Pharmacy Policy Bulletin: J-0806 Galafold (migalastat) – Commercial and Healthcare Reform	
Number: J-0806	**Category:** Prior Authorization
Line(s) of Business: ☒ Commercial ☒ Healthcare Reform ☐ Medicare	**Benefit(s):** **Commercial:** **Prior Authorization (1.)** **1.** Miscellaneous Specialty Drugs Oral = Yes w/ Prior Authorization **Healthcare Reform:** Not Applicable
Region(s): ☒ All ☐ Delaware ☐ New York ☐ Pennsylvania ☐ West Virginia	**Additional Restriction(s):** None
Version: J-0806-008	**Original Date:** 11/07/2018
Effective Date: 10/28/2024	**Review Date:** 10/02/2024

Drugs Product(s):	• Galafold (migalastat)
FDA-Approved Indication(s):	• Treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (*GLA*) variant based on in vitro assay data

Background:	• Galafold is a pharmacological chaperone that reversibly binds to the active site of the alpha-galactosidase A (alpha-Gal A) protein, which is deficient in Fabry disease and encoded by the galactosidase alpha gene, *GLA*. • Fabry disease is an X-linked lysosomal disorder caused by *GLA* mutations that lead to excessive deposition of neutral glycosphingolipids in the vascular endothelium of several organs and in epithelial and smooth muscle cells. Mutations in *GLA* cause deficiencies in the alpha-Gal A protein (responsible for breaking down glycosphingolipids) that can vary in severity. Certain variants (mutations) result in the production of abnormally folded and less stable forms of the protein that retain their enzymatic activity and may be stabilized by Galafold – these variants are considered to be "amenable." The stabilization restores alpha-Gal A protein trafficking into lysosomes and its intralysosomal activity, resulting in a breakdown of the glycosphingolipids. • Progressive endothelial accumulation of glycosphingolipids accounts for the associated clinical abnormalities of skin, eye, kidney, heart, brain, and peripheral nervous system. • In clinical trials, randomized patients were naïve to enzyme replacement therapy (ERT) or if they were previously treated with ERT (Fabrazyme (agalsidase beta)), had discontinued ERT at least 6 months prior to randomization. Long-term safety and efficacy of concurrent use of Galafold and Fabrazyme have not been established. • Prescribing Considerations: ○ Galafold should be prescribed by or in consultation with a geneticist, nephrologist, cardiologist, or a physician who specializes in the treatment of Fabry disease.

	○ Galafold is substantially excreted by the kidneys and not recommended for use in patients with severe renal impairment or end-stage renal disease requiring dialysis. No dosage adjustment is required in patients with mild to moderate renal impairment (eGFR at least 30 mL/min/1.73 m^2 and above).

Approval Criteria

I. Initial Authorization
When a benefit, coverage of Galafold may be approved when all of the following criteria are met **(A. through D.)**:
- **A.** The member is 18 years of age or older.
- **B.** The member has a diagnosis of Fabry disease (ICD-10: E75.21) confirmed by biochemical and/or genetic testing.
- **C.** Results of in vitro assay data show the presence of an amenable *GLA* variant that has been interpreted by a clinical genetics professional as causing Fabry disease in the clinical context of the patient.
- **D.** The member is not receiving concomitant enzyme replacement therapy (ERT) such as Fabrazyme (agalsidase beta).

II. Reauthorization
When a benefit, reauthorization of Galafold may be approved when all of the following criteria are met **(A. and B.)**:
- **A.** The prescriber attests that the member has experienced positive clinical response to therapy.
- **B.** The member is not receiving concomitant enzyme replacement therapy (ERT) such as Fabrazyme (agalsidase beta).

III. An exception to some or all of the criteria above may be granted for select members and/or circumstances based on state and/or federal regulations.

Limitations of Coverage

I. Coverage of drug(s) addressed in this policy for disease states outside of the FDA-approved indications should be denied based on the lack of clinical data to support effectiveness and safety in other conditions unless otherwise noted in the approval criteria.
II. For Commercial or HCR members with a closed formulary, a non-formulary product will only be approved if the member meets the criteria for a formulary exception in addition to the criteria outlined within this policy.

Authorization Duration

- Commercial and HCR Plans: If approved, up to a 12 month authorization may be granted.

Automatic Approval Criteria

None

References:

1. Galafold [package insert]. Philadelphia, PA: Amicus Therapeutics U.S., LLC; June 2024.
2. DRUGDEX System (Micromedex 2.0). Greenwood Village, CO: Truven Health Analytics; 2024.
3. Medscape. Overview of Fabry Disease. Available at: https://emedicine.medscape.com/article/1952086-overview. Accessed August 26, 2021.

4. U.S. National Library of Medicine. Genetics Home Reference. Fabry disease. Available at: https://ghr.nlm.nih.gov/condition/fabry-disease. Accessed August 26, 2021.