AETNA BETTER HEALTH®
Coverage Policy/Guideline

Name:	Miglustat products	Page:	1 of 3
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).

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)

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.

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.

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.

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

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.