Clinical Policy: Alglucosidase Alfa (Lumizyme)

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

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for alglucosidase alfa (Lumizyme®).

FDA Approved Indication(s)
Lumizyme is indicated for patients with Pompe disease (acid alpha-glucosidase [GAA]) deficiency.

Policy/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.
   3.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PHAR.57 - Global Biopharm Policy.

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

Approval duration: 12 months

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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.PHAR.57 - Global Biopharm Policy**.**

Background

Description/Mechanism of Action:

Pompe disease (acid maltase deficiency, glycogen storage disease type II, glycogenosis type II) is an inherited disorder of glycogen caused by the absence or marked deficiency of the lysosomal enzyme GAA. Alglucosidase alfa provides an exogenous source of GAA. Binding to mannose-6-phosphate receptors on the cell surface has been shown to occur via carbohydrate groups on the GAA molecule, after which it is internalized and transported into lysosomes, where it undergoes proteolytic cleavage that results in increased enzymatic activity. It then exerts enzymatic activity in cleaving glycogen.

Formulations:

Lumizyme (alglucosidase alfa): Lyophilized product for reconstitution; for intravenous use

- 50 mg/10 mL vial; 5 mg/mL (3.6 to 5.4 units/mg)

Appendices

Appendix A: Abbreviation Key

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:

a. For infantile-onset disease: no invasive ventilator supported needed, gains in motor function as evidenced by the Alberta Infant Motor Scale (AIMS), continued survival;
b. 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.*

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	

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.