Commercial PA Criteria

Effective: October 30, 2023

Prior Authorization: Opfolda (miglustat)

Products Affected: Opfolda (miglustat) oral capsules

Medication Description: 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).

Covered Uses: OPFOLDA is indicated, in combination with Pombiliti, for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency)

Exclusion Criteria:

1. Pregnancy
2. Gaucher Disease

Required Medical Information:

1. Diagnosis
2. Current Medications
3. Previous Therapies Tried and Failed

Prescriber Restriction: 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.

Age Restriction: Patient is ≥ 18 year of age

Coverage Duration:

Initial: 6 months

Continuation: 12 months

Other Criteria:

Initial Approval Criteria

1. **Acid Alpha-Glucosidase Deficiency (Pompe Disease).** Approve if the patient meets the following (A, B, C, and D):
   A. Patient weighs ≥ 40 kg; **AND**
   B. The medication will be used in combination with Pombiliti; **AND**
   C. 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**

D. 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 acid alpha-glucosidase gene mutation

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.

References:

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

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	10/30/2023