Pombiliti® (cipaglucosidase alfa-atga)
(Intravenous)

Document Number: IC-0731

Last Review Date: 04/07/2025
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Dates Reviewed: 11/2023, 02/2024, 04/2025

I. Length of Authorization

Coverage will be provided for 12 months and may be renewed.

II. Dosing Limits

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

- 462 billable units every 14 days

III. Initial Approval Criteria [1,4]

Coverage is provided in the following conditions:

- Patient age is at least 18 years of age; **AND**
- Patient has documented baseline values for percent predicted forced vital capacity (FVC) and/or 6-minute walk test (6MWT); **AND**
- Females of reproductive potential must have a negative pregnancy test prior to initiating therapy and will use effective methods of contraception during treatment; **AND**
- Patient 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**
- Patients 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**
- Patient 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 variants in the *GAA* gene by molecular genetic testing; **AND**

- Patient 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]

Coverage can be renewed based on the following criteria:

- Patient 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**
- Patient has demonstrated a beneficial response to therapy compared to pretreatment baseline as indicated by stabilization or improvement in FVC and/or 6-MWT; **AND**
- Patient 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. - Patients 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 2025.
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. Leslie N, Bailey L. Pompe Disease. GeneReviews. www.ncbi.nlm.nih.gov/books/NBK1261/. Initial Posting: August 31, 2007; Last Update: November 2, 2023. Accessed on March 13, 2025.
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 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

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
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