Effective Date: 04/01/2022
Last Reviewed: 12/2021, 6/2022, 2/2023, 01/2024 Pharmacy Scope: Medicaid Medical Scope: Commercial, Medicare-Medicaid Plan (MMP)

Lumizyme® (alglucosidase alfa)
(Intravenous)

***Effective 04/01/2022 – Medication only available on the Pharmacy Benefit for Medicaid Members ONLY**

Policy Statement:

Lumizyme (alglucosidase alfa) is covered under the Pharmacy Benefit for Medicaid members and covered under the Medical Benefit for Commercial and MMP members when used within the following guidelines. Use outside of these guidelines may result in non-payment unless approved under an exception process.

I. Length of Authorization

Coverage will be provided for 6 months and may be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- Lumizyme 50 mg vial: 46 vials every 14 days

B. Max Units (per dose and over time) [HCPCS Unit]:

- 230 billable units every 14 days

III. Initial Approval Criteria[1,4,7,8]

Coverage is provided in the following conditions:

- Documented baseline age-appropriate values for one or more of the following:
    - Infantile-onset disease: muscle weakness, motor function, respiratory function, cardiac involvement, percent predicted forced vital capacity (FVC), and/or 6-minute walk test (6-MWT); **OR**
    - Late-onset (non-infantile) disease: FVC and/or 6-MWT; **AND**

*****NOTE:** For very young patients in which FVC or 6-MWT are not suitable for measuring, requests will be reviewed on a case-by case basis.*

Universal Criteria

- Will not be used in combination with other enzyme replacement therapies (i.e., avalglucosidase-alfa); **AND**
- Patients susceptible to fluid volume overload or those with an acute underlying respiratory illness or compromised cardiac or respiratory function, will be closely monitored for exacerbation of their cardiac or respiratory status during infusion ; **AND**

Pompe disease (Acid alpha-glucosidase (GAA) deficiency) †

- Diagnosis has been confirmed by one of the following:
    - Deficiency of acid alpha-glucosidase (GAA) enzyme activity; **OR**
    - Detection of biallelic pathogenic variants in the GAA gene by molecular genetic testing; **AND**

† FDA approved indication(s)

IV. Renewal Criteria[1,4,7,8]

Authorizations can be renewed based on the following criteria:

- Patient continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: anaphylaxis and hypersensitivity reactions, immune-mediated cutaneous reactions, systemic immune-mediated reactions, acute cardiorespiratory failure, cardiac arrhythmia during general anesthesia, etc.; **AND**
- Patient is being monitored for antibody formation (including neutralizing antibodies); **AND**
- Patient has demonstrated a beneficial response to therapy compared to pretreatment age- appropriate baseline values in one or more of the following:
    - Infantile-onset disease: stabilization or improvement in muscle weakness, motor function, respiratory function, cardiac involvement, FVC, and/or 6MWT; OR
    - Late-onset (non-infantile) disease: stabilization or improvement in FVC and/or 6MWT

V. Dosage/Administration[1,7,8]

Indication	Dose
Pompe disease	20 mg/kg administered as an intravenous (IV) infusion every 2 weeks

VI. Billing Code/Availability Information

HCPCS Code:

- J0221 – Injection, alglucosidase alfa, (Lumizyme), 10 mg; 1 billable unit = 10 mg

NDC:

- Lumizyme 50 mg single-dose vial for injection: 58468-0160-xx

VII. References

1. Lumizyme [package insert]. Cambridge, MA; Genzyme Corporation.; April 2023 . Accessed November 2023.
2. Cupler EJ, Berger KI, Leshner RT, et al. Consensus treatment recommendations for late-onset Pompe disease. Muscle Nerve. 2012 Mar; 45(3):319-33. doi: 10.1002/mus.22329. Epub 2011 Dec 15.
3. Kishnani PS, Steiner RD, Bali D, et al. Pompe disease diagnosis and management guidelines. *Genet Med* 2006; 8:267-88.
4. Nancy L, Bailey L. Pompe Disease. GeneReviews. www.ncbi.nlm.nih.gov/books/NBK1261/ (Accessed on August 11, 2018).
5. Tarnopolsky M, Katzberg H, Petrof BJ, et al. Pompe Disease: Diagnosis and Management. Evidence-Based Guidelines from a Canadian Expert Panel. Can J Neurol Sci. 2016 Jul;43(4):472-85.
6. Kishnani PS, Hwu WL, et al. Introduction to the Newborn Screening, Diagnosis, and Treatment for Pompe Disease Guidance Supplement. Pediatrics 2017 Jul:(1):S1-S3.
7. van der Ploeg AT, Clemens PR, Corzo D, et al. A randomized study of alglucosidase alfa in late-onset Pompe's disease. N Engl J Med. 2010 Apr 15;362(15):1396-406. doi: 10.1056/NEJMoa0909859.
8. Nicolino M, Byrne B, Wraith JE, et al. Clinical outcomes after long-term treatment with alglucosidase alfa in infants and children with advanced Pompe disease. Genet Med. 2009 Mar;11(3):210-9. doi: 10.1097/GIM.0b013e31819d0996.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E74.02	Pompe disease