Clinical Policy: Miglustat (Zavesca)

Reference Number: CP.PHAR.164
Effective Date: 02.01.16
Last Review Date: 11.26
Line of Business: Commercial, HIM/ICHRA, Medicaid

Coding Implications
Revision Log

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

Description
Miglustat (Zavesca®) is a glucosylceramide synthase inhibitor.

FDA Approved Indication(s)
Zavesca is indicated as monotherapy for the treatment of adult patients with mild/moderate type 1 Gaucher disease (GD1) for whom enzyme replacement therapy is not a therapeutic option.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of health plans affiliated with Centene Corporation® that Zavesca or miglustat is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

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

1. Diagnosis of 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 symptomatic (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. Miglustat is prescribed as monotherapy;
6. If request is for brand Zavesca, member must use generic miglustat, unless contraindicated or clinically significant adverse effects are experienced;
7. Dose does not exceed 300 mg (3 capsules) per day.

Approval duration: 12 months

B. Niemann-Pick Disease Type C (must meet all)**:**

1. Diagnosis of NPC confirmed by genetic analysis indicating biallelic pathogenic variants in either *NPC1* or *NPC2*;

2. Prescribed by or in consultation with a geneticist, neurologist, endocrinologist, or metabolic disease specialist;
3. Member presents with at least one neurological sign or symptom of the disease (*see Appendix D*);
4. If request is for brand Zavesca, member must use generic miglustat, unless contraindicated or clinically significant adverse effects are experienced;
5. Dose does not exceed 600 mg (6 capsules) per day.

Approval duration: 12 months

C. Other diagnoses/indications (must meet 1 or 2)**:**

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace/ICHRA) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace/ICHRA, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace/ICHRA) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace/ICHRA, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace/ICHRA, and CP.PMN.53 for Medicaid.

II. Continued Therapy

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

1. Member meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B);*
2. Member is responding positively to therapy as evidenced by increased or stabilized platelet count or hemoglobin, reduced or stabilized spleen or liver volume, or decreased bone pain;
3. Miglustat is prescribed as monotherapy;
4. If request is for brand Zavesca, member must use generic miglustat, unless contraindicated or clinically significant adverse effects are experienced;
5. If request is for a dose increase, new dose does not exceed 300 mg (3 capsules) per day.

Approval duration: 12 months

B. Niemann-Pick Disease Type C (must meet all)**:**
1. Member meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B);*
2. Member is responding positively to therapy as evidenced by an improvement or stabilization in a domain affected by NPC (e.g., ambulation, fine motor skills, swallowing, sitting, or speech);
3. If request is for brand Zavesca, member must use generic miglustat, unless contraindicated or clinically significant adverse effects are experienced;
4. If request is for a dose increase, new dose does not exceed 600 mg (6 capsules) per day.

Approval duration: 12 months

C. Other diagnoses/indications (must meet 1 or 2)**:**
1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace/ICHRA) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace/ICHRA, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace/ICHRA) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace/ICHRA, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace/ICHRA, and CP.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policy – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace/ICHRA, and CP.PMN.53 for Medicaid, or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
GD1: type 1 Gaucher disease
NPC: Niemann-Pick disease type C

Appendix B: Therapeutic Alternatives
This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
Cerezyme® (imiglucerase)	Individualize to each patient; initial dose ranges from 2.5 units/kg by IV infusion 3 times a week to 60 units/kg once every 2 weeks; disease severity may dictate treatment be initiated at relatively high dose or relatively frequent administration	Individualized
Elelyso® (taliglucerase alfa)	60 units/kg IV every other week	Individualized
VPRIV® (velaglucerase alfa)	60 units/kg IV every other week	Individualized

Therapeutic alternatives are listed as Brand name® (generic) when the drug is available by brand name only and generic (Brand name®) when the drug is available by both brand and generic.

Appendix C: Contraindications/Boxed Warnings
None reported

Appendix D: General Information
- GD1 is a heterogeneous disorder which involves the visceral organs, bone marrow, and bone in almost all affected patients. Common conditions resulting from GD1 include anemia, thrombocytopenia, hepatomegaly, splenomegaly, and bone disease. Therefore, hemoglobin level, platelet count, liver volume, spleen volume, and bone pain are clinical parameters that can indicate therapeutic response to GD1 therapies. In some clinical trials, stability has been defined as the following thresholds of change from baseline: hemoglobin level < 1.5 g/dL decrease, platelet count < 25% decrease, liver volume < 20% increase, and spleen volume < 25% increase.
- There is currently insufficient evidence that supports the combination use of enzyme replacement therapy with Zavesca.
- Examples of neurological signs or symptoms of NPC include hearing loss, vertical supranuclear gaze palsy, dysarthria, ataxia, dystonia, impaired ambulation, dysarthria, dysphagia, seizures, dementia

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
GD1	100 mg PO TID	300 mg/day

VI. Product Availability
Capsule: 100 mg

VII. References

1. Zavesca Prescribing Information. Irvine, CA: Actelion Pharmaceuticals US, Inc.; August 2022. Available at https://www.zavesca.com. Accessed January 14, 2026.
2. Charrow J, Andersson HC, Kaplan P. Enzyme replacement therapy and monitoring for children with type 1 Gaucher disease: consensus recommendations. J Pediatr. 2004;144:112-20.
3. 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.
4. Pastores GM, Weinreb NJ, Aerts H, et al. Therapeutic goals in the treatment of Gaucher disease. Semin Hematol. 2004;41(suppl 5):4-14.
5. Andersson HC, Charrow J, Kaplan P, et al. Individualization of long-term enzyme replacement therapy for Gaucher disease. Genet Med. 2005;7(2):105-110.
6. Gary SE, Ryan E, Steward AM, et al. Recent advances in the diagnosis and management of Gaucher disease. Expert Rev Endocrinol Metab. 2018 Mar;13(2):107–118.
7. Leonart LP, Fachi MM, Boger B, et al. A systematic review and meta-analyses of longitudinal studies on drug treatments for Gaucher disease. Ann Pharmacother. 2022;0(0). doi:10.1177/10600280221108443.
8. Mengel E, Patterson MC, Da Riol RM, et al. Efficacy and safety of arimoclomol in Niemann-Pick disease type C: Results from a double-blind, randomised, placebo-controlled, multinational phase 2/3 trial of a novel treatment. *J Inherit Metab Dis*. 2021;44(6):1463-1480. doi:10.1002/jimd.12428
9. Hiwot T, Porter FD, Bremova-Ertl T, et al. 2025 Consensus clinical management guidelines for Niemann-Pick disease type C. J Inherit Metab Dis. 2026;49(3):e70185. doi:10.1002/jimd.70185
10. Geberhiwot T, Moro Alessandro, Dardis A, et al. Consensus clinical management guidelines for Niemann-Pick disease type C. Orphanet Journal of Rare Diseases 2018 April 6;13(1):50.
11. Patterson MC, Clayton P, Gissen P, et al. Recommendations for the detection and diagnosis of Niemann-Pick disease type C: An update. Neurol Clin Pract. 2017;7(6):499-511.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1202	Miglustat, oral, 65 mg

Reviews, Revisions, and Approvals	Date	P&T Approval Date
2Q 2022 annual review: no significant changes; removed the requirement for mild to moderate GD1 severity for coverage based on	02.23.22	05.22

Reviews, Revisions, and Approvals	Date	P&T Approval Date
subjectivity of defining disease severity; references reviewed and updated.		
Template changes applied to other diagnoses/indications and continued therapy section.	09.30.22	
2Q 2023 annual review: no significant changes; references reviewed and updated.	02.07.23	05.23
2Q 2024 annual review: no significant changes; references reviewed and updated.	01.09.24	05.24
Added criteria for use in NPC.	11.26.24	02.25
2Q 2025 annual review: no significant changes; added generic redirection; added HCPCS code for miglustat; references reviewed and updated.	03.10.25	05.25
2Q 2026 annual review: no significant changes; updated initial approval duration from 6 months to 12 months; references reviewed and updated. Added ICHRA line of business.	04.10.26	05.26
Updated NPC diagnostic criterion to require confirmation by biallelic pathogenic variants in *NPC1* or *NPC2* gene per updated international consensus guidelines.	08.24.26	11.26

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. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. 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. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

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Note: For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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