

## 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)

- One of the following:
  - Genetic testing showing acid alpha-glucosidase (GAA) mutation, or
  - An enzyme assay showing absent or decreased GAA activity from blood, skin, or muscle tissues, AND
- Age and weight consistent with FDA-approved indication (adults weighing  $\geq 40$  kg), AND
- Used in combination with Pombiliti (cipaglucosidase alfa-atga), AND
- 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 |
|------------------|--------------------------------------|

### 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*