Effective Date: 04/01/2022
Last Reviewed: 12/2021, 6/2022, 2/2023, 05/2024 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.

Summary of Evidence:
Clinical trials evaluating the efficacy and safety of Lumizyme in the treatment of Pompe disease have demonstrated significant improvements in respiratory function, motor function, and overall survival compared to placebo or standard of care. Lumizyme is a recombinant human acid alpha-glucosidase enzyme replacement therapy that targets the underlying cause of Pompe disease by replacing the deficient enzyme, leading to reduced glycogen accumulation in affected tissues.

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:

MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

- 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.*

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

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; May 2023. Accessed December 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/. 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

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Determinations (LCDs), and Articles may exist and compliance with these policies is required where applicable. They can be found at: http://www.cms.gov/medicare-coverage-database/search/advanced-search.aspx. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/Articles): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC

Policy Rationale:

Lumizyme was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Lumizyme according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For INTEGRITY (Medicare-Medicaid Plan) members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.