

Reference number(s)
6204-A

SPECIALTY GUIDELINE 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 are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

- A. Initial requests: acid alpha-glucosidase enzyme assay or genetic testing results supporting diagnosis.
- B. Continuation requests: chart notes documenting a positive response to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, muscle strength).

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. Member is not improving on current enzyme replacement therapy (ERT) (e.g., Lumizyme, Nexviazyme).
- E. The requested medication will be taken in combination with Opfolda (miglustat).

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III when both of the following criteria are met:

- A. Member is responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, or muscle strength).
- B. The requested medication will be taken in combination with Opfolda (miglustat).

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V. REFERENCES

1. Pombiliti [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; September 2023.