</th>
<th>Date</th>
<th>Approval Date</th>
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<tbody>
<tr>
<td>Policy split from USS.SPMN.33 Lysosomal Storage Disorders and converted to new template. Removed all safety criteria. Expanded diagnosis criteria to include option for DNA testing. Modified approval duration to 6 months for initial and 12 months for re-auth.</td>
<td>08/16</td>
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References


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. This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. 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.

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