Galafold® (migalastat)
(Oral)

Document Number: IC-0380

Last Review Date: 02/02/2023
Date of Origin: 09/05/2018
Dates Reviewed: 09/2018, 02/2019, 02/2020, 02/2021, 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]:

- Galafold 123 mg capsules: 14 capsules per 28 days

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

- 123 mg every other day

III. Initial Approval Criteria [1]

Coverage is provided in the following conditions:

- Patient is at least 18 years of age; **AND**

Universal Criteria [1]

- Patient has NOT undergone or is scheduled to undergo kidney transplantation, or is currently on dialysis; **AND**
- Patient has an eGFR >30 mL/min/1.73 m^2; **AND**
- Must not be used in combination with agalsidase beta; **AND**

Fabry Disease (alpha-galactosidase A deficiency) † Φ [1,3,7,11]

- 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 (Gb_3/GL-3) or globotriaosylsphingosine (lyso-Gb_3); **OR**
  - Detection of pathogenic mutations in the *GLA* gene by molecular genetic testing; **AND**

- Patient has an amenable galactosidase alpha gene (*GLA*) variant* that is determined by or in consultation with a clinical genetics professional to be pathogenic; **AND**
- Baseline value for urine GL-3 and/or GL-3 inclusions

**Refer to prescribing information for amenable GLA variants*

† FDA approved indication(s); ‡ Compendia recommended Indication(s); Φ 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: severe kidney infections, etc.; **AND**
- Disease response with treatment as defined by a reduction in urine GL-3 and/or GL-3 inclusions compared to pre-treatment baseline

V. Dosage/Administration [1]

Indication	Dose
Fabry Disease	▪ The recommended dosage of Galafold is 123 mg orally once every other day at the same time of day. – *Patients should take Galafold on an empty stomach and should not consume any food or caffeine for at least 2 hours before and 2 hours after taking a dose for a minimum of 4 hour fast.* – *Galafold should NOT be taken on 2 consecutive days.*

VI. Billing Code/Availability Information

HCPCS Code:

- J8499 – Prescription drug, oral, non-chemotherapeutic, Not Otherwise Specified
- C9399 – Unclassified drugs or biologicals *(Hospital Outpatient Use ONLY)*

NDC:

- Galafold 123 mg capsules (wallet-pack containing 14 capsules): 71904-0100-xx

VII. References

1. Galafold [package insert]. Philadelphia, PA; Amicus Therapeutics, Inc.; December 2022. Accessed January 2023.

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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 12, 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. Adera M, Overton C, Boudes P. A double-blind, randomized, placebo-controlled study to evaluate the efficacy, safety and pharmacodynamics of AT1001 in patients with Fabry disease and AT1001-responsive GLA mutations. Molecular Genetics and Metabolism. February 2011 Volume 102, Issue 2, Pages S4–S5
9. Germain DP, Hughes DA, Nicholls K, et al. Treatment of Fabry's Disease with the Pharmacologic Chaperone Migalastat. N Engl J Med. 2016 Aug 11;375(6):545-55. doi: 10.1056/NEJMoa1510198.
10. 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.
11. 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 12, 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