Galafold (migalastat)

Override(s)	Approval Duration
Prior Authorization Quantity Limit	1 year

Medications	Quantity Limit
Galafold (migalastat)	May be subject to quantity limit

APPROVAL CRITERIA

Initial requests for Galafold (migalastat) may be approved if the following criteria are met:

I. Documentation is provided that individual has a diagnosis of Fabry disease as defined with either of the following (ACMG, NSGC):
   A. Complete deficiency or less than 5% of mean normal alpha-galactosidase A (α-Gal A) enzyme activity in leukocytes, dried blood spots, or serum (plasma) analysis; **OR**
   B. Galactosidase alpha (*GLA*) gene mutation by gene sequencing;

AND

II. Documentation is provided that individual has an amendable *GLA* gene variant based on the human embryonic kidney-293 (HEK-293) assay;

AND

III. Individual has one or more symptoms or physical findings attributable to Fabry disease (ACMG), such as but not limited to:
   A. Burning pain in the extremities (acroparesthesias); **OR**
   B. Cutaneous vascular lesions (angiokeratomas); **OR**
   C. Corneal verticillata (whorls); **OR**
   D. Decreased sweating (anhidrosis or hypohidrosis); **OR**
   E. Personal or family history of exercise, heat, or cold intolerance; **OR**
   F. Personal or family history of kidney failure.

Continuation requests for Galafold (migalastat) may be approved if the following criteria are met:

I. Individual has had a positive therapeutic response to treatment.

Galafold (migalastat) may not be approved for the following:

I. Individual has severe renal impairment or end-stage renal disease; **OR**

II. Individual is using in combination with agalsidase beta (Fabrazyme) or pegunigalsidase alfa-iwxj (Elfabrio).

Key References:

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3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Gal A, Hughes DA, Winchester B. Toward a consensus in the laboratory diagnostics of Fabry disease - recommendations of a European expert group. J Inherit Metab Dis. 2011;34(2):509-514. Accessed September 7, 2023.
5. Germain DP, Hughes DA, Nicholls K, et al. Treatment of Fabry's Disease with the Pharmacologic Chaperone Migalastat. N Engl J Med 2016;375:545-555.
6. Hughes DA, Nicholls K, Shankar SP, et al. Oral Pharmacological Chaperone Migalastat Compared with Enzyme Replacement Therapy in Fabry Disease: 18-Month Results from the Randomized Phase III ATTRACT Study. J Med Genet 2017;54:288-296.
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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. Focused Revision Sept. 2020. Accessed September 7, 2023.
9. Schiffmann R, Hughes D, Linthorst G, et al. Screening, diagnosis, and management of patients with Fabry disease: conclusions from a "Kidney Disease: Improving Global Outcomes" (KDIGO) Controversies Conference. Kidney Intl. 2017;91:284-293. Accessed September 7, 2023.
10. Wang RY, Bodamer OA, Watson MS, Wilcox WR; American College of Medical Genetics (ACMG) Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. Genet Med. 2011;13(5):457-484. Accessed September 7, 2023.