

## PHARMACY POLICY STATEMENT

### Marketplace

<b>DRUG NAME</b>	<b>Galafold (migalastat)</b>
BILLING CODE	Must use valid NDC
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

Galafold is a pharmacologic chaperone that can be used instead of enzyme replacement therapy (ERT) for the treatment of Fabry disease. 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. Unlike ERT with Fabrazyme, Galafold can be taken orally and is only indicated for adult patients. Importantly, Galafold is only indicated in patients with certain amenable gene variants. It is estimated that the amenable variants are present in 35-50% of the Fabry disease patient population.

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

#### Fabry Disease

For **initial** authorization:

1. Member is 18 years of age or older; 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 orally every other day. (Limit: 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 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

<b>05/20/2019</b>	New policy for Galafold created.
<b>06/18/2021</b>	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.

References:

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- 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.
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- 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
- 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

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Revised date: 06/18/2021