Pharmacy Medical Necessity Guidelines: Zavesca® (miglustat)

Effective: February 14, 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

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

Fax Numbers:
RXUM: 617.673.0988

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 (nonneuronopathic) 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 (also 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.

Zavesca (miglustat) competitively and reversibly 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, Zavesca (miglustat) decreases the rate of glycosphingolipid biosynthesis. This results in a reduced amount of glycosphingolipid substrate and allows the residual activity of the deficient glucosylceramidase enzyme to be more effective (substrate reduction therapy).

Zavesca (miglustat) is available as a capsule for oral administration and is Food and Drug Administration (FDA)-approved for the management of type 1 Gaucher disease in adult patients for whom ERT is not a therapeutic option (e.g., secondary to allergy, hypersensitivity, or poor venous access). Treatment with Zavesca (miglustat) has been shown to significantly decrease liver and spleen size and improve hemoglobin and platelet levels.

Zavesca (miglustat) is not the only FDA-approved oral treatment of type 1 Gaucher disease. Cerdelga (eliglustat) is a second oral glucosylceramide synthase inhibitor approved for first-line treatment of adults with type 1 Gaucher disease.

FDA-APPROVED INDICATIONS
Zavesca (miglustat) is indicated as monotherapy for treatment of adult patients with mild/moderate type 1 Gaucher disease for whom ERT is not a therapeutic option.

COVERAGE GUIDELINES
The plan may authorize coverage of Zavesca (miglustat) for Members when all of the following criteria are met:

1. Documented diagnosis of type 1 Gaucher disease

AND
2. Member cannot be treated with enzyme replacement therapy (e.g., Cerezyme) **AND**

3. Member is over 18 years of age

**LIMITATIONS**
None

**CODES**
None

**REFERENCES**

**APPROVAL HISTORY**
August 2004: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:
- July 12, 2005: No changes
- June 13, 2006: No changes
- May 8, 2007: No changes
- May 13, 2008: No changes
- May 12, 2009: No changes
- January 1, 2010: Removal of Tufts Medicare Preferred language (separate criteria have been created specifically for Tufts Medicare Preferred).
- May 11, 2010: No changes
- May 10, 2011: No changes
- April 10, 2012: No changes
- March 12, 2013: No changes
- March 11, 2014: No changes
- March 10, 2015: No changes
- January 1, 2016: Administrative change to rebranded template.
• February 9, 2016: No changes
• February 14, 2017: No changes
• April 11, 2017: Administrative update, Adding Tufts Health RITogether to the template.

**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 CareLink℠ 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.