Pharmacy Medical Necessity Guidelines: Cerdelga™ (eliglustat)

Effective: June 1, 2017

Prior Authorization Required ✓ Type of Review – Care Management
Not Covered ✓ Type of Review – Clinical Review
Pharmacy (RX) or Medical (MED) Benefit RX Department to Review RXUM

Fax Numbers:
RXUM: 617.673.0988

This Pharmacy Medical Necessity Guideline applies to the following:

**Tufts Health Plan Commercial Plans**
- Tufts Health Plan Commercial Plans – large group plans
- Tufts Health Plan Commercial Plans – small group and individual plans

**Tufts Health Public Plans**
- Tufts Health Direct – Health Connector
- Tufts Health Together – A MassHealth Plan
- Tufts Health RITogether – A Rite Care + Rhody Health Partners Plan

**Tufts Health Freedom Plan products**
- Tufts Health Freedom Plan - large group plans
- Tufts Health Freedom Plan - small group plans

Note: For Tufts Health Plan Medicare Preferred Members, please refer to the Tufts Health Plan Medicare Preferred Prior Authorization Criteria. Background, applicable product and disclaimer information can be found on the last page.

**OVERVIEW**

Gaucher disease, the most common lysosomal storage disorder, is an autosomal recessive disease. Type 1 Gaucher disease (nonneuropathic) is characterized by a functional deficiency of the enzyme glucocerebrosidase. Glucocerebrosidase catalyzes the conversion of the glycosphingolipid glucocerebroside into glucose and ceramide as part of the normal degradation pathway for membrane lipids. A deficiency in glucocerebrosidase results in lipidosis characterized by accumulation of insoluble glucocerebroside (otherwise known as glycosylceramide) in various tissues with resultant pathology, such as hepatosplenomegaly, anemia, thrombocytopenia, osteoporosis, and bone pain.

Enzyme replacement therapy (ERT) with human β-glucocerebrosidase catalyzes the hydrolysis of glucocerebroside. ERT has been shown to reduce organomegaly and improve hematologic and biochemical parameters in patients with type 1 Gaucher disease. ERT is administered via intravenous infusion.

Cerdelga (eliglustat) inhibits glucosylceramide synthase, the initial enzyme in a series of reactions that result in the synthesis of most glycosphingolipids, including glucocerebroside. Unlike ERT, which increases the degradation of insoluble glycosphingolipids, Cerdelga (eliglustat) decreases the rate of glycosphingolipid biosynthesis.

Cerdelga (eliglustat) is available as a capsule for oral administration and is Food and Drug Administration (FDA)-approved for long-term treatment of type 1 Gaucher disease in certain adult patients. Treatment with Cerdelga (eliglustat) has been shown to significantly reduce spleen and liver size and significantly improve hemoglobin and platelet levels.

Cerdelga (eliglustat) is the second oral agent to be FDA-approved for the treatment of type 1 Gaucher disease in adults. Zavesca (miglustat) is also an oral glucosylceramide synthase inhibitor approved for use in patients with type 1 Gaucher disease who are not candidates for ERT.

**FDA-APPROVED INDICATIONS**

Cerdelga (eliglustat) is indicated for the long-term treatment of adult patients with Gaucher disease type 1 (GD1) who are cytochrome P450 (CYP) 2D6 extensive metabolizers (EMs), intermediate metabolizers (IMs), or poor metabolizers (PMs) as detected by an FDA-cleared test.

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

The plan may authorize coverage of Cerdelga (eliglustat) for Members when all the following criteria are met and limitations do not apply:

1. Documented diagnosis of Gaucher disease type 1 (GD1) AND
2. Member is over 18 years of age AND
3. Documentation the Member is a cytochrome P450 2D6 extensive metabolizer (EMs), intermediate metabolizer (IMs), or poor metabolizer (PMs) as detected by an FDA-cleared test

LIMITATIONS

1. The coverage of Cerdelga (eliglustat) will be limited as follows:
   a. For Extensive (EMs) and Intermediate metabolizers (IMs) – 60 capsules per 30 days
   b. For Poor metabolizers (PMs) – 30 capsules per 30 days

CODES

None

REFERENCES

5. Cox TM, Drellichman G, Cravo R et al. ENCORE: A multi-national, randomized, controlled, open-label, non-inferiority study comparing eliglustat with imiglucerase in Gaucher disease type 1 patients on enzyme replacement therapy who have reached therapeutic goals. Poster presented at Lysosomal Disease Network World Symposium. San Diego, CA; 2014 February 12.

**APPROVAL HISTORY**
April 14, 2015: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:
- January 1, 2016: Administrative change to rebranded template.
- February 9, 2016: Approval duration modified to life of plan.
- February 14, 2017: No changes. Effective 2/14/17, Medical Necessity Guideline applies to Tufts Health Together.

**BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION**
Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. They are used in conjunction with a Member’s benefit document and in coordination with the Member’s physician(s). The plan makes coverage decisions on a case-by-case basis considering the individual Member’s health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

This Pharmacy Medical Necessity Guideline does not apply to Uniformed Services Family Health Plan Members or to certain delegated service arrangements. Unless otherwise noted in the Member’s benefit document or applicable Pharmacy Medical Necessity Guideline, Pharmacy Medical Necessity Guidelines do not apply to CareLinkSM Members. For self-insured plans, drug coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a coverage guideline and a self-insured Member’s benefit document, the provisions of the benefit document will govern. Applicable state or federal mandates will take precedence.

For Tufts Health Plan Medicare Preferred, please refer to Tufts Health Plan Medicare Preferred Prior Authorization Criteria.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to Member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.