

## miglustat (gaa deficiency) (Opfolda®)

### 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:

- Through 3/31/2024: C9399, J8499: Oral prescrip drug non chemo
- Effective 4/1/2024 and after: 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)

#### Special Instructions and pertinent information

#### The following condition(s) require Prior Authorization/Preservice:

#### Late-onset Pompe Disease (LOPD)

1. One of the following:
  - a. Genetic testing showing acid alpha-glucosidase (GAA) mutation, or
  - b. An enzyme assay showing absent or decreased GAA activity from blood, skin, or muscle tissues, AND
2. Age and weight consistent with FDA-approved indication (adults weighing  $\geq 40$  kg), AND
3. Used in combination with Pombiliti (cipaglucosidase alfa-atga), AND
4. Inadequate response to one currently approved ERT for LOPD: Lumizyme (alglucosidase alfa) OR Nexviazyme (avalglucosidase alfa-ngpt)

#### Covered Doses:

Actual body weight	
$\geq 50$ kg	260 mg given orally every other week

≥40 kg to <50 kg	195 mg given orally every other week
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**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; 9/2023.

**Review History**

Date of Last Annual Review: 02/28/2024

Date of last revision: 04/03/2024

Changes from previous policy version:

- Effective 4/1/2024 and after: J1202, Miglustat, oral, 65 mg

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