

Medical Policy:

Opfolda (miglustat) oral capsules

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

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The treating physician or primary care provider must submit to EmblemHealth, or ConnectiCare, as applicable (hereinafter jointly referred to as “EmblemHealth”), the clinical evidence that the member meets the criteria for the treatment or surgical procedure. Without this documentation and information, EmblemHealth will not be able to properly review the request preauthorization or post-payment review. The clinical review criteria expressed below reflects how EmblemHealth determines whether certain services or supplies are medically necessary. This clinical policy is not intended to pre-empt the judgment of the reviewing medical director or dictate to health care providers how to practice medicine. Health care providers are expected to exercise their medical judgment in rendering appropriate care. Health care providers are expected to exercise their medical judgment in rendering appropriate care.

EmblemHealth established the clinical review criteria based upon a review of currently available clinical information (including clinical outcome studies in the peer reviewed published medical literature, regulatory status of the technology, evidence-based guidelines of public health and health research agencies, evidence-based guidelines and positions of leading national health professional organizations, views of physicians practicing in relevant clinical areas, and other relevant factors). EmblemHealth expressly reserves the right to revise these conclusions as clinical information changes and welcomes further relevant information. Each benefit program defines which services are covered. The conclusion that a particular service or supply is medically necessary does not constitute a representation or warranty that this service or supply is covered and/or paid for by EmblemHealth, as some programs exclude coverage for services or supplies that EmblemHealth considers medically necessary.

If there is a discrepancy between this guideline and a member's benefits program, the benefits program will govern. Identification of selected brand names of devices, tests and procedures in a medical coverage policy is for reference only and is not an endorsement of any one device, test or procedure over another. In addition, coverage may be mandated by applicable legal requirements of a state, the Federal Government or the Centers for Medicare & Medicaid Services (CMS) for Medicare and Medicaid members. All coding and web site links are accurate at time of publication.

EmblemHealth may also use tools developed by third parties, such as the MCG™ Care Guidelines, to assist us in administering health benefits. The MCG™ Care Guidelines are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice. EmblemHealth Services Company, LLC, has adopted this policy in providing management, administrative and other services to EmblemHealth Plan, Inc., EmblemHealth Insurance Company, EmblemHealth Services Company, LLC, and Health Insurance Plan of Greater New York (HIP) related to health benefit plans offered by these entities. ConnectiCare, an EmblemHealth company, has also adopted this policy. All of the aforementioned entities are affiliated companies under common control of EmblemHealth Inc.

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 cipaglucoisidase alfa-atga; patient should begin fasting approximately 2 hours prior to migLUstat dose and continue fasting until 1 hour after start of cipaglucoisidase alfa-atga infusion
- (40 kg to less than 50 kg) 195 mg orally every other week 1 hour before IV infusion of cipaglucoisidase alfa-atga; patient should begin fasting approximately 2 hours prior to migLUstat dose and continue fasting until 1 hour after start of cipaglucoisidase 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
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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.