

PRIOR AUTHORIZATION POLICY

POLICY: Pompe Disease – Enzyme Replacement Therapy – Pombiliti Prior Authorization Policy

- Pombiliti® (cipaglucosidase alfa-atga intravenous infusion – Amicus)

REVIEW DATE: 05/08/2024

OVERVIEW

Pombiliti, a hydrolytic lysosomal glycogen-specific recombinant human α -glucosidase enzyme, is indicated in combination with Opfolda® (miglustat capsules), an enzyme stabilizer, for **late-onset Pompe disease** (lysosomal acid α -glucosidase deficiency) in adults weighing ≥ 40 kg who are not improving on their current enzyme replacement therapy.¹

Disease Overview

Pompe disease (glycogen storage disease type II, or acid maltase deficiency), is a rare lysosomal storage disorder characterized by a deficiency in acid α -glucosidase activity leading to the accumulation of glycogen, particularly in muscle.^{2,3} The onset, progression, and severity of Pompe disease is variable. Infantile-onset Pompe disease usually manifests in the first few months of life and death often occurs in the first year of life, if left untreated.² Clinical manifestations of infantile-onset Pompe disease includes hypotonia, difficulty feeding, and cardiopulmonary failure.⁴ Late-onset Pompe disease has a more variable clinical course and can manifest any time after 12 months of age.^{3,4} Patients typically present with progressive muscle weakness which can progress to respiratory insufficiency. The diagnosis of Pompe disease is established by demonstrating decreased acid α -glucosidase activity in blood, fibroblasts, or muscle tissue; or by genetic testing.

POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of Pombiliti. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with Pombiliti as well as the monitoring required for adverse events and long-term efficacy, approval requires Pombiliti to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Pombiliti is recommended in those who meet the following criteria:

FDA-Approved Indication

- 1. Acid Alpha-Glucosidase Deficiency (Pompe Disease).** Approve for 1 year if the patient meets ALL of the following (A, B, C, D, E, and F):
 - A) Patient is ≥ 18 year of age; AND
 - B) Patient weighs ≥ 40 kg; AND
 - C) The medication will be used in combination with Opfolda (miglustat capsules); AND
 - D) Patient has not demonstrated an improvement in objective measures after receiving ONE of the following for at least one year (i or ii):

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Note: Examples of objective measures include forced vital capacity (FVC) and six-minute walk test (6MWT).

- i. Lumizyme (alglucosidase alfa intravenous infusion); OR
 - ii. Nexviazyme (avalglucosidase alfa-ngpt intravenous infusion); AND
- E) Patient has late-onset acid alpha-glucosidase deficiency (late-onset Pompe disease) with diagnosis established by ONE of the following (i or ii):
- i. Patient has a laboratory test demonstrating deficient acid alpha-glucosidase activity in blood, fibroblasts, or muscle tissue; OR
 - ii. Patient has a molecular genetic test demonstrating biallelic pathogenic or likely pathogenic acid alpha-glucosidase (GAA) gene variants; AND
- F) The medication is prescribed by or in consultation with a geneticist, neurologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Coverage of Pombiliti is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

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.