AETNA BETTER HEALTH®
Coverage Policy/Guideline

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Effective Date:	6/20/2025	Last Review Date:	6/2025

Applies to:	☒ Illinois	☐ Florida	☐ New Jersey
	☒ Maryland	☒ Florida Kids	☒ Pennsylvania Kids
	☐ Michigan	☒ Virginia	☐ Kentucky PRMD

Intent:

The intent of this policy/guideline is to provide information to the prescribing practitioner outlining the coverage criteria for miglustat products under the patient's prescription drug benefit.

Description:

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).

Compendial Uses

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

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

Applicable Drug List:

Yargesa (miglustat)
miglustat (generic)
Opfolda (miglustat)

Policy/Guideline:

Documentation

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

- Gaucher disease type 1: beta-glucocerebrosidase (glucosidase) enzyme assay or genetic testing results supporting diagnosis.
- 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 or genetic testing results supporting diagnosis.
    - Continuation requests: chart notes 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.

Coverage Criteria

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

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, and
- 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)[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 mutations in NPC1 or NPC2 genes.

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

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 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).

Continuation of Therapy

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

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.
- Member is not experiencing an inadequate response or any intolerable adverse events from therapy.

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

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.
- Member is not experiencing an inadequate response or any intolerable adverse events from therapy.

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).

Approval Duration and Quantity Restrictions:

Initial and Renewal: 12 months

Quantity Level Limit:

- Yargesa (miglustat) 100 mg capsules:
  - 90 capsules per 30 days
  - Exception limit: 180 capsules per 30 days
- Opfolda (miglustat) 65 mg capsules:
  - 8 capsules per 28 days

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 11, 2024.
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; October 2023.