Clinical Policy: Alglucosidase Alfa (Lumizyme)

Reference Number: CP.PHAR.160
Effective Date: 02.01.16
Last Review Date: 02.25
Line of Business: Commercial, HIM, Medicaid

Coding Implications
Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

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 health plans affiliated with Centene Corporation® 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:
Medicaid/HIM – 6 months
Commercial – 6 months or to the member's renewal date, whichever is longer

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 one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or

b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or

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 for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

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

1. Member meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B);*
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:
Medicaid/HIM – 12 months
Commercial – 6 months or to the member's renewal date, whichever is longer

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 one of the following policies (a or b):
   a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
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 for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

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 – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid, or evidence of coverage documents.

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	P&T Approval Date
2Q 2021 annual review: no significant changes; references reviewed and updated.	02.09.21	05.21
2Q 2022 annual review: no significant changes; added requirement that Lumizyme not be prescribed concurrently with Nexviazyme; references reviewed and updated.	02.14.22	05.22
Template changes applied to other diagnoses/indications and continued therapy section.	09.30.22	
2Q 2023 annual review: no significant changes; references reviewed and updated.	02.07.23	05.23
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.	01.09.24	05.24
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.	12.02.24	02.25

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. The Health Plan 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. "Health Plan" means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan's affiliates, as applicable.

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 Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members

and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note: For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.