

PHARMACY POLICY STATEMENT

North Carolina Marketplace

DRUG NAME	Lumizyme (alglucosidase alfa)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Lumizyme is an enzyme replacement therapy for the treatment of Pompe disease, also known as acid alpha-glucosidase (GAA) deficiency or glycogen storage disease type II. Pompe disease is a rare, genetic lysosomal storage disorder that results in the buildup of glycogen in cell lysosomes causing serious and life-threatening muscle damage and weakness. Lumizyme replaces the deficient GAA enzyme to reduce the glycogen accumulation.

Pompe disease can be broadly classified as infantile-onset within the first few months of life (IOPD) or late-onset beyond infancy (LOPD). Classic IOPD is rapidly progressive with severe cardiomyopathy. Non-classic IOPD progresses slower with less severe cardiomyopathy. LOPD does not typically present with cardiomyopathy and has more variable symptoms, especially skeletal muscle weakness.

Lumizyme (alglucosidase alfa) will be considered for coverage when the following criteria are met:

Pompe disease (acid α -glucosidase [GAA] deficiency)

For **initial** authorization:

1. Medication must be prescribed by or in consultation with a geneticist, cardiologist, neurologist, pulmonologist, or metabolic specialist; AND
2. Member has a diagnosis of Pompe disease confirmed by an enzyme activity assay showing GAA deficiency (0% to 40% of normal); AND
3. Molecular genetic testing shows pathogenic mutation of the GAA gene; AND
4. Members with late onset Pompe disease must show signs or symptoms (i.e., motor weakness, reduced respiratory parameters).
5. **Dosage allowed/Quantity limit:** 20 mg/kg IV infusion every 2 weeks

If all the above requirements are met, the medication will be approved for 12 months.

For **reauthorization**:

1. Chart notes must document positive clinical response such as improved or stabilized motor function or ambulation, pulmonary function, or cardiomyopathy.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Lumizyme (alglucosidase alfa) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
07/07/2021	New policy for Lumizyme created.
11/09/2022	Annual review; no changes.
11/22/2023	Annual review; no changes.

References:

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3. Schoser B, Stewart A, Kanters S, et al. Survival and long-term outcomes in late-onset Pompe disease following alglucosidase alfa treatment: a systematic review and meta-analysis. *J Neurol.* 2017;264(4):621-630. doi:10.1007/s00415-016-8219-8
4. Wang RY, Bodamer OA, Watson MS, Wilcox WR; ACMG Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genet Med.* 2011;13(5):457-484. doi:10.1097/GIM.0b013e318211a7e1
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6. van der Ploeg AT, Kruijshaar ME, Toscano A, et al. European consensus for starting and stopping enzyme replacement therapy in adult patients with Pompe disease: a 10-year experience. *Eur J Neurol.* 2017;24(6):768-e31. doi:10.1111/ene.13285
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8. Burton BK, Kronn DF, Hwu WL, Kishnani PS; Pompe Disease Newborn Screening Working Group. The Initial Evaluation of Patients After Positive Newborn Screening: Recommended Algorithms Leading to a Confirmed Diagnosis of Pompe Disease. *Pediatrics.* 2017;140(Suppl 1):S14-S23. doi:10.1542/peds.2016-0280D
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10. Marques JS. The Clinical Management of Pompe Disease: A Pediatric Perspective. *Children (Basel).* 2022;9(9):1404. Published 2022 Sep 16. doi:10.3390/children9091404

Effective date: 04/01/2024

Revised date: 11/22/2023