curative

Galafold Prior Authorization

Drug(s) Applied:	**Galafold** (migalastat)

Criteria:
Drug(s) Applied will be approved when the requested medication is being used for an FDA approved indication and all of the following criteria are met:

I. Initial Therapy Criteria
- **A. Fabry disease** as indicated by chart notes within past 180 days
  1. Diagnosis was confirmed by mutation in the galactosidase alpha (GLA) gene **and**
  2. Patient has a confirmed amenable GLA variant based on in vitro assay data (a complete list of amenable variants is available in the Galafold prescribing information, or a specific variant can be verified as amenable at http://www.galafoldamenabilitytable.us/reference) **and**
  3. If the amendable GLA variant is found to be of uncertain clinical significance (VUS, variant of uncertain significance) or may be benign (not causing Fabry disease), then the patient's amenable GLA variant has been interpreted by a clinical genetics professional as causing Fabry disease **and**
  4. Patient has evidence of Fabry-related symptoms and/or organ involvement (renal, cardiac, neurological, or gastrointestinal) **and**
  5. Patient's age is 18 years old or older **and**
  6. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., endocrinology, genetics, nephrology) **and**
  7. Patient's eGFR is > 30 mL/min/1.73m2 **and**
  8. Patient is not on dialysis nor diagnosed with End State Kidney Disease **and**
  9. Chart notes and/or prescriber do not provide documentation of concurrent use with enzyme replacement therapy (ERT) (e.g., Fabrazyme, Elfabrio) for the requested indication.

Approval Duration: 6 months

II. Continued Therapy Criteria
- **A. Fabry disease** as indicated by chart notes within past 12 months
  1. Patient meets the initial therapy criteria above **and**
  2. Patient's eGFR remains > 30 mL/min/1.73m2 with chart notes indicative of

ongoing monitoring **and**

3. Patient is not on dialysis nor diagnosed with End State Kidney Disease **and**
4. Chart notes and/or prescriber do not provide documentation of concurrent use with enzyme replacement therapy (ERT) (e.g., Fabrazyme, Elfabrio) for the requested indication **and**
5. Documented clinical benefit since starting the requested agent (i.e. positive biochemical marker response, renal stabilization or improvement, reduction or stabilization of left ventricular max index (LVMi), absence of new Fabry-associated clinical events)

Approval Duration: 12 months

References:

1. Galafold prescribing information. Amicus Therapeutics US, Inc. March 2025.
2. Ortiz A, Germain DP, Desnick RJ, et al. Fabry disease revisited: Management and treatment recommendations for adult patients. Molecular Genetics and Metabolism. 2018
3. Germain DP, Fouilhoux A, Decramer S, et al. Consensus recommendations for diagnosis, management and treatment of Fabry disease in paediatric patients. Clin Genet. 2019
4. Germain DP, Hughes DA, Nicholls K, et al. Treatment of Fabry's disease with the pharmacologic chaperone migalastat. N Engl J Med. 2016.
5. Bichet DG, Hopkin RJ, Aguiar P, et al. Consensus recommendations for the treatment and management of patients with Fabry disease on migalastat. Front Med (Lausanne). 2023.
6. Hughes DA, Sunder-Plassmann G, Jovanovic A, et al. Renal and multisystem effectiveness of 3.9 years of migalastat in a global real-world cohort: results from the followME Fabry Pathfinders Registry. J Inherit Metab Dis. 2025.

Policy Owned by: Curative PBM team