

<b>Effective Date: 12/01/2020</b>
Reviewed Date: 9/2020, 5/2021, 4/2022, 3/2023, 5/2024, 2/2025, 3/2026
Scope: Medicaid

## Miglustat 100 mg capsules and Opfolda (miglustat) 65 mg capsules

### POLICY

#### I. 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 Indications

Miglustat is 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).

##### Compendial Uses

- Neiman-Pick disease (NPC) type C
- Pompe Disease (lysosomal acid alpha-glucosidase deficiency), late onset (Please refer to Pombiliti & Opfolda Policy via Pharmacy Medical Benefit)

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

#### II. CRITERIA FOR INITIAL APPROVAL

##### **Gaucher disease type 1**

Authorization of 6 months may be granted for treatment of Gaucher disease type 1 when the diagnosis of Gaucher disease was confirmed by enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) enzyme activity or by genetic testing with documentation provided, and the member has a documented inadequate response or intolerable adverse events with enzyme replacement therapy.

##### **Neiman-Pick disease type C (NPC)**

Authorization of 6 months may be granted for treatment of Neiman-Pick disease type C (NPC) when all of the following criteria are met:

- A. The diagnosis is confirmed by one of the following:
  - a. Genetically confirmed variant in both alleles of NPC1 or NPC2.
  - b. Mutation in only one allele of NPC1 or NPC2 plus either positive filipin staining or elevated cholestane-triol level (>2 times the upper limit of normal).
- B. If the request is for miglustat 100 mg capsules, documentation of inadequate response, intolerance, or contraindication to Opfolda 65 mg capsules.

#### III. CONTINUATION OF THERAPY

##### **Gaucher disease type 1**

Authorization of 6 months may be granted for continued treatment in members requesting reauthorization for Gaucher disease type 1 when all of the following are met:

- A. Documentation that the patient meets the criteria for initial approval

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- B. Documentation that the patient is not experiencing an inadequate response or any intolerable adverse events from therapy.

**Neiman-Pick disease type C (NPC)**

Authorization of 6 months may be granted for continued treatment in members requesting reauthorization for Neiman-Pick disease type C (NPC) when all of the following are met:

- A. Documentation that the patient meets the criteria for initial approval
- B. Documentation that the patient is not experiencing an inadequate response or any intolerable adverse events from therapy.

**IV. QUANTITY LIMIT**

Miglustat 100 mg capsule has a quantity limit of 3 capsules per day for treatment of Gaucher disease.

Opfolda 65 mg capsule has a quantity limit of 9 capsules per day for the treatment of Neiman-Pick disease type C (NPC).

**V. REFERENCES**

1. Miglustat [package insert]. Horsham, PA: Patriot Pharmaceuticals, LLC.; December 2023.
2. Opfolda [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC.; July 2024.