

STANDARD MEDICARE PART B MANAGEMENT

POMBILITI (cipaglucosidase alfa-atga)

POLICY

I. 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 Indication

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 enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

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

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

III. CRITERIA FOR INITIAL APPROVAL

Late-onset Pompe disease

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

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

IV. 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:

- A. The member is currently receiving therapy with the requested medication.
- B. The requested medication is being used to treat an indication enumerated in Section III.

| Reference number(s) |
|---------------------|
| 6203-A |

- C. 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).

V. SUMMARY OF EVIDENCE

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

1. The prescribing information for Pombiliti.
2. The available compendium
 - a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
 - b. Micromedex DrugDex
 - c. American Hospital Formulary Service- Drug Information (AHFS-DI)
 - d. Lexi-Drugs
 - e. Clinical Pharmacology
3. Glycogen Storage Disease Type II (Pompe Disease), Gene Reviews article

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

VI. EXPLANATION OF RATIONALE

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

Support for using enzyme assays 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.

VII. REFERENCES

1. Pombiliti [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; September 2023.
2. Leslie N, Tinkle BT. Glycogen Storage Disease Type II (Pompe Disease). GeneReviews 2013 May 9.