Prior Authorization Review Panel

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CHC-MCO Policy Submission

A separate copy of this form must accompany each policy submitted for review.
Policies submitted without this form will not be considered for review.

Plan: PA Health & Wellness	**Submission Date: 05/01/2021**
Policy Number: PA.CP.PHAR.160	**Effective Date: 01/2018** **Revision Date: 04/2021**
Policy Name: Alglucosidase Alfa (Lumizyme)	

Type of Submission – Check all that apply:

- ☐ **New Policy**
- ✓ **Revised Policy***
- ☐ **Annual Review - No Revisions**
- ☐ **Statewide PDL -** *Select this box when submitting policies for Statewide PDL implementation and when submitting policies for drug classes included on the Statewide PDL.*

***All revisions to the policy must be highlighted using track changes throughout the document.**

Please provide any changes or clarifying information for the policy below:

2Q 2021 annual review: references reviewed and updated.

Name of Authorized Individual (Please type or print): **Auren Weinberg, MD**	**Signature of Authorized Individual:** 

Clinical Policy: Alglucosidase Alfa (Lumizyme)

Reference Number: PA.CP.PHAR.160
Effective Date: 01/18
Last Review Date: 04/2021

Coding Implications
Revision Log

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 Pennsylvania Health and 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;
2. Dose does not exceed 20 mg/kg every 2 weeks.

Approval duration: 6 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 Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.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. 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 Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.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): 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 9, 2021.
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	Approval 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.	02.27.18	
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	