MEDICAL MUTUAL®

Drug Policy

Policy:	Galafold ® (migalastat)	Annual Review Date: 09/21/2023 Last Revised Date: 09/21/2023

OVERVIEW

Galafold 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. Certain GLA variants produce abnormally folded and less stable forms of the α-galactosidase A (α-GAL) enzyme, however the enzyme still retains activity. Galafold is a pharmacologic chaperone which binds to the active site of α-GAL, which stabilizes the enzyme and allows it to be trafficked from the endoplasmic reticulum to lysosomes. In the lysosome, Galafold dissociates from the enzyme allowing it to exert its pharmacologic activity.

POLICY STATEMENT

This policy involves the use of Galafold. Prior authorization is recommended for pharmacy benefit coverage of Galafold. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

Because of the specialized skills required for evaluation and diagnosis of patients treated with Galafold as well as the monitoring required for adverse events and long-term efficacy, initial approval requires Galafold be prescribed by or in consultation with a physician who specializes in the condition being treated. All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

RECOMMENDED AUTHORIZATION CRITERIA

Coverage of Galafold is recommended in those who meet the following criteria:

FDA-Approved Indications

1. **Fabry Disease.** Approve if the patient meets the following criteria (a, b, c, d, AND e):
   - **a.** The patient is 18 years of age or older; AND
   - **b.** The patient has confirmed diagnosis of Fabry disease and has an amenable galactosidase alpha gene (GLA) variant based on *in vitro* assay data; AND
   - **c.** The patients is currently experiencing signs and symptoms of Fabry disease (e.g. neuropathic pain, cornea verticillate, clustered angiokeratoma); AND

d. The patient will not take Galafold combined with other enzyme replacement therapy (ERT) (e.g., Fabrazyme)
e. Galafold is prescribed by or in consultation with a geneticist, nephrologist, or a physician who specializes in the treatment of Fabry disease.

Initial Approval/ Extended Approval.
A) *Initial Approval:6 months (180 days)*
B) *Extended Approval: 1 year (365 days)*

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Galafold has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. **Concurrent Use with Fabrazyme.** One small study (n = 23) assessed a single dose of Galafold (150 mg or 450 mg) used concurrently with Fabrazyme or agalsidase alpha. While a single dose of Galafold significantly increased α-GAL activity, the long-term safety and efficacy of concurrent use of Galafold and Fabrazyme have not been established. Galafold is not FDA approved for concurrent use with Fabrazyme.

2. **Use in patients with severe renal impairment or end-stage renal disease requiring dialysis.** Migalastat is substantially excreted by the kidneys. Systemic exposure was significantly increased in subjects with severe renal impairment (eGFR less than 30 mL/min/1.73 m2). GALAFOLD has not been studied in patients with Fabry disease who have an eGFR less than 30 mL/min/1.73 m2. GALAFOLD is not recommended for use in patients with severe renal impairment or end-stage renal disease requiring dialysis.

3. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

REFERENCES

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