

POMBILITI (cipaglucosidase alfa-atga)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Pombiliti (cipaglucosidase alfa-atga) is indicated for late-onset Pompe disease, a rare genetic disorder. In Pompe disease, a gene mutation prevents the body from making an enzyme or making enough of the enzyme called acid alpha-glucosidase (GAA), necessary for proper muscle function. GAA is used by the heart and muscle cells to convert stored glycogen into energy. Without sufficient enzyme action, glycogen builds up in the cells, ultimately weakening the heart and other muscles. Infusion of Pombiliti replaces the deficient GAA, reducing the accumulated glycogen in the body (1).

Regulatory Status

FDA-approved indication: Pombiliti is a hydrolytic lysosomal glycogen-specific enzyme indicated, in combination with Opfolda, an enzyme stabilizer, for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing ≥ 40 kg and who are not improving on their current enzyme replacement therapy (ERT) (1).

Pombiliti has boxed warnings for severe hypersensitivity reactions, infusion-associated reactions (IARs), and risk of acute cardiorespiratory failure in susceptible patients: (1)

- Severe hypersensitivity reactions, including anaphylaxis may occur. In severe hypersensitivity reactions (e.g., anaphylaxis), Pombiliti should be discontinued immediately, and appropriate treatment initiated.
- Infusion-associated reactions (IARs) have also been reported to occur at any time during infusion and/or up to a few hours after the infusion has concluded. In severe IARs, immediate discontinuation of Pombiliti and appropriate care should be provided. In mild to moderate IARs, rechallenging with slower infusion rates or temporarily stopping the infusion have been shown to reduce symptoms.
- Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function for whom fluid restriction is indicated may be at risk of serious exacerbation of their cardiac or respiratory status during Pombiliti infusion. Vitals should be monitored more frequently in this population, and some patients may require prolonged observation times.

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Pombiliti may cause embryo-fetal harm. Females of reproductive potential should use effective contraception during treatment and for at least 60 days after the last dose (1).

The safety and effectiveness of Pombiliti in pediatric patients less than 18 year of age have not been established (1).

Summary

Pombiliti (cipaglucosidase alfa-atga) is a lysosomal glycogen-specific enzyme indicated for adults with late-onset Pompe disease (acid α -glucosidase [GAA] deficiency). Pombiliti has a boxed warning that hypersensitivity reactions, infusion-associated reactions and cardiorespiratory failure in susceptible patients has been observed during and after infusions. Appropriate medical support should be available during infusion and some patients may require prolonged observation time after infusion has concluded. The safety and effectiveness of Pombiliti in pediatric patients less than 18 years of age have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Pombiliti while maintaining optimal therapeutic outcomes.

References

1. Pombiliti [package insert]. Philadelphia, PA: Amicus Therapeutics, Inc.; July 2024.