AETNA BETTER HEALTH®
Coverage Policy/Guideline

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| Name: | miglustat products | Page: | 1 of 3 |
| Effective Date: | 7/15/2024 | Last Review Date: | 5/2024 |

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| 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. 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.

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)**

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:**

- 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.