

## **Clinical Policy: Alglucosidase Alfa (Lumizyme)**

Reference Number: CP.PHAR.160

Effective Date: 02.01.16

Last Review Date: 05.18

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<sup>®</sup>) 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<sup>®</sup> 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. Dose does not exceed 20 mg/kg every 2 weeks.

**Approval duration: 6 months**

### **B. Other diagnoses/indications**

1. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PHAR.21 for health insurance marketplace, and CP.PMN.53 for Medicaid.

## **II. Continued Therapy**

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

1. Currently receiving medication via Centene 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 C for examples*);
3. 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 health plan 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 for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PHAR.21 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.PHAR.21 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: 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.

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*\*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**

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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; August 2014. Available at <http://www.lumizyme.com>. Accessed February 27, 2018.
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.

#### 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
Policy split from CP.PHAR.48. Policy converted to new template.	01.16	02.16
Age restriction removed; Positive response to therapy added; Background section converted to new template; Lumizyme PI remains the same; Myozyme is no longer available in the U.S.	12.16	02.17
Added max dose criteria. Added examples of what may constitute positive response to therapy.	08.24.17	11.17
2Q 2018 annual review: no significant changes from previously approved corporate policy; policies combined for Commercial and Medicaid lines of business; HIM added; Commercial: removed Myozyme from the policy as it is no longer available in the U.S.; added diagnosis confirmation testing requirement; 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.	02.27.18	05.18

#### 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

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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.

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**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.

**For Health Insurance Marketplace members,** when applicable, this policy applies only when the prescribed agent is on your health plan approved formulary. Request for non-formulary drugs must be reviewed using the formulary exception policy.

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