

Pombiliti™ (cipaglucosidase alfa-atga)

When requesting Pombiliti™ (cipaglucosidase alfa-atga), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

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).

Coverage Guidelines

Late-Onset Pompe Disease

The individual must meet all of the following criteria for authorization:

- The diagnosis of late-onset acid alpha-glucosidase deficiency (late-onset Pompe disease) was established by one of the following:
 - Has a laboratory test demonstrating deficient acid alpha-glucosidase activity in blood, fibroblasts, or muscle tissue; OR
 - Has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic acid alpha-glucosidase gene variants;
- Is 18 years of age or older;
- Weighs 40 kg or greater;
- Pombiliti will be used in combination with Opfolda;
- Has not demonstrated an improvement in objective measures (e.g., forced vital capacity or six-minute walk test) after receiving Lumizyme (alglucosidase alfa) OR Nexviazyme (avalglucosidase alfa-ngpt) for at least one year;
- Pombiliti is prescribed by or in consultation with one of the following specialists: a geneticist, neurologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Approval duration: 12 months

Dosing Recommendation

The recommended dosage is 20 mg/kg (actual body weight) administered every other week as an intravenous infusion.

References

1. Pombiliti® intravenous infusion [prescribing information]. Philadelphia, PA: Amicus; September 2023.
2. Chien YH, Hwu WL, Lee NC. Pompe disease: Early diagnosis and early treatment make a difference. *Pediatr Neonatol.* 2013;54:219-227.
3. Llerena Junior JC, Nascimento OJM, Oliveira ASB, et al. Guidelines for the diagnosis, treatment and clinical monitoring of patients with juvenile and adult Pompe disease. *Arq Neuropsiquiatr.* 2016;74:166-176.
4. Cupler EJ, Berger KI, Leshner RT, et al. Consensus treatment recommendations for late-onset Pompe disease. *Muscle Nerve.* 2012;45:319-333.