

Specialty Guideline Management

Lumizyme

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Lumizyme	alglucosidase alfa

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 Indications¹

Lumizyme is indicated for patients with Pompe disease (acid alpha-glucosidase [GAA] deficiency).

All other indications are considered experimental/investigational and not medically necessary.

Documentation

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

Initial Requests:

- Acid alpha-glucosidase enzyme assay reflecting deficiency of enzyme activity (less than 1% of normal controls for infantile-onset Pompe Disease and less than 40% of normal controls for late-onset Pompe Disease) or genetic testing results supporting diagnosis.

Reference number(s)
2055-A

- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline for late-onset Pompe Disease (e.g., progressive proximal muscle weakness, respiratory insufficiency, cardiomyopathy).

Continuation Requests:

- Chart notes or medical records documenting a positive response to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, cardiorespiratory function, decrease in left ventricular mass index [LVMI], delay in death).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Pompe Disease¹⁻²

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

- Diagnosis of Pompe disease was confirmed by enzyme assay demonstrating a deficiency of acid alpha-glucosidase enzyme activity (less than 1% of normal controls for infantile-onset Pompe Disease and less than 40% of normal controls for late-onset Pompe Disease) or by genetic testing.
- Member exhibits clinical signs and symptoms of disease at baseline for late-onset Pompe Disease (e.g. progressive proximal muscle weakness, respiratory insufficiency, cardiomyopathy).

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria who are responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, cardiorespiratory function, decrease in left ventricular mass index [LVMI], delay in death).

References

1. Lumizyme [package insert]. Cambridge, MA: Genzyme Corporation; December 2024.
2. Sperry E, Leslie N, Berry L, et al. Pompe Disease. 2007 Aug 31 [Updated 2025 Aug 21]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK1261/>