	TTER HEALTH® Policy/Guideline		<b>*a</b>	etna™
Name:	miglustat product	S	Page:	1 of 3
Effective Date: 7/15/2024			Last Review Date	: 5/2024
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:**

- 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:

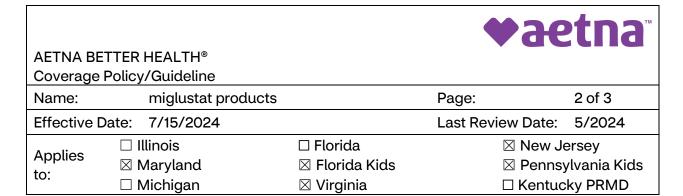
Zavesca (miglustat) Yargesa (miglustat) miglustat (generic) Opfolda (miglustat)

#### **Policy/Guideline:**

#### Documentation

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

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



## C. Late-onset Pompe disease:

- 1. <u>Initial requests</u>: acid alpha-glucosidase enzyme assay or genetic testing results supporting diagnosis.
- 2. <u>Continuation requests</u>: 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.

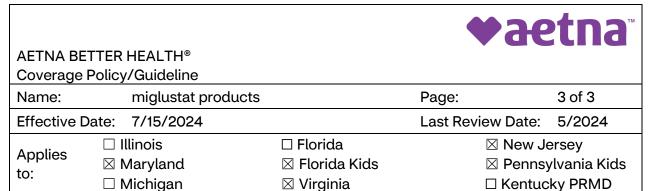
#### C. Late-onset Pompe disease (Opfolda only)

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)



Authorization may be granted for continued treatment in members requesting reauthorization for Gaucher disease type 1 when ALL 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.
- 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:**

- 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.; December 2022.
- 3. Lexicomp Online, Lexi-Drugs Online. Waltham, MA: UpToDate, Inc.; Updated November 24, 2023. https://online.lexi.com. Accessed December 6, 2023.
- 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; September 2023
- 6. Yargesa [package insert]. Parsippany, NJ: Edenbridge Pharmaceuticals, LLC; January 2022.