

Clinical Policy: Alglucosidase Alfa (Lumizyme)

Reference Number: LA.PHAR.160

Effective Date: 08.19.22

Last Review Date: 03.05.25

Line of Business: Medicaid

[Coding Implications](#)

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

****Please note: This policy is for medical benefit****

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 Louisiana Healthcare Connections 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 (GAA deficiency) confirmed by one of the following (a, b, or c):
 - 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: 6 months

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to LA.PMN.255
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy LA.PMN.53

II. Continued Therapy

A. Pompe Disease (must meet all):

1. Member is currently receiving medication via Louisiana Healthcare Connections benefit or member has previously met initial approval criteria;
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. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to LA.PMN.255
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy LA.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

- A.** Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policy LA.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; March 2024. Available at <http://www.lumizyme.com>. Accessed December 2, 2024.
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 | LDH Approval Date |
|---|----------|-------------------|
| Converted corporate to local policy. | 07.22 | 08.19.22 |
| Template changes applied to other diagnoses/indications and continued therapy section. References reviewed and updated. | 06.02.23 | 10.05.23 |

| Reviews, Revisions, and Approvals | Date | LDH Approval Date |
|--|----------|-------------------|
| Added verbiage this policy is for medical benefit only. | | |
| Annual review: added exclusion for concomitant use with Pombiliti+Opfolda; references reviewed and updated. | 05.07.24 | 08.20.24 |
| Annual review; added increased lysosomal glycogen as an additional option for confirming a Pompe disease diagnosis; references reviewed and updated. | 03.05.25 | |

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. LHCC makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable LHCC administrative policies and procedures.

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