

miglustat (Opfolda)

Commercial Medical Benefit Drug Policy

Place of Service

Infusion Center Administration
Office Administration
Outpatient Facility Administration

Drug Details

USP Category: GENETIC OR ENZYME OR PROTEIN DISORDER: REPLACEMENT, MODIFIERS, TREATMENT

Mechanism of Action: Enzyme stabilizer

HCPCS:

J1202:Miglustat, oral, 65 mg

How Supplied:

65 mg capsule

- 4 count bottle: 71904-300-01
- 24 count bottle: 71904-300-02
- 100 count bottle: 71904-300-03

Condition(s) listed in policy *(see coverage criteria for details)*

- Late-onset Pompe Disease (LOPD)

Any condition not listed in this policy requires a review to confirm it is medically necessary. For conditions that have not been approved for intended use by the Food and Drug Administration (i.e., off-label use), the criteria outlined in the Health and Safety Code section 1367.21 must be met.

Special Instructions and Pertinent Information

Provider must submit documentation (such as office chart notes, lab results or other clinical information) to ensure the member has met all medical necessity requirements.

The member's specific benefit may impact drug coverage. Other utilization management processes, and/or legal restrictions may take precedence over the application of this clinical criteria.

For billing purposes, drugs must be submitted with the drug's assigned HCPCS code (as listed in the drug policy) and the corresponding NDC (national drug code). An unlisted, unspecified, or miscellaneous code should not be used if there is a specific code assigned to the drug.

Coverage Criteria

The following condition(s) require Prior Authorization/Preservice.

Late-onset Pompe Disease (LOPD)

Meets medical necessity if all the following are met:

1. One of the following (a or b)
 - a. Genetic testing showing acid alpha-glucosidase (GAA) mutation
 - b. An enzyme assay showing absent or decreased GAA activity from blood, skin, or muscle tissues
2. Age and weight consistent with FDA-approved indication (adults weighing ≥ 40 kg)

3. Used in combination with Pombiliti (cipaglucosidase alfa-atga)
4. Inadequate response to one currently approved ERT for LOPD: Lumizyme (alglucosidase alfa)
OR Nexviazyme (avalglucosidase alfa-ngpt)

Covered Doses:

Actual body weight	
At least 50 kg	260 mg given orally every other week
At least 40 kg to less than 50 kg	195 mg given orally every other week

Coverage Period:

Yearly, based on continued response to therapy

ICD-10:

E74.02

References

1. AHFS®. Available by subscription at <http://www.lexi.com>
2. DrugDex®. Available by subscription at <http://www.micromedexsolutions.com/home/dispatch>
3. Opfolda (miglustat) Prescribing Information. Amicus Therapeutics US LLC, Philadelphia, PA: 7/2024.

Review History

Date of Last Annual Review: 1Q2026

Changes from previous policy version:

- No clinical change following annual review.

*Blue Shield of California Medication Policy to Determine Medical Necessity
Reviewed by P&T Committee*