Pharmacy Drug Policy & Procedure

Policy Name:	Pombiliti and Opfolda (cipaglucosidase alfa and miglustat)	Policy#:	2775P

Purpose of the Policy

The purpose of this policy is to define coverage criteria for Pombiliti and Opfolda (cipaglucosidase alfa and miglustat).

Statement of the Policy

Health Alliance Medical Plans will approve the use of Pombiliti and Opfolda (cipaglucosidase alfa and miglustat) under the applicable specialty benefit if the following criteria are met.

Criteria

1. Coverage Criteria for the Treatment of Pompe Disease

1.1 Diagnosis of late onset Pompe disease as supported by BOTH of the following:
- Enzyme assay showing a deficiency of acid alpha-glucosidase (GAA) activity in the blood, skin, or muscle
- Genetic testing showing a mutation in the GAA gene

1.2 Patient has clear signs of Pompe disease such as impairment in lung function or ability to move

1.3 Documentation showing baseline sitting forced vital capacity (FVC) >30% of the predicted value for healthy adults

1.4 Documentation showing baseline 6-minute walk distance (6MWD) is at least 75 meters

1.5 Age 18 years or older and at least 40kg

1.6 Prescribed by or in consultation with a geneticist (genetic disorder doctor) or specialist in Pompe disease

1.7 Documentation provided patient is no longer improving on enzyme replacement therapy (Lumizyme or Nexviazyme)

1.8 Review of chart notes documenting diagnosis and confirming that patient has met all above requirements for treatment by both a pharmacist and medical director

2. Exclusion Criteria

2.1 Concomitant use with enzyme replacement therapy is considered a duplication of therapy and excluded from coverage

3. Managed Dose Limit

3.1 Opfolda: #120 capsules/30 days

4. Approval Period

4.1 Initial Approval: 12 months

4.2 Reapproval: 12 months with documentation of positive clinical response as evidenced by an improvement, stabilization, or slowing of progression of percent-predicted FVC and/or 6MWD

CPT Codes

HCPCS Codes	
J1203	Injection, cipaglucosidase alfa-atga, 5 mg
J1202	Miglustat, oral, 65 mg

References

1. Pombiliti (cipaglucosidase alfa) [prescribing information]. Philadelphia, PA: Amicus Therapeutics UF, LLC; September 2023.
2. Opfolda (miglustat) [prescribing information]. Philadelphia, PA: Amicus Therapeutics US LLC; July 2024.
3. Schoser B, et al. Safety and efficacy of cipaglucosidase alfa plus miglustat versus alglucosidase alfa plus placebo in late-onset Pompe disease (PROPEL): an international, randomised, double-blind, parallel-group, phase 3 trial, Lancet Neurol. 2023 Oct;22(10):e11.

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