Reference number
2650-A

SPECIALTY GUIDELINE MANAGEMENT

GALAFOLD (migalastat)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Galafold is indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (*GLA*) variant based on in vitro assay data.

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Initial requests: laboratory confirmation of an amenable galactosidase alpha (GLA) variant.
B. Continuation requests: lab results or chart notes documenting a positive response to therapy.

III. CRITERIA FOR INITIAL APPROVAL

Fabry disease with an amenable galactosidase alpha gene (*GLA*) variant
Authorization of 12 months may be granted for treatment of Fabry disease with an amenable galactosidase alpha gene (*GLA*) variant when both of the following criteria are met:
A. Member has an amenable galactosidase alpha gene *(GLA)* variant based on in vitro assay data; and
B. The requested medication will not be used in combination with enzyme replacement therapy (ERT) for the treatment of Fabry disease.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III who are responding to therapy (e.g., reduction in plasma globotriaosylceramide [GL-3, Gb3] or GL-3/Gb3 inclusions, improvement and/or stabilization in renal function, pain reduction).

V. REFERENCES

1. Galafold [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; June 2023.

2. Biegstraaten M, Arngrimsson 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; 1036.
3. Ortiz A, Germain DP, Desnick RJ, et al. Fabry disease revisited: Management and treatment recommendations for adult patients. Mol Genet Metab. 2018;123(4):416-427.
4. Mehta A, Hughes DA. Fabry Disease. 2002 Aug 5 [Updated 2023 Mar 9]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2024. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1292/. Accessed February 2, 2024.

DOCUMENT HISTORY

Written:	Specialty Clinical Development (JL) 08/2018
Revised:	PS 02/2019, BI 06/2019 (off-cycle changes for alignment), FS 02/2020, CM/AP 02/2021, 05/2021 (CPO recommendation), MAP 02/2022 (annual), MAP 05/2022 (CPO recs), MAP 02/2023 (annual), MAP 02/2024 (annual)
Reviewed:	CHART/ GAD 08/2018, GAD 03/2019, DNC 06/2019, MM 06/2019, CHART 02/27/2020, 02/25/2021, 05/13/2021, 02/24/2022, 06/02/2022, 02/23/2023, 02/29/2024
External Review:	08/2018, 04/2019, 08/2019, 04/2020, 04/2021, 04/2022, 04/2023, 04/2024