

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

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

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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) cipaglusosidase 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 cipaglusosidase alfa-atga infusion; patient should begin fasting approximately 2 hours prior to migLUstat dose and continue fasting until 1 hour after start of cipaglusosidase 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 \geq 18 year of age; **AND**
- B. Patient weighs \geq 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.