

Standard Guideline Management

Pombiliti

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Pombiliti	cipaglucosidase alfa-atga

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications¹

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 greater than or equal to 40 kg and who are not improving on their current enzyme replacement therapy (ERT).

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

- Initial requests:
 - Acid alpha-glucosidase enzyme assay reflecting deficiency of enzyme activity (less than 40% of normal controls) or genetic testing results supporting diagnosis.
 - Chart notes or medical records documenting clinical signs and symptoms of disease (e.g. progressive proximal muscle weakness, respiratory insufficiency, cardiomyopathy).
- Continuation requests:
 - Chart notes or medical records documenting a positive response to therapy (e.g.,

improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, muscle strength).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Late-onset Pompe disease¹⁻²

Authorization of 12 months may be granted for treatment of late-onset Pompe disease when all of the following criteria are met:

- Member is 18 years of age or older.
- Member weighs greater than or equal to 40 kg.
- Diagnosis was confirmed by enzyme assay demonstrating a deficiency of acid alpha-glucosidase enzyme activity (less than 40% of normal controls) or by genetic testing.
- Member exhibits clinical signs and symptoms of disease (e.g. progressive proximal muscle weakness, respiratory insufficiency, cardiomyopathy).
- Member is not improving on current enzyme replacement therapy (ERT) (e.g., Lumizyme, Nexvazyme).
- The requested medication will be taken in combination with Opfolda (miglustat).

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria section when both of the following criteria are met:

- Member is responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, or muscle strength).
- The requested medication will be taken in combination with Opfolda (miglustat).

References

1. Pombiliti [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; July 2024.
2. Sperry E, Leslie N, Berry L, et al. Pompe Disease. 2007 Aug 31 [Updated 2025 Aug 21]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025.