CareSource

PHARMACY POLICY STATEMENT **Arkansas PASSE**	
DRUG NAME	Galafold (migalastat)
BILLING CODE	Must use valid NDC code
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
COVERAGE REQUIREMENTS	Prior Authorization Required (Non-Preferred Product) QUANTITY LIMIT— 14 capsules per 28 days
LIST OF DIAGNOSES CONSIDERED **NOT** MEDICALLY NECESSARY	Click Here

Galafold (migalastat) will only be considered for coverage under the **pharmacy** benefit when the following criteria are met:

Members must be clinically diagnosed with one of the following disease states and meet their individual criteria as stated.

FABRY DISEASE

For **initial** authorization:

1. Member is 18 years of age or older; AND
2. Member has diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data documented in chart notes; AND
3. Member has a documented baseline level of plasma globotriaosylsphingosine (lyso-GL_3) or urinary globotriaosylceramide (GL-3); AND
4. **Dosage allowed:** 123 mg every other day.

If member meets all the requirements listed above, the medication will be approved for 3 months.

For **reauthorization**:

1. Member has responded to therapy with chart notes documenting one of the following:
   a) Achieved and maintains at least a 20% reduction in plasma globotriaosylsphingosine (lyso-GL_3) levels; OR
   b) Achieved and maintains at least a 20% reduction in urinary globotriaosylceramide (GL-3).

If member meets all the reauthorization requirements above, the medication will be approved for an additional 6 months.

CareSource considers Galafold (migalastat) not medically necessary for the treatment of the diseases that are not listed in this document.

DATE	ACTION/DESCRIPTION
05/20/2019	New policy for Galafold created.
12/22/2021	Removed prescriber specialty requirement and removed “Member does not have any of the following” section.

References:

1. Benjamin ER, et al. The validation of pharmacogenetics for the identification of Fabry patients to be treated with migalastat. Genetics in medicine. 2017 Apr;19(4):430.

2. ClinicalTrials.gov. Identifier NCT01218659. Study to compare the efficacy and safet of oral AT1001 and enzyme replacement therapy in patients with fabry disease. Available: clinicaltrials.gov/ct2/show/NCT01218659.
3. ClinicalTrials.gov. Identifier NCT00925301. Study of the effects of oral AT1001 (migalastat hydrochloride) in patients with fabry disease. Available: clinicaltrials.gov/ct2/show/NCT00925301.
4. 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.
5. Ellaway C. Paediatric fabry disease. Transl pediatr. 2016; 5(1): 37-42.
6. Fabrazyme [package insert]. Cambridge, MA: Genzyme Corporation; May 2010.
7. Galafold [prescribing information]. Cranbury, NJ: Amicus Therapeutics U.S., Inc. 2018 August.
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. Germain DP, et al. Pharmacological chaperone therapy by active-site-specific chaperones in Fabry disease: in vitro and preclinical studies. Int J Clin Pharmacol Ther. 2009;47 Suppl 1:S111-7.
10. 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.
11. 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.
12. National institute for health and care excellence. Migalastat for treating Fabry disease. 2017 Feb. Available from: nice.org.uk/guidance/hst4/chapter/1-Recommendations.
13. 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.
14. Wang R, et al. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. Genetics in Medicine. 2011 May;13(5):457.
15. Wanner C, et al. European expert consensus statement on therapeutic goals in Fabry disease. Molecular genetics and metabolism. 2018 Jun 12.

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