



## PRIOR AUTHORIZATION POLICY

**POLICY:** Fabry Disease – Galafold Prior Authorization Policy

- Galafold® (migalastat capsules – Amicus Therapeutics)

**REVIEW DATE:** 11/06/2024

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### 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 AND HAVE DISCRETION IN MAKING INDIVIDUAL COVERAGE DETERMINATIONS. 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.

## CIGNA NATIONAL FORMULARY COVERAGE:

### OVERVIEW

Galafold, an oral alpha-galactosidase A ( $\alpha$ -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.<sup>1</sup>

### Disease Overview

Fabry disease is a rare inherited X-linked lysosomal storage disorder.<sup>2-4</sup> Absent or significantly reduced  $\alpha$ -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.<sup>3,4</sup> Life expectancy in patients with Fabry disease is reduced; median survival is typically 50 to 55 years in men and 70 years in women.<sup>2</sup>

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

## **POLICY STATEMENT**

Prior Authorization is recommended for prescription 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.

• **Galafold® (migalastat capsules – Amicus Therapeutics)**  
**is(are) covered as medically necessary when the following criteria is(are) met for FDA-approved indication(s) or other uses with supportive evidence (if applicable):**

### **FDA-Approved Indication**

- 1. Fabry Disease.** Approve for 1 year if the patient meets ALL of the following (A, B, and C):
  - A)** Patient is  $\geq 18$  years of age; AND
  - B)** Patient has an amenable galactosidase alpha gene (*GLA*) variant based on in vitro assay data; 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® (migalastat capsules – Amicus Therapeutics)**  
**is(are) considered experimental, investigational or unproven for ANY other use(s) 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  $\alpha$ -Gal activity, the long-term safety and efficacy of concurrent use of Galafold and Fabrazyme has not been established.<sup>6</sup> 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]. Cranbury, NJ: Amicus Therapeutics; June 2023.
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  $\alpha$ -Galactosidase A in Fabry Patients when Co-Administered with Infused Agalsidase. *PLoS ONE*. 2015;10: e0134341.

## HISTORY

Type of Revision	Summary of Changes	Review Date
Annual Revision	Added concurrent use with Elfabrio as a condition not recommended for approval.	11/15/2023
Annual Revision	No criteria changes.	11/06/2024

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