Lumizyme® (alglucosidase alfa)
(Intravenous)

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I. Length of Authorization

Coverage will be provided for 12 months and may be renewed.

II. Dosing Limits

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

- Lumizyme 50 mg single-dose 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]

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 [1]

- Will not be used in combination with other enzyme replacement therapies (i.e., avalglucosidase-alfa); **AND**
- Patient has not experienced a severe hypersensitivity reaction including anaphylaxis to alglucosidase alfa; **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) † Φ [1,4]

- 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

† FDA approved indication(s); ‡ Compendia recommended indication(s); Φ Orphan Drug

IV. Renewal Criteria [1,4]

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

V. Dosage/Administration [1]

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.; February 2020. Accessed January 2022.

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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: May 11, 2017. Accessed on January 20, 2022.
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 Local Coverage Articles (LCAs)may exist and compliance with these policies is required where applicable. They can be found at: https://www.cms.gov/medicare-coverage-database/new-search/. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/LCA): 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)

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
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