

Specialty Guideline Management

Galafold

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Galafold	migalastat

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 Indications¹

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.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Initial Requests:

Galafold SGM 2650-A P2026.docx

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Reference number(s)
2650-A

- Laboratory confirmation of an amenable galactosidase alpha (GLA) variant.
- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g., acroparesthesias, angiokeratomas, gastrointestinal symptoms, corneal opacities).

Continuation Requests:

- Chart notes or medical records documenting a positive response to therapy (e.g., reduction in plasma globotriaosylceramide [GL-3, Gb3], GL-3/Gb3 inclusions or plasma lyso-GL-3, improvement and/or stabilization in renal function, pain reduction).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Fabry Disease with an Amenable Galactosidase Alpha Gene (GLA) variant¹⁻⁴

Authorization of 12 months may be granted for treatment of Fabry disease with an amenable galactosidase alpha gene (GLA) variant when all of the following criteria are met:

- Member is 18 years of age or older.
- Member has an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data
- Member exhibits clinical signs and symptoms of disease at baseline (e.g., acroparesthesias, angiokeratomas, gastrointestinal symptoms, corneal opacities).
- The requested medication will not be used in combination with enzyme replacement therapy (ERT) for the treatment of Fabry disease.

Continuation of Therapy

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

References

1. Galafold [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; March 2025.
2. Biegstraaten M, Arnggrimsson 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 2024 Apr 11]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. *GeneReviews* [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK1292/>. Accessed February 3, 2026.