CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Galafold (migalastat)
BILLING CODE	Must use valid NDC
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.
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 an amenable galactosidase alpha gene (GLA) variant (refer to package insert) based on in vitro assay data documented in chart notes; AND
4. Member does NOT have severe renal impairment or end-stage renal disease requiring dialysis; AND
5. Galafold will NOT be used in combination with Fabrazyme.
6. **Dosage allowed/Quantity limit:** 123 mg (1 capsule) orally every other day.

   (QL: 14 capsules per 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.

References:

1. Galafold [prescribing information]. Philadelphia, PA: Amicus Therapeutics US, LLC; February 2021.
2. Desnick R, et al. Fabry disease, an under-recognized multisystemic disorder: expert recommendations for diagnosis, management, and enzyme replacement therapy. Annals of internal medicine. 2003 Feb 18;138(4):338 46.
3. Ellaway C. Paediatric fabry disease. Transl pediatr. 2016; 5(1): 37-42.
4. Hopkin R, et al. The management and treatment of children with Fabry disease: A United States-based perspective. Molecular genetics and metabolism. 2016 Feb 1;117(2):104-13.
5. 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.
6. Wang R, et al. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. Genetics in Medicine. 2011 May;13(5):457.
7. Wanner C, et al. European expert consensus statement on therapeutic goals in Fabry disease. Molecular genetics and metabolism. 2018 Jun 12.
8. 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.
9. 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.
10. National institute for health and care excellence. Migalastat for treating Fabry disease. 2017 Feb. Available from: nice.org.uk/guidance/hst4/chapter/1-Recommendations.
11. 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
12. 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
13. Germain DP, Fouilhoux A, Decramer S, et al. Consensus recommendations for diagnosis, management and treatment of Fabry disease in paediatric patients. *Clin Genet.* 2019;96(2):107-117. doi:10.1111/cge.13546

Effective date: 04/01/2023
Revised date: 11/22/2022