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Specialty Guideline Management miglustat products

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Zavesca	miglustat
Yargesa	miglustat
Opfolda	miglustat

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications[1,2,5,6]

miglustat (generic)/Yargesa/Zavesca:

Indicated as monotherapy for the treatment of adult patients with mild to moderate type 1 Gaucher disease for whom enzyme replacement therapy is not a therapeutic option (e.g. due to allergy, hypersensitivity, or poor venous access).

Opfolda:

Indicated, in combination with Pombiliti, for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing greater than or equal to 40 kg and who are not improving on their current enzyme replacement therapy (ERT).

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Compendial Uses

Niemann-Pick disease, type C[3,4]

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Gaucher disease type 1:
  - Initial requests:
    - Beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.
    - Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).
  - Continuation requests:
    - Chart notes or medical records documenting benefit from therapy (e.g. improvement in liver volume, spleen volume, hemoglobin concentration, platelet count).
- Niemann-Pick disease, type C:
  - Genetic testing results showing mutations in NPC1 or NPC2 genes.
- Late-onset Pompe disease:
  - Initial requests:
    - Acid alpha-glucosidase enzyme assay reflecting deficiency of enzyme activity (less than 40% of normal controls) or genetic testing results supporting diagnosis.
    - Chart notes or medical records documenting clinical signs and symptoms of disease (e.g. progressive proximal muscle weakness, respiratory insufficiency, cardiomyopathy).
  - Continuation requests:
    - Chart notes or medical records documenting a positive response to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, muscle strength).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

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Coverage Criteria

Gaucher Disease Type 1 (miglustat (generic)/Yargesa/Zavesca Only)[1,2,6,7]

Authorization of 12 months may be granted for treatment of Gaucher disease type 1 when ALL of the following criteria are met:

- The diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.
- The Member exhibits clinical signs and symptoms of disease at baseline (e.g. bone disease, splenomegaly, hepatomegaly, cytopenia).
- The member has a documented inadequate response to, intolerable adverse events with, or a clinical reason to not use enzyme replacement therapy (e.g., allergy, hypersensitivity, poor venous access).

Niemann-Pick Disease, Type C (miglustat (generic)/Yargesa/Zavesca Only)[3,4]

Authorization of 12 months may be granted for treatment of Niemann-Pick disease, type C when the diagnosis was confirmed by genetic testing results showing pathogenic variants in NPC1 or NPC2 genes.

Late-onset Pompe disease (Opfolda only)[4,5,8]

Authorization of 12 months may be granted for treatment of late-onset Pompe disease when all of the following criteria are met:

- Member is 18 years of age or older.
- Member weighs greater than or equal to 40 kg.
- Diagnosis was confirmed by enzyme assay demonstrating a deficiency of acid alpha-glucosidase enzyme activity (less than 40% of normal controls) or by genetic testing.
- Member is not improving on current enzyme replacement therapy (ERT) (e.g., Lumizyme, Nexviazyme).
- The requested medication will be taken in combination with Pombiliti (cipaglucosidase alfa-atga).
- Member exhibits clinical signs and symptoms of disease (e.g. progressive proximal muscle weakness, respiratory insufficiency, cardiomyopathy).

Continuation of Therapy

Gaucher Disease Type 1 (miglustat (generic)/Yargesa/Zavesca Only)

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for Gaucher disease type 1 when all of the following criteria are met:

- The diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.
- The member is receiving benefit from therapy (e.g. improvement in liver volume, spleen volume, hemoglobin concentration, platelet count) and is not experiencing any intolerable adverse events.

Niemann-Pick Disease, Type C (miglustat (generic)/Yargesa/Zavesca only)

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for Niemann-Pick disease, type C when all of the following criteria are met:

- Member meets the criteria for initial approval.
- The member is receiving benefit from therapy and is not experiencing any intolerable adverse events.

Late-onset Pompe disease (Opfolda only)

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for late-onset Pompe disease when both of the following criteria are met:

- Member is responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, or muscle strength).
- The requested medication will be taken in combination with Pombiliti (cipaglucosidase alfa-atga).

References

1. Zavesca [package insert]. Titusville, NJ: Actelion Pharmaceuticals US, Inc.; August 2022.
2. miglustat [package insert]. Titusville, NJ: CoTherix, Inc.; Decmeber 2022.
3. Lexicomp Online, Lexi-Drugs Online. Waltham, MA: UpToDate, Inc.; Updated November 2, 2024. https://online.lexi.com. Accessed December 1, 2025.
4. National Organization for Rare Disorders. (2003). NORD guide to rare disorders. Philadelphia: Lippincott Williams & Wilkins.
5. Opfolda [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; July 2024.
6. Yargesa [package insert]. Parsippany, NJ: Edenbridge Pharmaceuticals, LLC; July 2024.
7. Hughes DA, Pastores GM. Gaucher Disease. 2000 Jul 27 [Updated 2023 Dec 7]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025.
8. Sperry E, Leslie N, Berry L, et al. Pompe Disease. 2007 Aug 31 [Updated 2025 Aug 21]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025.