Clinical Policy Title:	miglustat
Policy Number:	RxA.570
Drug(s) Applied:	Zavesca®
Original Policy Date:	01/01/2020
Last Review Date:	10/19/2023
Line of Business Policy Applies to:	All lines of business (except Medicare)

Criteria

I. Initial Approval Criteria

A. Type 1 Gaucher Disease (must meet all)**:**

1. Diagnosis of mild to moderate GD1 confirmed by one of the following (a or b):
   a. Enzyme assay demonstrating a deficiency in beta-glucocerebrosidase (glucosidase) activity;
   b. DNA testing;
2. Age ≥ 18 years;
3. Member is having following disease manifestations (e.g., anemia, thrombocytopenia, bone disease, hepatomegaly, splenomegaly);
4. Failure of two enzyme replacement therapies (i.e., Cerezyme®, Elelyso®, Vpriv®), unless member is unable to take enzyme replacement therapies due to one of the following (a or b):
   a. Allergy or hypersensitivity;
   b. Poor venous access;
5. Zavesca® is prescribed as monotherapy;
6. Dose does not exceed 300 mg/day.

Approval Duration
Commercial: 12 months
Medicaid: 12 months

II. Continued Therapy Approval

A. Type 1 Gaucher Disease (must meet all)**:**

1. Member is currently receiving medication that has been authorized by RxAdvance or the member has met initial approval criteria listed in this policy;
2. Member is responding positively to therapy as evidenced by increased or stabilized platelet count or hemoglobin, reduced or stabilized spleen or liver volume, decreased bone pain, improvement in fatigue, constipation, and peripheral neuropathy;
3. Zavesca® is prescribed as monotherapy;
4. If request is for a dose increase, new dose does not exceed 300 mg/day.

Approval Duration
Commercial: 12 months
Medicaid: 12 months

References

1. 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. Available at: https://pubmed.ncbi.nlm.nih.gov/25987174/. Accessed July 27, 2022.
2. Pastores GM, Weinreb NJ, Aerts H, et al. Therapeutic goals in the treatment of Gaucher disease. Semin Hematol. 2004; 41(suppl 5): 4-14. Available at: https://pubmed.ncbi.nlm.nih.gov/15468045/. Accessed July 27, 2022.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	01/2020	01/01/2020
Policy was reviewed: 1. Policy title table was updated: Line of business policy applies was updated to All lines of business. 2. Continued therapy criteria II.A.1 was rephrased to "Currently receiving medication that has been authorized by RxAdvance…". 3. Updated verbiage from "symptomatic" to "disease manifestations" when describing improvement in clinical criteria. 4. Added symptoms to criteria in continued therapy criteria when describing disease improvement. 5. References were updated.	10/06/2020	12/07/2020
Policy was reviewed: 1. Continued therapy criteria II.A.1 was rephrased to "Member is currently receiving medication that has been authorized by RxAdvance…" 2. References were reviewed and updated.		
PA policy reviewed: 1. Initial Approval Criteria I.A.6 and Continued Therapy Criteria II.A.4: New dose does not exceed criteria updated from 600mg to 300 mg daily. 2. References were reviewed and updated.	07/27/2022	10/19/2022
Policy was reviewed.	10/19/2023	10/19/2023