



AETNA BETTER HEALTH®  
Coverage Policy/Guideline

Name: miglustat products

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Effective Date: 2/10/2024

Last Review Date: 12/1/2023

Applies to:	<input type="checkbox"/> Illinois	<input type="checkbox"/> Florida	<input type="checkbox"/> New Jersey
	<input checked="" type="checkbox"/> Maryland	<input checked="" type="checkbox"/> Florida Kids	<input checked="" type="checkbox"/> Pennsylvania Kids
	<input type="checkbox"/> Michigan	<input checked="" type="checkbox"/> Virginia	<input type="checkbox"/> 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:

#### 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).
2. Opfolda:
  - b. 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).

#### 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)  
Opfolda (miglustat)

### 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.



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C. Late-onset Pompe disease:

1. Initial requests: acid alpha-glucosidase enzyme assay or genetic testing results supporting diagnosis.
2. 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).

**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.

**C. Late-onset Pompe disease (Opfolda only)**

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

1. Member is 18 years of age or older.
2. Member weighs greater than or equal to 40 kg.
3. Diagnosis was confirmed by enzyme assay demonstrating a deficiency of acid alpha-glucosidase enzyme activity or by genetic testing.
4. The requested medication will be taken in combination with Pombiliti (cipaglucosidase alfa-atga).
5. Member is not improving on current enzyme replacement therapy (ERT) (e.g., Lumizyme, Nexviazyme).

**Continuation of Therapy**

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



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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.

**C. Late-onset Pompe disease (Opfolda only)**

Authorization may be granted for continued treatment in members requesting reauthorization for late-onset Pompe disease who are responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, or muscle strength).

**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

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.; 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.