Clinical Policy: Alglucosidase Alfa (Lumizyme)

Reference Number: PA.CP.PHAR.160
Effective Date: 01/2018
Last Review Date: 01/2026

Description
Alglucosidase alfa (Lumizyme®) is a hydrolytic lysosomal glycogen-specific enzyme.

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

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness that Lumizyme is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Pompe Disease (must meet all):

1. Diagnosis of Pompe disease (acid alpha-glucosidase [GAA] deficiency) confirmed by one of the following:
   a. Enzyme assay confirming low GAA activity;
   b. DNA testing;
   c. Increased lysosomal glycogen;
2. Lumizyme is not prescribed concurrently with Nexviazyme™ or the combination of Pombiliti™ with Opfolda™;
3. Dose does not exceed 20 mg/kg every 2 weeks.

Approval duration: 12 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued Approval

A. Pompe Disease (must meet all)**:**

1. Currently receiving medication via PA Health & Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.PHARM.01) applies;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's Pompe disease manifestation profile (*see Appendix D for examples*);
3. Lumizyme is not prescribed concurrently with Nexviazyme or the combination of Pombiliti™ with Opfolda™;
4. If request is for a dose increase, new dose does not exceed 20 mg/kg every 2 weeks.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA. PHARM.01) applies; or
2. Refer to PA.CP.PMN.53

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

6MWT: 6 minute walk test
AIMS: Alberta Infant Motor Scale
FDA: Food and Drug Administration
GAA: acid alpha-glucosidase

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): none reported.
- Boxed warning(s): hypersensitivity reactions including anaphylaxis, immune-mediated reactions; risk of acute cardiorespiratory failure.

Appendix D: Measures of Therapeutic Response

- Pompe disease manifests as a clinical spectrum that varies with respect to age at onset*, rate of disease progression, and extent of organ involvement. Patients can present with a variety of signs and symptoms, which can include cardiomegaly, cardiomyopathy, hypotonia, muscle weakness, respiratory distress (eventually requiring assisted ventilation), and skeletal muscle dysfunction. In infantile-onset disease, death typically occurs in the first year of life.
- While there is not one generally applicable set of clinical criteria that can be used to determine appropriateness of continued therapy, clinical parameters that can indicate therapeutic response to Lumizyme include:
  - For infantile-onset disease: no invasive ventilator supported needed, gains in motor function as evidenced by the Alberta Infant Motor Scale (AIMS), continued survival;
  - For late-onset disease: improved or maintained forced vital capacity, improved or maintained 6 minute walk test (6MWT) distance.

**Although infantile-onset disease typically presents in the first year of life, age of onset alone does not necessarily distinguish between infantile- and late-onset disease since juvenile-onset disease can present prior to 12 months of age.*

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Pompe disease	20 mg/kg IV every 2 weeks	20 mg/kg/2 weeks

VI. Product Availability

Single-use vial: 50 mg

VII. References

1. Lumizyme Prescribing Information. Cambridge, MA: Genzyme Corporation; December 2024. Available at http://www.lumizyme.com. Accessed December 6, 2025.
2. Kishnani PS, Steiner RD, Bali D, et al. American College of Medical Genetics and Genomics (ACMG) Work Group on management of Pompe disease. Pompe disease diagnosis and management guideline. *Genet Med.* 2006; 8(5): 267-268.
3. Cupler EJ, Berger KI, Leshner RT, et al. Consensus treatment recommendations for late-onset Pompe disease. Muscle Nerve 2012;45:319-33.
4. Stevens D, Milani-Nejad S, Mozaffar T. Pompe disease: a clinical, diagnostic, and therapeutic overview. *Curr Treat Options Neurol*. 2022 November;24(11):573-88. doi:10.1007/s11940-022-00736-1.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J0220	Injection, alglucosidase alfa, 10 mg, not otherwise specified
J0221	Injection, alglucosidase alfa, (Lumizyme), 10 mg

Reviews, Revisions, and Approvals	Date
2Q 2018 annual review: Added max dose criteria. Added examples of what may constitute positive response to therapy. Added requirement for documentation of positive response to therapy for reauthorization; changed approval durations from length of benefit to 6/12 months; references reviewed and updated.	02/2018
2Q 2019 annual review: references reviewed and updated.	04/2019
2Q 2020 annual review: references reviewed and updated.	04/2020
2Q 2021 annual review: references reviewed and updated.	04/2021
2Q 2022 annual review: no significant changes; added requirement that Lumizyme not be prescribed concurrently with Nexviazyme; references reviewed and updated.	04/2022
2Q 2023 annual review: no significant changes; references reviewed and updated.	04/2023
2Q 2024 annual review: no significant changes; added exclusion for concomitant use with Pombiliti+Opfolda to align with the Pombiliti criteria; references reviewed and updated.	04/2024
1Q 2025 annual review: moving forward to 1Q annual review cycle to consolidate with the Opfolda+Pombiliti annual review cycle; added increased lysosomal glycogen as an additional option for confirming a Pompe disease diagnosis; references reviewed and updated.	01/2025

Reviews, Revisions, and Approvals	Date
1Q 2026 annual review: no significant changes; updated initial auth duration from 6 months to 12 months; references reviewed and updated.	01/2026