Medical Policy:

Opfolda (miglustat) oral capsules

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.399	February 25, 2025	October 30, 2023

Definitions

Opfolda is indicated, in combination with Pombiliti, for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing ≥40 kg and who are not improving on their current enzyme replacement therapy (ERT).

Length of Authorization

12 months

Dosing Limits [Medical Benefit]

- (50 kg or greater, actual body weight) 260 mg orally every other week 1 hour before IV infusion of cipaglucosidase alfa-atga; patient should begin fasting approximately 2 hours prior to migLUstat dose and continue fasting until 1 hour after start of cipaglucosidase alfa-atga infusion
- (40 kg to less than 50 kg) 195 mg orally every other week 1 hour before IV infusion of cipaglucosidase alfa-atga; patient should begin fasting approximately 2 hours prior to migLUstat dose and continue fasting until 1 hour after start of cipaglucosidase alfa-atga infusion

Guideline

I. INITIAL CRITERIA

1. **Acid Alpha-Glucosidase Deficiency (Pompe Disease).** Approve if the patient meets the following (A, B, C, D, E, and F):
    A. Patient is ≥ 18 years of age; **AND**
    B. Patient weighs ≥ 40 kg; **AND**
    C. The medication will be used in combination with Pombiliti; **AND**
    D. Patient has not demonstrated an improvement in objective measures after receiving one of the following for at least one year (i or ii):
    Note: Examples of objective measures include forced vital capacity (FVC) and six-minute walk test (6MWT)
        i. Lumizyme (alglucosidase alfa) intravenous infusion; **OR**
        ii. Nexviazyme (avalglucosidase alfa-ngpt) intravenous infusion; **AND**
    E. Patient has late-onset acid alpha-glucosidase deficiency (late-onset Pompe disease) with diagnosis established by one of the following (i or ii):
        i. Patient has a laboratory test demonstrating deficient acid alpha-glucosidase activity in blood, fibroblasts, or muscle tissue; **OR**
        ii. Patient has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic acid alpha-glucosidase (GAA) gene variants; **AND**
    F. The medication is prescribed by or in consultation with a geneticist, neurologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

II. RENEWAL CRITERIA

1. Member has responded positively to the treatment as determined by the prescribing physician; **AND**
2. Member has not experienced unacceptable toxicity from the drug.

Applicable Procedure Codes

Code	Description
J1202	Miglustat, oral, 65 mg

Applicable NDCs

Code	Description
71904-0300-xx	Opfolda Capsule

ICD-10 Diagnoses

Code	Description
E74.02	Pompe Disease

Revision History

Company(ies)	DATE	REVISION

EmblemHealth & ConnectiCare	2/25/2025	Annual Review: length of authorization: updated from Initial: 6 months Continuation: 12 months to 12 months. Initial criteria: Updated the following statement from: "Patient has a molecular genetic test demonstrating acid alpha-glucosidase gene mutation" to "biallelic pathogenic or likely pathogenic acid alpha-glucosidase (GAA) gene variants; AND"
EmblemHealth & ConnectiCare	5/2/2024	Annual Review: deleted J8499, added J1202, No criteria changes
EmblemHealth & ConnectiCare	10/30/2023	New Policy

References

1. Product Information: OPFOLDA™ oral capsules, miglustat oral capsules. Amicus Therapeutics US LLC (per FDA), Philadelphia, PA, 2023.