

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

## SPECIALTY GUIDELINE MANAGEMENT

### miglustat

#### 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).

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

##### II. REQUIRED DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review: beta-glucocerebrosidase enzyme assay or genetic testing results supporting diagnosis

##### III. 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, and the member has a documented inadequate response or intolerable adverse events with enzyme replacement therapy.

##### IV. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for continued treatment in members requesting reauthorization for Gaucher disease type 1 who are not experiencing an inadequate response or any intolerable adverse events from therapy.

##### V. QUANTITY LIMIT

Miglustat: 3 capsules per day

##### VI. REFERENCES

1. Miglustat [package insert]. Horsham, PA: Patriot Pharmaceuticals, LLC.; December 2023.