

Fabrazyme® (agalsidase beta) (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]:

- Fabrazyme 5 mg single-dose vial: 6 vials per 14 days
- Fabrazyme 35 mg single-dose vial: 3 vials per 14 days

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

- 115 billable units every 14 days

III. Initial Approval Criteria ¹

Coverage is provided in the following conditions:

- Patient is at least 2 years of age; **AND**

Universal Criteria

- Must not be used in combination with migalastat; **AND**

Fabry Disease (alpha-galactosidase A deficiency) † Φ ^{1,3,7,13}

- Documented diagnosis of Fabry disease with biochemical/genetic confirmation by one of the following:
 - α-galactosidase A (α-Gal A) activity in plasma, isolated leukocytes, and/or cultured cells (males only); **OR**
 - Plasma or urinary globotriaosylceramide (Gb₃/GL-3) or globotriaosylsphingosine (lyso-Gb₃); **OR**
 - Detection of pathogenic mutations in the *GLA* gene by molecular genetic testing; **AND**
- Baseline value for plasma GL-3 and/or GL-3 inclusions

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

IV. Renewal Criteria ¹

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 severe hypersensitivity reactions, severe infusion-associated reactions, compromised cardiac function, etc.; **AND**
- Disease response with treatment as defined by a reduction in plasma GL-3 and/or GL-3 inclusions compared to pre-treatment baseline

V. Dosage/Administration ¹

Indication	Dose
Fabry Disease	1 mg/kg of body weight infused every two weeks as an intravenous (IV) infusion.

VI. Billing Code/Availability Information

HCPCS Code:

- J0180 – Injection, agalsidase beta, 1 mg; 1 billable unit = 1 mg

NDC:

- Fabrazyme 5 mg single-dose vial for injection: 54868-0041-xx
- Fabrazyme 35 mg single-dose vial for injection: 54868-0040-xx

VII. References

1. Fabrazyme [package insert]. Cambridge, MA; Genzyme Corporation.; March 2021. Accessed January 2023.
2. Mehta A, Beck M, Eyskens F, et al. Fabry disease: a review of current management strategies. QJM. 2010 Sep; 103(9):641-59.
3. Mehta A, Hughes DA. Fabry Disease. In: Adam MP, Everman DB, Mirzaa GM, et al., editors. GeneReviews®. [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2023. Initial Posting: August 5, 2002; Last Update: January 27, 2022. Accessed on January 5, 2023. www.ncbi.nlm.nih.gov/books/NBK1292/.
4. Biegstraaten M, Arngrímsson R, Barbey F, et al. Recommendations for initiation and cessation of enzyme replacement therapy in patients with Fabry disease: the European Fabry Working Group consensus document. Orphanet J Rare Dis. 2015 Mar 27;10:36.

5. Hopkin RJ, Jefferies JL, Laney DA, et al. The management and treatment of children with Fabry disease: A United States-based perspective. *Mol Genet Metab*. 2016 Feb;117(2):104-13.
6. Laney DA, Bennett RL, Clarke V, et al. Fabry disease practice guidelines: recommendations of the National Society of Genetic Counselors. *J Genet Couns*. 2013 Oct;22(5):555-64.
7. Kes VB, Cesarik M, Zavoreo I, et al. Guidelines for diagnosis, therapy and follow up of Anderson-Fabry disease. *Acta Clin Croat*. 2013 Sep;52(3):395-405.
8. Branton MH, Schiffmann R, Sabnis SG, et al. Natural history of Fabry renal disease: influence of alpha-galactosidase A activity and genetic mutations on clinical course. *Medicine (Baltimore)*. 2002 Mar;81(2):122-38.
9. Banikazemi M, Bultas J, Waldek S, et al. Agalsidase-beta therapy for advanced Fabry disease: a randomized trial. 2007 Jan 16;146(2):77-86. doi: 10.7326/0003-4819-146-2-200701160-00148. Epub 2006 Dec 18.
10. Wraith E, Tylki-Szymanska A, Guffon N, et al. Safety and efficacy of enzyme replacement therapy with agalsidase beta: an international, open-label study in pediatric patients with Fabry disease. *J Pediatr*. 2008 Apr;152(4):563-70, 570.e1. doi: 10.1016/j.jpeds.2007.09.007. Epub 2007 Dec 3.
11. Eng CM, Guffon N, Wilcox WR, et al; International Collaborative Fabry Disease Study Group. Safety and efficacy of recombinant human alpha-galactosidase A replacement therapy in Fabry's disease. *N Engl J Med*. 2001 Jul 5;345(1):9-16. doi: 10.1056/NEJM200107053450102.
12. Henderson N, Berry L, Laney DA. Fabry Disease practice resource: Focused revision. *J Genet Couns*. 2020 Oct;29(5):715-717. doi: 10.1002/jgc4.1318.
13. Mauer M, Kopp JB, Wallace E. (2022). Fabry disease: Clinical features and diagnosis. In Curhan GC, Glassock RJ (Eds.), *UpToDate*. Last updated: August 12, 2021. Accessed on January 5, 2023. Available from https://www.uptodate.com/contents/fabry-disease-clinical-features-and-diagnosis?search=anderson-fabry%20disease&source=search_result&selectedTitle=1~75&usage_type=default&display_rank=1#.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E75.21	Fabry (-Anderson) 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)
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>.

Language Assistance Services

ATTENTION: If you do not speak English, language assistance services, free of charge, are available to you. Call 1.800.940.5049 (TTY: 763.847.4013).

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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Minneapolis, MN 55459-0212
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200 Independence Avenue, SW
Room 509F, HHH Building
Washington, D.C. 20201
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