AETNA BETTER HEALTH®
Coverage Policy/Guideline

Name:	Miglustat products: miglustat (generic) / Yargesa / Zavesca	Page:	1 of 2
Effective Date:	11/8/2024	Last Review Date:	9/6/2024
Applies to:	☒ New Jersey		

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:

A. FDA-Approved Indications
   1. miglustat (generic)/Yargesa/Zavesca:
      a. 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).

B. Compendial Uses
   1. Niemann-Pick disease, type C

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

Applicable Drug List:

Zavesca (miglustat)
Yargesa (miglustat)
miglustat (generic)

Policy/Guideline:

Documentation

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

A. Gaucher disease type 1: beta-glucocerebrosidase enzyme assay or genetic testing results supporting diagnosis.
B. Niemann-Pick disease, type C: genetic testing results showing mutations in *NPC1* or *NPC2* genes.

Criteria for Initial Approval:

A. Gaucher disease type 1 (miglustat (generic)/Yargesa/Zavesca only)

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

1. The diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing, and

2. 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).

B. Niemann-Pick disease, type C (miglustat (generic)/Yargesa/Zavesca only)

Authorization 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

A. Gaucher disease type 1 (miglustat (generic)/Yargesa/Zavesca only)

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

1. The diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing.
2. Member is not experiencing an inadequate response or any intolerable adverse events from therapy.

B. Niemann-Pick disease, type C (miglustat (generic)/Yargesa/Zavesca only)

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

1. Member meets the criteria for initial approval.
2. 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: Reference Formulary for drug specific quantity level limits

- Zavesca (miglustat) 100 mg capsules: 90 capsules per 30 days
- Yargesa (miglustat) 100 mg capsules: 90 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.; July 2022.
3. Lexicomp Online, Lexi-Drugs Online. Waltham, MA: UpToDate, Inc.; Updated November 7, 2022. https://online.lexi.com. Accessed December 2, 2022.
4. National Organization for Rare Disorders. (2003). *NORD guide to rare disorders.* Philadelphia: Lippincott Williams & Wilkins.