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.

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	
≥ 50 kg	260 mg given orally every other week
≥40 kg to <50 kg	195 mg given orally every other week

Coverage Period:
Initial: 1 year
Reauthorization: Yearly if there is continued benefit from therapy

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]. Philadelphia, PA: Amicus Therapeutics US LLC; September 2023.

Review History

Date of Last Annual Review: 1Q2025
Changes from previous policy version:

- No clinical changes following annual review

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