RXADVANCE
2 Park Central Drive
Southborough, MA 01772

Clinical Policy Title:	alglucosidase alfa
Policy Number:	RxA.608
Drug(s) Applied:	Lumizyme®
Original Policy Date:	03/06/2020
Last Review Date:	12/07/2020
Line of Business Policy Applies to:	All lines of business

Background

Alglucosidase alfa (Lumizyme®) is a hydrolytic lysosomal glycogen-specific enzyme. It is indicated for patients with Pompe disease (acid alpha-glucosidase [GAA]) deficiency.

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
alglucosidase alfa (Lumizyme®)	Pompe disease	20 mg/kg IV every 2 weeks	20 mg/kg for 2 weeks

Dosage Forms

- Single-use vial: 50 mg

Clinical Policy

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

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. Prescribed by or in consultation with a metabolic specialist or biochemical geneticist;
3. For late-onset Pompe disease only, genetic testing to identify the specific mutation to confirm the diagnosis;
4. Force vital capacity (FVC) 30-79% of predicted value and ability to walk 40 meters on a 6-minute walk test (assistive devices permitted);
5. Muscle weakness in the lower extremities;
6. Dose does not exceed 20 mg per kg every 2 weeks.

Approval Duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

A. Pompe Disease (must meet all):

1. Member is currently receiving medication that has been authorized by RxAdvance or the member has met initial approval criteria listed in this policy;
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 per kg every 2 weeks.

Approval Duration
Commercial: 6 months
Medicaid: 12 months

III. Appendices

APPENDIX A: Abbreviation/Acronym Key
6MWT: 6-minute walk test
AIMS: Alberta Infant Motor Scale
FDA: Food and Drug Administration
GAA: acid alpha-glucosidase
FVC: Force vital capacity

APPENDIX B: Therapeutic Alternatives
Not applicable.

APPENDIX C: Contraindications/Boxed Warnings

- Contraindication(s):
  - None.

- Boxed Warning(s):
  - Risk of anaphylaxis, hypersensitivity, and immune-mediated reactions;
  - Risk of cardiorespiratory failure.

APPENDIX D: General Information
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.

References

1. Lumizyme Prescribing Information. Cambridge, MA: Genzyme Corporation; February 2020. Available at http://www.lumizyme.com. Accessed October 08, 2020.
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. Accessed October 08, 2020.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	03/2020	03/06/2020
Policy was reviewed: 1. Clinical policy title was updated as "alglucosidase alfa'. 2. Lines of business policy applies to was updated to all lines of business. 3. Initial approval criteria updated "FVC, late-onset Pompe disease and prescribed info added". 4. Continued therapy approval criteria II.A.1 was rephrased to "Member is currently receiving medication that has been authorized by RxAdvance…". 5. Appendices updated. 6. References were reviewed and updated.	10/08/2020	12/07/2020