Mass General Brigham
Health Plan

Galafold (migalastat)
Effective 01/01/2025

Plan	☐ MassHealth UPPL ☒ Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☒ Pharmacy Benefit ☐ Medical Benefit		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	**Medical Benefit** **Pharmacy Benefit**	Phone: 833-895-2611 Phone: 800-711-4555	Fax: 888-656-6671 Fax: 844-403-1029
Exceptions	N/A		

Overview
Galafold (migalastat) is indicated for treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data

Galafold is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaoscylceramide (KIC GL-3) substrate. Continued approval of this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

Coverage Guidelines
Authorization may be granted for members new to the plan within the past 90 days and are currently receiving treatment with the requested medication, excluding when the product is obtained as samples or via manufacturer's patient assistance programs.
OR
Authorization may be granted when the following criteria is met:

1. Member is 18 years of age or older
2. Diagnosis of Fabry disease
3. The prescriber is a clinical genetics specialist or nephrologist or consult with either specialist is provided
4. Submission of results from enzyme assay test showing reduced or absent α-galactosidase A (α-GAL) enzyme activity in plasma, leukocytes, tears, or biopsied tissue
5. The member has GLA mutations which are amenable to treatment with Galafold

Limitations

1. Approvals will be granted for 12 months
2. Galafold will not be authorized in combination with enzyme replacement therapy (ERP)

References

1. Benjamin ER, Della Valle MC, Wu X, et al. The validation of pharmacogenetics for the identification of Fabry patients to be treated with migalastat. Genet Med 2017; 19:430
2. Galafold (migalastat) [prescribing information]. Cranbury, NJ: Amicus Therapeutics US Inc; October 2024.

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):545-555
4. 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; 117:104
5. McCafferty EH, Scott LJ. Migalastat: A Review in Fabry Disease. Drugs 2019; 79:543
6. Terryn W, Cochat P, Froissart R, et al. Fabry nephropathy: indications for screening and guidance for diagnosis and treatment by the European Renal Best Practice. Nephrol Dial Transplant 2013; 28:505

Review History

09/18/2019 – Reviewed
09/16/2020 – Reviewed at P&T.
12/11/2024 – Reviewed at December P&T. No changes. Effective 1/1/2025.