Medical Policy:
Pombiliti (cipaglucosidase alfa-atga) intravenous injection

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.400	October 30, 2023	October 30, 2023

Definitions

POMBILITI is indicated, in combination with Opfolda, 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

6 months

Dosing Limits [Medical Benefit]

20 mg/kg (actual body weight) cipaglucosidase alfa-atga IV infusion over 4 hours administered every other week, in combination with migLUstat 195 mg or 260 mg orally approximately 1 hour prior to cipaglucosidase alfa-atga infusion; 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

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 year of age; **AND**
   B. Patient weighs ≥ 40 kg; **AND**
   C. The medication will be used in combination with Opfolda; **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 acid alpha-glucosidase gene mutation; **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

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
J3590	Unclassified

Applicable NDCs

Code	Description
71904-0200-xx	Pombiliti

ICD-10 Diagnoses

Code	Description
E74.02	Pompe Disease

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	10/30/2023	New Policy

References

1. Product Information: POMBILITI™ intravenous injection, cipaglucosidase alfa-atga intravenous injection. Amicus Therapeutics US LLC (per FDA), Philadelphia, PA, 2023.