Reference number(s)
6203-A

Standard Medicare Part B Management Pombiliti

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Pombiliti	cipaglucosidase alfa-atga

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

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 greater than or equal to 40 kg and who are not improving on their current enzyme replacement therapy (ERT).

All other indications will be assessed on an individual basis. Submissions for indications other than those listed in this criteria should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions:

- Initial requests: acid alpha-glucosidase enzyme assay or genetic testing results supporting diagnosis.
- Continuation or therapy: chart notes documenting a positive response to therapy.

© 2026 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.

Reference number(s)
6203-A

Coverage Criteria

Late-onset Pompe disease[1]

Authorization of 12 months may be granted for treatment of late-onset Pompe disease when all of the following criteria are met:

- Member is 18 years of age or older.
- Member weighs greater than or equal to 40 kg.
- Diagnosis was confirmed by enzyme assay demonstrating a deficiency of acid alpha-glucosidase enzyme activity or by genetic testing.
- Member is not improving on current enzyme replacement therapy (ERT) (e.g., Lumizyme, Nexviazyme).
- The requested medication will be taken in combination with Opfolda (miglustat).

Continuation of Therapy

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization of 12 months may be granted when all of the following criteria are met:

- The member is currently receiving therapy with the requested medication.
- The requested medication is being used to treat an indication in the coverage criteria section.
- The member is receiving benefit from therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, or muscle strength).
- The requested medication will be taken in combination with Opfolda (miglustat).

Summary of Evidence

The contents of this policy were created after examining the following resources:

- The prescribing information for Pombiliti
- The available compendium
    - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    - Micromedex DrugDex
    - American Hospital Formulary Service-Drug Information (AHFS-DI)
    - Lexi-Drugs
    - Clinical Pharmacology
- Pompe Disease, Gene Reviews article.

© 2026 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Pombiliti are covered.

Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for using enzyme assay and genetic testing to diagnose Pompe disease can be found in a Gene Reviews article. The diagnosis of Pompe disease is established in a patient with either deficiency of acid alpha-glucosidase enzyme activity or biallelic pathogenic variants in GAA on molecular genetic testing.

References

1. Pombiliti [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; July 2024.
2. Sperry E, Leslie N, Berry L, et al. Pompe Disease. 2007 Aug 31 [Updated 2025 Aug 21]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025.

© 2026 CVS Caremark. All rights reserved.

This document contains confidential and proprietary information of CVS Caremark and cannot be reproduced, distributed or printed without written permission from CVS Caremark. This document contains prescription brand name drugs that are trademarks or registered trademarks of pharmaceutical manufacturers that are not affiliated with CVS Caremark.