Pharmacy Medical Necessity Guidelines: Miglustat

Effective: January 18, 2021

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

These pharmacy medical necessity guidelines apply to the following:

Commercial Products

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Tufts Health Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

RXUM: 617.673.0988

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS

Miglustat is a glucosylceramide synthase inhibitor indicated as monotherapy for treatment of adult patients with mild/moderate type 1 Gaucher disease for whom enzyme replacement therapy 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. Documentation the Member cannot be treated with enzyme replacement therapy (e.g., Cerezyme)

AND

3. The Member is at least 18 years of age

LIMITATIONS

- The plan does not cover brand Zavesca. Refer to the Pharmacy Medical Necessity Guidelines for non-covered medications.

CODES

None

REFERENCES

1. Charrow J, Esplin JA, Gribble J, et al. Gaucher Disease: Recommendations on Diagnosis, Evaluation and Monitoring. *Arch Intern Med.* 1998;158(16):1754-60.
2. Cox T, Aerts J, Andria G, et al. The role of the iminosugar N-butyldeoxynojirimycin (miglustat) in the management of type I (non-neuronopathic) Gaucher disease: a position statement. *J Inherit Metab Dis*. 2003;26(6):513-26.
3. Cox TM, Amato D, Hollak CE, et al. Evaluation of miglustat as maintenance therapy after enzyme therapy in adults with stable type 1 Gaucher disease: a prospective, open-label non-inferiority study. *Orphanet J Rare Dis*. 2012 Dec 27;7:102.
4. Elstein D, Dweck A, Attias D, et al. Oral maintenance clinical trial with miglustat for type I Gaucher disease: switch from or combination with intravenous enzyme replacement. *Blood*. 2007 Oct 1;110(7):2296-301.
5. The Garrod Association. Ontario Guidelines for Treatment of Gaucher Disease by Enzyme Replacement with Imiglucerase or Velaglucerase, or Substrate Reduction Therapy with Miglustat. Version 9. August 2011. URL: garrod.ca/wp-content/uploads/ONTARIO-GUIDELINES-FOR-TREATMENT-OF-GAUCHER-August-2011-2.pdf. Available on Internet. Accessed 2015 January 18.
6. Martins AM, Valadares ER, Porta G, et al. Recommendations on Diagnosis, Treatment, and Monitoring for Gaucher Disease. *J Pediatr.* 2009 Oct;155(4 Suppl):S10-8.

7. National Gaucher Foundation. Gaucher Disease. URL: quacherdisease.org/. Available from Internet. Accessed 2015 January 18.
8. Wang RY, Bodamer OA, Watson MS, et al. Lysosomal storage diseases: Diagnostic confirmation and management of presymptomatic individuals. *Genetics in Medicine*. 2011 May; 13(5):464-6.
9. Weinreb NJ, Aggio MC, Andersson HC, et al. Gaucher disease type 1: revised recommendations on evaluations and monitoring for adult patients. *Semin Hematol*. 2004;41(suppl 5):15-22.
10. Zavesca (miglustat) [package insert]. South San Francisco, CA: Actelion Pharmaceuticals US, Inc.; November 2017.

APPROVAL HISTORY

August 2004: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:
1. July 12, 2005: No changes.
2. June 13, 2006: No changes.
3. May 8, 2007: No changes.
4. May 13, 2008: No changes.
5. May 12, 2009: No changes.
6. January 1, 2010: Removal of Tufts Medicare Preferred language (separate criteria have been created specifically for Tufts Medicare Preferred).
7. May 11, 2010: No changes.
8. May 10, 2011: No changes.
9. April 10, 2012: No changes.
10. March 12, 2013: No changes.
11. March 11, 2014: No changes.
12. March 10, 2015: No changes.
13. January 1, 2016: Administrative change to rebranded template.
14. February 9, 2016: No changes.
15. February 14, 2017: No changes.
16. April 11, 2017: Administrative update, Adding Tufts Health RITogether to the template.
17. February 13, 2018: No changes.
18. January 8, 2019: No changes.
19. January 14, 2020: No changes.
20. January 12, 2021: Administrative updates. Due to the generic availability of miglustat, updated the title of the Medical Necessity Guideline from "Zavesca (miglustat)" to "Miglustat" and added the following Limitation to be line with current coverage of multi-source brand Zavesca "The plan does not cover brand Zavesca. Refer to the Pharmacy Medical Necessity Guidelines for non-covered medications."

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. 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.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

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.

Provider Services