



Lumizyme® (alglucosidase alfa) (Intravenous)

Document Number: IC-0079

Last Review Date: 02/02/2023

Date of Origin: 11/28/2011

Dates Reviewed: 12/2011, 02/2013, 02/2014, 08/2014, 07/2015, 10/2016, 10/2017, 10/2018, 02/2019, 02/2020, 02/2021, 09/2021, 02/2022, 02/2023

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, etc.); **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) † 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); Ⓢ Orphan Drug

IV. Renewal Criteria ^{1,4}

Coverage may be renewed based on the following criteria:

- Patient continues to meet the 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	Administer 20 mg/kg body weight 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 2022. Accessed January 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: May 11, 2017. Accessed on January 09, 2023.
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/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/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

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PCHP:

Provides free aids and services to people with disabilities to communicate effectively with us, such as:

- Qualified sign language interpreters
- Written information in other formats (large print, audio, accessible electronic formats, other formats)

Provides free language services to people whose primary language is not English, such as:

- Qualified interpreters
- Information written in other languages

If you need these services, contact a Grievance Specialist.

If you believe that PCHP has failed to provide these services or discriminated in another way on the basis of race, color, national origin, age, disability, or sex, you can file a grievance with:

Grievance Specialist
PreferredOne Community Health Plan
PO Box 59052
Minneapolis, MN 55459-0052
Phone: 1.800.940.5049 (TTY: 763.847.4013)
Fax: 763.847.4010
customerservice@preferredone.com

You can file a grievance in person or by mail, fax, or email. If you need help filing a grievance, a Grievance Specialist is available to help you.

You can also file a civil rights complaint with the U.S. Department of Health and Human Services, Office for Civil Rights, electronically through the Office for Civil Rights Complaint Portal, available at <https://ocrportal.hhs.gov/ocr/portal/lobby.jsf>, or by mail or phone at:

U.S. Department of Health and Human Services
200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
1-800-368-1019, 800-537-7697 (TDD)

Complaint forms are available at <http://www.hhs.gov/ocr/office/file/index.html>.

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ATENCIÓN: si habla español, tiene a su disposición servicios gratuitos de asistencia lingüística. Llame al 1.800.940.5049 (TTY: 763.847.4013).

LUS CEEV: Yog tias koj hais lus Hmoob, cov kev pab txog lus, muaj kev pab dawb rau koj. Hu rau 1.800.940.5049 (TTY: 763.847.4013).

XIYYEEFFANNAA: Afaan dubbattu Oroomiffa, taiaajiila qarqaarsa afaanii, kanfaltiidhaan ala, ni argama. Bilbilaa 1.800.940.5049 (TTY: 763.847.4013).

CHÚ Ý: Nếu bạn nói Tiếng Việt, có các dịch vụ hỗ trợ ngôn ngữ miễn phí dành cho bạn. Gọi số 1.800.940.5049 (TTY: 763.847.4013).

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200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
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