

Enzyme and Metabolic Disorder Therapies
Pombiliti (cipaglucosidase alfa-atga)
Effective 07/01/2025

Plan	<input checked="" type="checkbox"/> MassHealth <input type="checkbox"/> Commercial/Exchange		Program Type	<input checked="" type="checkbox"/> Prior Authorization
Benefit	<input type="checkbox"/> Pharmacy Benefit <input checked="" type="checkbox"/> Medical Benefit			<input type="checkbox"/> Quantity Limit <input type="checkbox"/> Step Therapy
Specialty Limitations	N/A			
Contact Information	Medical Benefit Pharmacy Benefit		Phone: 833-895-2611 Phone: 800-711-4555	Fax: 888-656-6671 Fax: 844-403-1029
Exceptions	N/A			

Overview

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).

Coverage Guidelines

Authorization may be granted for members when all the following criteria are met:

1. Diagnosis of late-onset Pompe Disease
2. Prescriber is a specialist in genetic or metabolic diseases or consult notes from specialist are provided
3. Member's current weight is ≥ 40 kg
4. ONE of the following confirming diagnosis:
 - a. results from GAA assay test showing reduced or absent activity from cultured skin fibroblasts
 - b. lymphocyte testing
 - c. blood spot assay
 - d. genetic testing confirming mutation in GAA gene
5. Member is ≥ 18 years of age
6. Inadequate response or adverse reaction to **ONE**, or contraindication to **BOTH** of the following*:
 - a. Lumizyme
 - b. Nexvazyme
7. Requested agent will be used in combination with Opfolda (miglustat 65 mg)
8. Dosing is appropriate within the FDA labeling

**Lumizyme or Nexvazyme should not be used concurrently with Opfolda and Pombiliti*

Continuation of Therapy

Resubmission by prescriber will infer a positive response to therapy.

Limitations

1. Approvals may be granted for 1 year.

References

1. Pombiliti [package insert] Philadelphia (PA): Amicus Therapeutics; 2024 Nov.

Review History

6/12/24 – Created for P&T. Adopted MH criteria (Enzyme and Metabolic Disorder Therapies guideline). Pombiliti will require PA through medical benefit only. Effective 7/1/24.

06/11/25 – Reviewed and updated for P&T. Part of annual UM review. Updated formatting and references. Effective 7/1/25

