Mass General Brigham
Health Plan

Pombiliti (cipaglucosidase alfa-atga)
Effective 03/01/2024

Plan	☐ MassHealth UPPL ☒Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☐ Pharmacy Benefit ☒ Medical Benefit		
Specialty Limitations			
Contact Information	**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, an enzyme stabilizer, 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 new to the plan who are currently receiving treatment with the requested medication, excluding when the product is obtained as samples or via manufacturer's patient assistance programs.
OR
Authorization may be granted when the following criteria is met:

1. The member has a diagnosis of late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency)
2. Medical records confirming disease by ONE of the following:
    a. Absence or deficiency (less than 40% of the lab specific normal mean) of GAA enzyme activity in lymphocytes, fibroblasts, or muscle tissues as confirmed by an enzymatic assay
    b. Molecular genetic testing confirming mutations in the GAA gene
3. Clinical signs and symptoms of the disease (e.g., respiratory distress, skeletal muscle weakness, etc)
4. Medication is being used in combination with Opfolda (miglustat)
5. Weight is greater than or equal to 40kg
6. Member has had trial and inadequate response to Lumizyme AND Nexviazyme
7. Medication is not being used in combination with other miglustat products (i.e., Zavesca, Yargesa)

Continuation of Therapy
Authorization may be granted for continued treatment in members when the following criteria are met:

1. Patient demonstrates a positive clinical response to therapy (e.g., improvement in FVC, improvement in 6-minute walk distance [6MWD])
2. Medication is being used in combination with Opfolda (miglustat)

3. Medication is not being used in combination with other miglustat products (i.e., Zavesca, Yargesa)

Limitations

1. Initial approvals will be granted for 12 months.
2. Reauthorizations will be granted for 24 months.

References

1. Pombiliti Prescribing Information. Amicus Therapeutics US, LLC. Philadelphia, PA. Sept 2023.
2. Diaz, C., Diaz-Manera, J. Therapeutic Options for the Management of Pompe Disease: Current Challenges and Clinical Evidence in Therapeutics and Clinical Risk Management. Available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC9759116/. Accessed November 2, 2023.
3. Cleveland Clinic - Pompe Disease. Available at: https://my.clevelandclinic.org/health/diseases/15808-pompe-disease. Accessed November 2, 2023.
4. Cupler, E., Berger, K., Leshner, R., et al. Consensus Treatment Recommendations for Late-Onset Pompe Disease. Available at: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3534745/. Accessed November 2, 2023.
5. Barba-Romero MA, Barrot E, Bautista-Lorite J, et al. Clinical guidelines for late-onset Pompe disease. Available at: https://www.orpha.net/data/patho/Cpg/en/PompeLateOnset_ES_en_CPG_ORPHA420429.pdf. Accessed November 2, 2023.

Review History

2/14/2023: Created and Reviewed at Feb P&T, Effective 3/1/2024