Drug Coverage Policy

Effective Date.....2/15/2026
Coverage Policy Number.....IP0400
Policy Title..... Galafold

Fabry Disease – Galafold

- Galafold® (migalastat capsules – Amicus Therapeutics)

INSTRUCTIONS FOR USE
The following Coverage Policy applies to health benefit plans administered by Cigna Companies. Certain Cigna Companies and/or lines of business only provide utilization review services to clients and do not make coverage determinations. References to standard benefit plan language and coverage determinations do not apply to those clients. Coverage Policies are intended to provide guidance in interpreting certain standard benefit plans administered by Cigna Companies. Please note, the terms of a customer's particular benefit plan document [Group Service Agreement, Evidence of Coverage, Certificate of Coverage, Summary Plan Description (SPD) or similar plan document] may differ significantly from the standard benefit plans upon which these Coverage Policies are based. For example, a customer's benefit plan document may contain a specific exclusion related to a topic addressed in a Coverage Policy. In the event of a conflict, a customer's benefit plan document always supersedes the information in the Coverage Policies. In the absence of a controlling federal or state coverage mandate, benefits are ultimately determined by the terms of the applicable benefit plan document. Coverage determinations in each specific instance require consideration of 1) the terms of the applicable benefit plan document in effect on the date of service; 2) any applicable laws/regulations; 3) any relevant collateral source materials including Coverage Policies and; 4) the specific facts of the particular situation. Each coverage request should be reviewed on its own merits. Medical directors are expected to exercise clinical judgment where appropriate and have discretion in making individual coverage determinations. Where coverage for care or services does not depend on specific circumstances, reimbursement will only be provided if a requested service(s) is submitted in accordance with the relevant criteria outlined in the applicable Coverage Policy, including covered diagnosis and/or procedure code(s). Reimbursement is not allowed for services when billed for conditions or diagnoses that are not covered under this Coverage Policy (see "Coding Information" below). When billing, providers must use the most appropriate codes as of the effective date of the submission. Claims submitted for services that are not accompanied by covered code(s) under the applicable Coverage Policy will be denied as not covered. Coverage Policies relate exclusively to the administration of health benefit plans. Coverage Policies are not recommendations for treatment and should never be used as treatment guidelines. In certain markets, delegated vendor guidelines may be used to support medical necessity and other coverage determinations.

OVERVIEW
Galafold, an oral alpha-galactosidase A (α-Gal) pharmacological chaperone, is indicated for the treatment of **Fabry disease in adults with** an amenable galactosidase alpha gene (*GLA*) variant based on in vitro assay data.[1]

Disease Overview
Fabry disease is a rare inherited X-linked lysosomal storage disorder.[2-4] Absent or significantly reduced α-Gal activity leads to the accumulation of globotriaosylceramide (GL-3) in a wide variety of cells throughout the body. The accumulation of GL-3 leads to progressive multisystem disease, primarily impacting the kidney, heart, and nervous system.[3,4] Life expectancy in patients with Fabry disease is reduced; median survival is typically 50 to 55 years in men and 70 years in women.[2]

Currently, there have been more than 800 mutations to the gene encoding α-Gal identified.[5] About 60% are missense mutations resulting in single amino acid substitutions. Some of these mutated enzymes have activity levels similar to normal α-Gal; however, they have been found to be unstable and are retained in the endoplasmic reticulum.

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POLICY STATEMENT
Prior Authorization is required for benefit coverage of Galafold. All approvals are provided for the duration noted below. 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, approval requires Galafold to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Documentation: Documentation is required where noted in the criteria as **[documentation required]**. Documentation may include, but is not limited to, chart notes, laboratory tests, claims records, and/or other information. All documentation must include patient-specific identifying information.

Galafold is considered medically necessary when the following is met:

FDA-Approved Indication

1. **Fabry Disease.** Approve for 1 year if the patient meets ALL the following (A, B, and C):
   A) Patient is ≥ 18 years of age; AND
   B) Patient has a pathogenic, or likely pathogenic, amenable galactosidase alpha gene (*GLA*) variant based on in vitro assay data **[documentation required]**; AND
   C) The medication is prescribed by or in consultation with a geneticist, nephrologist, or a physician who specializes in the treatment of Fabry disease.

Conditions Not Covered

Galafold for any other use is considered not medically necessary , including the following (this list may not be all inclusive; criteria will be updated as new published data are available):

1. **Concurrent use with Fabrazyme® (agalsidase beta intravenous infusion).** 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 has not been established.[6] Galafold is not FDA approved for concurrent use with Fabrazyme.

2. **Concurrent Use with Elfabrio (pegunigalsidase alfa intravenous infusion).** Galafold has not been evaluated for use in combination with Elfabrio. It is not FDA approved for concurrent use with enzyme replacement therapy.

References

1. Galafold® capsules [prescribing information]. Philadelphia, PA: Amicus Therapeutics; March 2025.
2. Schiffmann R. Fabry Disease. *Handb Clin Neurol*. 2015;132:231-248.
3. Arends M, Wanner C, Hughes D, et al. Characterization of Classical and Nonclassical Fabry Disease: A Multinational Study. *J Am Soc Nephrol*. 2017;28:1631-1641.
4. Laney DA, Bennett RL, Clarke V, et al. Fabry Disease Practice Guidelines: Recommendations of the National Society of Genetic Counselors. *J Genet Counsel*. 2013;22:555-564.
5. 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;19:430-438.
6. Warnock DG, Bichet DG, Holida M, et al. Oral Migalastat HCl Leads to Greater Systemic Exposure and Tissue Levels of Active α-Galactosidase A in Fabry Patients when Co-Administered with Infused Agalsidase. *PLoS ONE*. 2015;10: e0134341.

Revision Details

Type of Revision	Summary of Changes	Date
Annual Revision	**Policy Title:** **Updated from** "Migalastat" **to** "Fabry Disease – Galafold." **Fabry Disease:** **Updated from** "Diagnosis of Fabry disease confirmed by documentation of ONE of the following: 1. Male individual with a pathogenic, or likely pathogenic, amenable galactosidase alpha gene (GLA) variant based on in vitro assay data OR 2. Female individual with a pathogenic, or likely pathogenic, amenable galactosidase alpha gene (GLA) variant or a male or female with an amenable GLA variant of uncertain significance (VUS) based on in vitro assay data with at least ONE of the following signs or symptoms of Fabry disease - Crises of severe pain in the extremities (acroparesthesia), Appearance of vascular cutaneous lesions (angiokeratomas), Sweating abnormalities (anhidrosis, hypohidrosis or hyperhidrosis), Albuminuria/proteinuria, Renal failure, Cardiomyopathy" **to** "Patient has a pathogenic, or likely pathogenic, amenable galactosidase alpha gene (GLA) variant based on in vitro assay data **[documentation required]**."	2/15/2026

The policy effective date is in force until updated or retired.