Pombiliti® (cipaglucosidase alfa-atga)
(Intravenous)

Document Number: OHSU HEALTHSERVICES-0731

Last Review Date: 04/01/2026
Date of Origin: 10/30/2023
Dates Reviewed: 11/2023, 02/2024, 04/2025, 04/2026

I. Length of Authorization

- Initial: Prior authorization validity will be provided initially for 12 months (365 days).
- Renewal: Prior authorization validity may be renewed every 12 months (365 days) thereafter.

II. Dosing Limits

Max Units (per dose and over time) [HCPCS Unit]:

- 462 billable units every 14 days

III. Initial Approval Criteria [1,4]

Prior authorization validity is provided in the following conditions:

- Member age is at least 18 years of age; **AND**
- Member has documented baseline values for percent predicted forced vital capacity (FVC) and/or 6-minute walk test (6MWT); **AND**
- Member does NOT have any FDA labeled contraindications to the requested agent; **AND**
- Member is experiencing an inadequate response or intolerance to their current enzyme replacement therapy; **AND**

Universal Criteria [1]

- Will not be used in combination with other enzyme replacement therapies; **AND**
- Will be used in combination with the enzyme stabilizer formulation miglustat (Opfolda); **AND**
- Members susceptible to fluid volume overload or those with an acute underlying respiratory illness or compromised cardiac or respiratory function, will be closely monitored for exacerbation of their cardiac or respiratory status during infusion; **AND**
- Member has an actual body weight of at least 40 kilograms; **AND**

Pompe Disease (Acid Alpha-Glucosidase [GAA] deficiency) † Φ [1,4]

- Diagnosis has been confirmed by one of the following:
  - Deficiency of acid alpha-glucosidase (GAA) enzyme activity; **OR**
  - Detection of biallelic pathogenic (or likely pathogenic) variants in the *GAA* gene by molecular genetic testing; **AND**
- Member has a diagnosis of late-onset (non-infantile) disease

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria [1,4]

Prior authorization validity may be renewed based on the following criteria:

- Member continues to meet the universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: severe hypersensitivity reactions including anaphylaxis, severe infusion-associated reactions, acute cardiorespiratory failure, etc.; **AND**
- Member has demonstrated a beneficial response to therapy compared to pretreatment baseline as indicated by stabilization or improvement in FVC and/or 6-MWT; **AND**
- Member is being monitored for antibody formation (including neutralizing antibodies)

V. Dosage/Administration [1]

Indication	Dose
Pompe Disease	Administer 20 mg/kg (of actual body weight) every two weeks as an intravenous infusion over approximately 4 hours. - Members must weigh ≥40 kg. - Pombiliti must be administered in combination with Opfolda (see PI for both products for dosing timeline). If the Opfolda dose is missed, Pombiliti should not be administered. - Prior to Pombiliti administration, consider pretreating with antihistamines, antipyretics, and/or corticosteroids. If premedication was used with previous enzyme replacement therapy (ERT), prior to Pombiliti administration, pretreat with antihistamines, antipyretics, and/or corticosteroids. - Start Pombiliti in combination with Opfolda two weeks after the last ERT dose. - Initiate Pombiliti ~1 hour after oral administration of Opfolda. If the Pombiliti infusion cannot be started within 3 hours of oral administration of Opfolda, reschedule Pombiliti in combination with Opfolda at least 24 hours after Opfolda was last taken.

VI. Billing Code/Availability Information

HCPCS Code:

- J1203 – Injection, cipaglucosidase alfa-atga, 5 mg; 1 billable unit = 5mg

NDC:

- Pombiliti 105 mg single-dose vial as a powder for injection: 71904-0200-xx

VII. References

1. Pombiliti [package insert]. Philadelphia, PA; Amicus Therapeutics, LLC.; July 2024. Accessed March 2026.
2. Cupler EJ, Berger KI, Leshner RT, et al. Consensus treatment recommendations for late-onset Pompe disease. Muscle Nerve. 2012 Mar; 45(3):319-33. doi: 10.1002/mus.22329. Epub 2011 Dec 15.
3. Kishnani PS, Steiner RD, Bali D, et al. Pompe disease diagnosis and management guidelines. *Genet Med* 2006; 8:267-88.
4. Sperry E, Leslie N, Berry L, et al. Pompe Disease. GeneReviews. www.ncbi.nlm.nih.gov/books/NBK1261/. Initial Posting: August 31, 2007; Last Update: August 21, 2025. Accessed March 2026.
5. Tarnopolsky M, Katzberg H, Petrof BJ, et al. Pompe Disease: Diagnosis and Management. Evidence-Based Guidelines from a Canadian Expert Panel. Can J Neurol Sci. 2016 Jul;43(4):472-85.
6. Kishnani PS, Hwu WL, et al. Introduction to the Newborn Screening, Diagnosis, and Treatment for Pompe Disease Guidance Supplement. Pediatrics 2017 Jul:(1):S1-S3.
7. Schoser B, Roberts M, Byrne BJ, et al. Safety and efficacy of cipaglucosidase alfa plus miglustat versus alglucosidase alfa plus placebo in late-onset Pompe disease (PROPEL): an international, randomised, double-blind, parallel-group, phase 3 trial [published correction appears in Lancet Neurol. 2023 Oct;22(10):e11]. *Lancet Neurol*. 2021;20(12):1027-1037. doi:10.1016/S1474-4422(21)00331-8.

Appendix A – Non-Quantitative Treatment Limitations (NQTL) Factor Checklist

Non-quantitative treatment limitations (NQTLs) refer to the methods, guidelines, standards of evidence, or other conditions that can restrict how long or to what extent benefits are provided under a health plan. These may include things like utilization review or prior authorization. The utilization management NQTL applies comparably, and not more stringently, to mental health/substance use disorder (MH/SUD) Medical Benefit Prescription Drugs and medical/surgical (M/S) Medical Benefit Prescription Drugs. The

table below lists the factors that were considered in designing and applying prior authorization to this drug/drug group, and a summary of the conclusions that Prime's assessment led to for each.

Factor	Conclusion
Indication	Yes: Consider for PA
Safety and efficacy	Yes: Consider for PA
Potential for misuse/abuse	No: PA not a priority
Cost of drug	Yes: Consider for PA

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E74.02	Pompe disease

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC