Clinical Policy: Alglucosidase Alfa (Lumizyme)

Reference Number: LA.PHAR.160
Effective Date:
Last Review Date: 07.22 Coding Implications
Line of Business: Medicaid 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

Prior Authorization is required. 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 or b):**
   a. **Enzyme assay confirming low GAA activity;**
   b. **DNA testing;**
2. **Lumizyme is not prescribed concurrently with Nexviazyme™;**
3. **Dose does not exceed 20 mg/kg every 2 weeks.**

Approval duration:
Medicaid – 6 months

B. Other diagnoses/indications

1. **Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): LA.PMN.53 for Medicaid.**

II. Continued Therapy

A. Pompe Disease (must meet all):

1. **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 ;**
4. **If request is for a dose increase, new dose does not exceed 20 mg/kg every 2 weeks.**

Approval duration:
Medicaid – 12 months

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

1. **Currently receiving medication via Louisiana Healthcare Connections benefit and documentation supports positive response to therapy.**
   Approval duration: Duration of request or 6 months (whichever is less); or
2. **Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): LA.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.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): risk of anaphylaxis, hypersensitivity, and immune-mediated reactions to Lumizyme infusions; risk of 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; February 2020. Available at http://www.lumizyme.com. Accessed February 14, 2022.
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.

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	

Important Reminder

Alglucosidase Alfa

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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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.

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