CVS Caremark®

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| Reference number(s) |
| 2098-A |

# Specialty Guideline Management miglustat products

## Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name |
| --- | --- |
| Zavesca | miglustat |
| Yargesa | miglustat |
| Opfolda | miglustat |

## Indications

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 Indications1,2,5,6

#### 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).

#### Opfolda:

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).

### Compendial Uses

Niemann-Pick disease, type C3,4

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

## 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.
* Late-onset Pompe disease:
  + Initial requests: acid alpha-glucosidase enzyme assay or genetic testing results supporting diagnosis.
  + 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).

## 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/Zavesca Only)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/Zavesca Only)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.

### Late-onset Pompe disease (Opfolda only)5

Authorization of 12 months may be granted for treatment of late-onset Pompe disease when all of the following criteria are met:

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

## Continuation of Therapy

### Gaucher Disease Type 1 (miglustat (generic)/Yargesa/Zavesca Only)

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/Zavesca only)

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.

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

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for late-onset Pompe disease when both of the following criteria are met:

* Member is responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, or muscle strength).
* The requested medication will be taken in combination with Pombiliti (cipaglucosidase alfa-atga).

## 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.