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

SPECIALTY GUIDELINE MANAGEMENT

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 meet:
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.

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 meet:

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.