alglucosidase alfa (Lumizyme)

Commercial Medical Benefit Drug Policy

Place of Service
Home Infusion Administration
Infusion Center Administration
Office Administration

Drug Details

USP Category: GENETIC OR ENZYME OR PROTEIN DISORDER: REPLACEMENT, MODIFIERS, TREATMENT

Mechanism of Action: human enzyme acid α-glucosidase (GAA)

HCPCS:
J0221:Injection, alglucosidase alfa, (lumizyme), 10 mg

How Supplied:
50 mg vial, as lyophilized powder (single-use)

Condition(s) listed in policy *(see coverage criteria for details)*

- Infantile or Late-Onset Pompe Disease

Any condition not listed in this policy requires a review to confirm it is medically necessary. For conditions that have not been approved for intended use by the Food and Drug Administration (i.e., off-label use), the criteria outlined in the Health and Safety Code section 1367.21 must be met.

Special Instructions and Pertinent Information

Provider must submit documentation (such as office chart notes, lab results or other clinical information) to ensure the member has met all medical necessity requirements.

The member's specific benefit may impact drug coverage. Other utilization management processes, and/or legal restrictions may take precedence over the application of this clinical criteria.

For billing purposes, drugs must be submitted with the drug's assigned HCPCS code (as listed in the drug policy) and the corresponding NDC (national drug code). An unlisted, unspecified, or miscellaneous code should not be used if there is a specific code assigned to the drug.

Members with the following plans: **PPO, Direct Contract HMO, and when applicable, ASO, Shared Advantage, HMO (non-direct)** may be required to have their medication administered at a preferred site of service, including the home, a physician's office, or an independent infusion center not associated with a hospital.

For members that cannot receive infusions in the preferred home or ambulatory setting AND meet one of the following criteria points, drug administration may be performed at a hospital outpatient facility infusion center.

CRITERIA FOR HOSPITAL OUTPATIENT FACILITY ADMINISTRATION

MCG Care Guidelines, 19th edition, 2015

ADMINISTRATION OF THIS DRUG IN THE HOSPITAL OUTPATIENT FACILITY SITE OF CARE REQUIRES ONE OF THE FOLLOWING: (*Supporting Documentation must be submitted)*

1. Patient is initiating therapy (allowed for the first 2 infusions) of Lumizyme or is being re-initiated on Lumizyme after at least 6 months off therapy. *Subsequent doses will require medical necessity for continued use in the hospital outpatient facility site of care.*

OR

Additional clinical monitoring is required during administration as evidenced by one of the following:

2. Patient has experienced a previous severe adverse event on Lumizyme based on documentation submitted.
3. Patient continues to experience moderate to severe adverse events on Lumizyme based on documentation submitted, despite receiving premedication such as acetaminophen, steroids, diphenhydramine, fluids, etc.
4. Patient is clinically unstable based on documentation submitted.
5. Patient is physically or cognitively unstable based on documentation submitted.

Coverage Criteria
The following condition(s) require Prior Authorization/Preservice.

Infantile or Late-Onset Pompe Disease

Meets medical necessity if all the following are met:

1. Diagnosis is infantile or late-onset Pompe Disease (also known as Glycogen Storage Disease Type II or acid maltase deficiency)
2. Meets ONE of the following:
   a. Genetic testing showing acid alpha-glucosidase (GAA) mutation
   b. An enzyme assay showing absent or decreased acid alpha-glucosidase (GAA) activity in blood, skin or muscle tissues

Covered Doses:
Up to 20 mg/kg given intravenously every 2 weeks

Coverage Period:
Through 9/30/2025: Indefinitely

Effective 10/1/2025 and after:
Initial: 1 year
Reauthorization: Yearly if there is continued benefit from therapy

ICD-10:
E74.02

References

1. AHFS. Available by subscription at http://www.lexi.com
2. DrugDex. Available by subscription at http://www.micromedexsolutions.com/home/dispatch
3. Lumizyme (alglucosidase alfa) Prescribing Information. Genzyme Corporation, Cambridge, MA: 12/2024.
4. The American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) Consensus Treatment Recommendations for Late-Onset Pompe Disease Muscle Nerve 2012 Mar 45(3): 319-333. Available at https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3534745/.
5. Stevens D, Milani-Nejad S, Mozaffar T. Pompe Disease: a Clinical, Diagnostic, and Therapeutic Overview. *Curr Treat Options Neurol*. 2022 Nov;24(11):573-588. Epub 2022 Aug 4. Available at https://www.ncbi.nlm.nih.gov/pmc/articles/PMC10035871/pdf/nihms-1847668.pdf.

Review History

Date of Last Annual Review: 3Q2025
Changes from previous policy version:

- Pompe Disease: Updated Coverage Period to require yearly reauthorization based on response to drug

Blue Shield of California Medication Policy to Determine Medical Necessity
Reviewed by P&T Committee