Mass General Brigham
Health Plan

Enzyme and Metabolic Disorder Therapies
Pombiliti (cipaglucosidase alfa-atga)
Effective 01/04/2027

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 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
If member is new to the plan (as evidenced by coverage effective date of less than or equal to 90 days), submission of medical records documenting that the member is currently receiving treatment with the requested drug, excluding when the product is obtained as samples or via manufacturer's patient assistance programs
OR
Authorization may be granted when all of 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. Medical records or a copy of testing to support **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. results from 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

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
11/4/26 – Reviewed and updated for P&T. Updated formatting. Effective 1/4/27