miglustat (Opfolda)

Commercial Pharmacy Benefit Drug Policy

Drug Details

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

Mechanism of Action: Enzyme stabilizer

Label Name	Quantity Limit
Opfolda 65 MG CAP	8 caps/28 days

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

- Late-onset Pompe Disease (LOPD)

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.

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 ≥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	
≥ 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: New policy
Date of last revision: 02/28/2024
Changes from previous policy version:

- New policy

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