ConnectiCare

Commercial/Healthcare Exchange PA Criteria

Effective: February 6th, 2019

Prior Authorization: Galafold

Products Affected: Galafold (migalastat) oral capsules

Medication Description:

Migalastat 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. Amenable GLA variants are detailed in the prescribing information. This indication is approved under Accelerated Approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate.

Migalastat is an alpha-Gal A pharmacological chaperone that reversibly binds to the active site of the alpha-galactosidase A (alpha-Gal A) protein (encoded by GLA). Migalastat restores the activity of alpha-Gal A by selectively binding to the active sites of certain mutant forms of alpha-Gal A, stabilizing them in the endoplasmic reticulum.

Migalastat is given as 123 mg orally once every other day at the same time of the day and should be given on an empty stomach. Food is not to be consumed at least 2 hours before and 2 hours after taking migalastat to allow for a minimum fast of 4 hours. The patient may consume clear liquids during the 4-hour period.

Covered Uses: Treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data.

Exclusion Criteria: N/A

Required Medical Information:

1. Documented diagnosis of Fabry disease with an amenable galactosidase alpha gene (GLA) variant

Age Restrictions: 18 years and older.

Prescriber Restrictions: N/A

Coverage Duration: 12 months

Other Criteria:

A. Patient has a documented diagnosis of Fabry disease; AND
B. Patient has an amenable galactosidase alpha gene (GLA) variant; AND
C. Will NOT be used in combination with agalsidase beta (Fabrazyme).

References:

1. Galafold [package insert]. Cranbury, NJ; Amicus; August 2018.
2. Mauer, M, Kopp JB, Schiffman R. Fabry Disease: Clinical Manifestations and Diagnosis. In: UpToDate, Curhan, GC, Glasscock, R (Ed), UpToDate, Waltham, MA, 2014. Miller RG, Jackson CE, Kasarskis EJ, et al. Practice parameter update: the care of the patient with amyotrophic lateral sclerosis: drug, nutritional, and respiratory therapies (an evidence-base review): report of the Quality Standards Subcommittee of the American Academy of Neurology. Neurology 2009; 73: 1218–1226.
3. Germain DP, Hughes DA, Nicholls K, et al. Treatment of Fabry's disease with the pharmacologic chaperone migalastat. N Engl J Med. 2016;375(6):545555. DOI: 10.1056/NEJMoa1510198.

4. Hughes DA, Nicholls K, Shankar SP, et al. Oral pharmacological chaperone migalastat compared with enzyme replacement therapy in Fabry disease: 18month results from the randomized phase III ATTRACT study. J Med Genet. 2017;54(4):288-296. DOI:10.1136/jmedgenet-2016-104178.
5. Fabrazyme [package insert]. Cambridge, MA; Genzyme; May 2010.
6. Hopkin RJ, Jefferies JL, Laney DA, et al. The management and treatment of children with Fabry disease: A United-States-based perspective. Mol Genet Metab. 2016; 117 (2):104-113. DOI: 10.1016/j.ymgme.2015.10.007.
7. Desnick RJ, Brady R, Barranger J, et al. Fabry disease, an under-recognized multisystemic disorder: expert recommendations for diagnosis, management, and enzyme replacement therapy. Ann Intern Med. 2003; 138(4):336-46.
8. NICE Guidance. Migalastat for treating Fabry disease. Published February 22, 2017. Available at: https://www.nice.org.uk/guidance/hst4/chapter/1Recommendations. Accessed September 13, 2018.
9. Lancy 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):555-64. DOI: 10.1007/s10897-013-9613-3.
10. Biegstraaten M, Arngrímsson 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 Journal of Rare Diseases. 2015; 10:36. DOI:10.1186/s13023-015-0253-6.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	01/28/2019
2	Annual Review	N/A	N/A	03/20/2020