Mass General Brigham
Health Plan

Pombiliti (cipaglucosidase alfa-atga)
Effective 07/01/2024

Plan	☒ MassHealth UPPL ☐Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☐ Pharmacy Benefit ☒ Medical Benefit		
Specialty Limitations	N/A		
Contact Information	**Medical and Specialty Medications** All Plans | Phone: 877-519-1908 | Fax: 855-540-3693		
	Non-Specialty Medications All Plans | Phone: 800-711-4555 | 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. Nexviazyme
7. Requested agent will be used in combination with Opfolda (miglustat 65 mg)
8. Dosing is appropriate within the FDA labeling

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

<u>Continuation of Therapy</u>
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; 2023 Sept.

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.