BlueCross BlueShield
Federal Employee Program.
Federal Employee Program®
750 9th St NW
Washington, D.C. 20001
202.942.1000
Fax 202.942.1125

5.30.057

Section:	Prescription Drugs	**Effective Date:**	April 1, 2024
Subsection:	Endocrine and Metabolic Drugs	**Original Policy Date:**	August 24, 2018
Subject:	Galafold	**Page:**	1 of 4

Last Review Date: March 8, 2024

Galafold

Description

Galafold (migalastat)

Background

Galafold (migalastat) is a pharmacological chaperone that reversibly binds to the active site of the alpha-galactosidase A (alpha-Gal A) protein (encoded by the galactosidase alpha gene, GLA), which is deficient in Fabry disease. This binding stabilizes alpha-Gal A allowing its trafficking into the lysosome where it exerts its action. Certain GLA variants (mutations) causing Fabry disease result in the production of abnormally folded and less stable forms of the alpha-Gal A protein which, however, retain enzymatic activity. Those GLA variants, referred to as amenable variants, produce alpha-Gal A proteins that may be stabilized by Galafold thereby restoring their trafficking to lysosomes and their intralysosomal activity. Clinical manifestations of Fabry disease include neuropathy, renal failure, cardiomyopathy, and cerebrovascular accidents (1).

Regulatory Status

FDA-approved indication: Galafold is an alpha-galactosidase A (alpha-Gal A) pharmacological chaperone 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 (1).

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials (1).

The safety and effectiveness of Galafold in pediatric patients have not been established (1).

Related policies
Elfabrio, Fabrazyme

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Galafold may be considered **medically necessary** if the conditions indicated below are met.

Galafold may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

Fabry disease

AND the following:

1. Patient has an amenable galactosidase alpha gene (GLA) variant based on an in vitro assay

Prior – Approval *Renewal* Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

Fabry disease

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity 42 capsules per 84 days

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary

Galafold is an alpha-galactosidase A (alpha-Gal A) pharmacological chaperone 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. The safety and effectiveness of Galafold in pediatric patients have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Galafold while maintaining optimal therapeutic outcomes.

References

1. Galafold [package insert]. Cranbury, NJ: Amicus Therapeutics U.S., Inc.; June 2023.

Policy History

Date	Action
August 2018	Addition to PA
October 2018	Changed quantity limits to match available blister packs
November 2018	Annual review
December 2019	Annual review
December 2020	Annual review and reference update
June 2021	Annual review and reference update
June 2022	Annual review and reference update
March 2023	Annual review and reference update. Changed policy number to 5.30.057

September 2023	Annual review and reference update. Per SME, added clinical manifestations of Fabry disease including neuropathy to background section
March 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 8, 2024 and is effective on April 1, 2024.