Effective Date: 04/01/2022
Last Reviewed: 12/2021, 6/2022, 2/2023, 05/2024, 02/2025 Pharmacy Scope: Medicaid Medical Scope: Commercial, Medicare

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 Medicare 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 for 6 months.

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 6-minute walk test (6-MWT); **OR**
    - Late-onset (non-infantile) disease: FVC and 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.*

Neighborhood Health Plan OF RHODE ISLAND™

Universal Criteria

- Will not be used in combination with other enzyme replacement therapies [i.e., avalglucosidase-alfa (Nexviazyme), or cipaglucosidase alfa-atga (Pombiliti)]; **AND**
- Patient has not experienced a severe hypersensitivity reaction including anaphylaxis to alglucosidase alfa (Lumizyme); **AND**
- Patient is not susceptible to fluid volume overload and does not have an acute underlying respiratory illness or compromised cardiac or respiratory function for whom fluid restriction is indicated; **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**

***Note: The diagnosis of Infantile-Onset Pompe Disease (IOPD) can be established rapidly after a positive newborn screening (NBS) result when physical examination, echocardiography, and elevated CPK support the diagnosis. It is recommended that the diagnosis be confirmed either by molecular genetic testing or by measurement of GAA activity in another tissue.[4]*

† 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 6MWT; OR
    - Late-onset (non-infantile) disease: stabilization or improvement in FVC and 6MWT

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

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

Effective Date: 04/01/2022
Last Reviewed: 12/2021, 6/2022, 2/2023, 05/2024, 02/2025 Pharmacy Scope: Medicaid Medical Scope: Commercial, Medicare

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; January 2025. Accessed February 2025.
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/. Initial Posting: August 31, 2007; Last Update: November 2, 2023.. Accessed on January 02, 2024.
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.
9. Sawada T, Kido J, Nakamura K. Newborn Screening for Pompe Disease. Int J Neonatal Screen. 2020 Jun; 6(2): 31.Published online 2020 Apr 5. doi: 10.3390/ijns6020031

Appendix 1 – Covered Diagnosis Codes

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