# Galafold® (migalastat)
## (Oral)

Document Number: IC-P0380

**Last Review Date: 02/01/2024**
**Date of Origin:** 09/05/2018
**Dates Reviewed: 09/2018, 02/2019, 02/2020, 02/2021, 02/2022, 02/2023, 02/2024**

## I. Length of Authorization

Initial coverage will be provided for 6 months and may be renewed every 12 months thereafter.

## 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

Coverage is provided in the following conditions:

- The patient has a diagnosis of Fabry disease AND BOTH of the following:
  - The diagnosis was confirmed by mutation in the galactosidase alpha (GLA) gene; **AND**
  - The patient has a confirmed amenable GLA variant based on in vitro assay data (a complete list of amenable variants is available in the Galafold prescribing information, or a specific variant can be verified as amenable at http://www.galafoldamenabilitytable.us/reference); **AND**
- If the patient has an FDA approved indication, ONE of the following:
  - The patient's age is within FDA labeling for the requested indication for the requested agent; **OR**
  - The prescriber has provided information in support of using the requested agent for the patient's age for the requested indication; **AND**
- The prescriber is a specialist in the area of the patient's diagnosis (e.g., endocrinologist, geneticist, nephrologist) or the prescriber has consulted with a specialist in the area of the patient's diagnosis; **AND**
- The prescriber has assessed current status of ALL of the following: renal function (e.g., proteinuria, glomerular filtration rate [GFR]), cardiac function (e.g., left ventricular

hypertrophy, conduction defects, mitral and/or aortic valve abnormalities), ophthalmological signs (e.g., corneal verticillate, subcapsular cataracts, conjunctival and/or retinal vasculopathy), peripheral nerve symptoms (e.g., neuropathic pain, heat and/or cold intolerance, impaired sweat function), and gastrointestinal involvement (e.g., nausea, vomiting, abdominal pain, diarrhea, constipation); **AND**

- The patient will NOT be using the requested agent in combination with enzyme replacement therapy (ERT) (e.g., Fabrazyme) for the requested indication; **AND**
- The patient does NOT have any FDA labeled contraindications to the requested agent

## IV. Renewal Criteria

Coverage can be renewed based on the following criteria:

- The patient has been previously approved for the requested agent through the plan's Prior Authorization process; **AND**
- The patient has had improvements or stabilization with the requested agent as indicated by ONE of the following:
  - Renal function (e.g., proteinuria, glomerular filtration rate [GFR]); **OR**
  - Cardiac function (e.g., left ventricular hypertrophy, conduction defects, mitral and/or aortic valve abnormalities); **OR**
  - Ophthalmological signs (e.g., corneal verticillate, subcapsular cataracts, conjunctival and/or retinal vasculopathy); **OR**
  - Peripheral nerve symptoms (e.g., neuropathic pain, heat and/or cold intolerance, impaired sweat function); **OR**
  - Gastrointestinal symptoms (e.g., nausea, vomiting, abdominal pain, diarrhea, constipation); **AND**
- The prescriber is a specialist in the area of the patient's diagnosis (e.g., endocrinologist, geneticist, nephrologist) or the prescriber has consulted with a specialist in the area of the patient's diagnosis; **AND**
- The patient will NOT be using the requested agent in combination with enzyme replacement therapy (ERT) (e.g., Fabrazyme) for the requested indication; **AND**
- The patient does NOT have any FDA labeled contraindications to the requested agent

## V. Dosage/Administration

| Indication | Dose |
|---|---|
| Fabry Disease | ▪ The recommended dosage of Galafold is 123 mg orally once every other day at the same time of day.<br>– *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.
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 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)

The preceding information is intended for non-Medicare coverage determinations. 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 Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied 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 |