

PHARMACY POLICY STATEMENT

North Carolina Marketplace

DRUG NAME	Galafold (migalastat)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Galafold is an alpha-galactosidase A (alpha-Gal A) pharmacological chaperone 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. It is estimated that the amenable variants are present in 35-50% of the Fabry disease patient population. Galafold is an alternative to enzyme replacement therapy (ERT) and is taken orally. It increases activity of the deficient enzyme instead of replacing it. Galafold was approved by the FDA in 2018 with accelerated approval status.

Fabry disease is an X-linked lysosomal storage disorder caused by mutations in the GLA gene that cause deficiency of the alpha-galactosidase A (alpha-Gal A) lysosomal enzyme. Normally this enzyme breaks down certain lipids in lysosomes, such as globotriaosylceramide (GL-3). Without it, GL-3 accumulates in blood vessels, the kidneys, heart, nerves, and other organs.

Galafold (migalastat) will be considered for coverage when the following criteria are met:

Fabry Disease

For **initial** authorization:

1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a medical geneticist, nephrologist, cardiologist, neurologist, or metabolic specialist; AND
3. Member has a confirmed diagnosis of Fabry disease; AND
4. Member has an amenable galactosidase alpha gene (GLA) variant (refer to package insert) based on in vitro assay data documented in chart notes; AND
5. Member does NOT have severe renal impairment or end-stage renal disease requiring dialysis; AND
6. Galafold will NOT be used in combination with ERT.
7. **Dosage allowed/Quantity limit:** 123 mg (1 capsule) orally every other day. QL: 14 capsules/ 28 days

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show positive clinical response such as stabilized kidney function (e.g., GFR, proteinuria), reduced plasma or tissue GL-3 levels, reduced left ventricular mass index, or other Fabry symptom improvement.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Galafold (migalastat) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/20/2019	New policy for Galafold created.
06/18/2021	Transferred to new template. Updated references. Added neurology to specialists. Removed baseline GL-3 level. Removed exclusions except renal impairment and combination therapy. Increased initial approval duration to 6 months and renewal to 12 months. Revised renewal criteria; removed % reductions.
11/22/2022	Annual review. Reorganized summary and added reference.
04/30/2025	Updated references. Changed not to be used with Fabrazyme to not to be used with ERT. Split diagnosis and amenable variant into separate criteria.

References:

1. Galafold [prescribing information]. Philadelphia, PA: Amicus Therapeutics US, LLC; 2025.
2. Hughes D, et al. Oral pharmacological chaperone migalastat compared with enzyme replacement therapy in Fabry disease: 18-month results from the randomised phase III ATTRACT study. *Journal of medical genetics*. 2017 Apr 1;54(4):288-96.
3. Feldt-Rasmussen U, Hughes D, Sunder-Plassmann G, et al. Long-term efficacy and safety of migalastat treatment in Fabry disease: 30-month results from the open-label extension of the randomized, phase 3 ATTRACT study. *Mol Genet Metab*. 2020;131(1-2):219-228. doi:10.1016/j.ymgme.2020.07.007
4. Germain D, et al. Treatment of Fabry's disease with the pharmacologic chaperone migalastat. *New England Journal of Medicine*. 2016 Aug 11;375(6):545-55.
5. National institute for health and care excellence. Migalastat for treating Fabry disease. 2017 Feb. Available from: [nice.org.uk/guidance/hst4/chapter/1-Recommendations](https://www.nice.org.uk/guidance/hst4/chapter/1-Recommendations).
6. Ortiz A, et al. Fabry disease revisited: management and treatment recommendations for adult patients. *Molecular genetics and metabolism*. 2018 Apr 1;123(4):416-27.
7. Wang R, et al. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genetics in Medicine*. 2011 May;13(5):457.
8. Laney DA, Bennett RL, Clarke V, et al. Fabry disease practice guidelines: recommendations of the National Society of Genetic Counselors. *J Genet Couns*. 2013;22(5):555-564. doi:10.1007/s10897-013-9613-3

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Revised date: 04/30/2025