BlueCross BlueShield
Federal Employee Program.
Federal Employee Program®
1310 G Street, N.W.
Washington, D.C. 20005
202.942.1000
Fax 202.942.1125

5.30.57

Section:	Prescription Drugs	**Effective Date:**	July 1, 2022
Subsection:	Endocrine and Metabolic Drugs	**Original Policy Date:**	August 24, 2018
Subject:	Galafold	**Page:**	1 of 4

Last Review Date: June 16, 2022

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 (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

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** in patients 18 years of age and older with Fabry disease and if the conditions indicated below are met.

Galafold may be considered **investigational** in patients less than 18 years of age and for all other indications.

Prior-Approval Requirements

Age 18 years of age and older

Diagnosis

The patient must have the following:

Fabry disease

AND the following:

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

The 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.; December 2021.

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

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 16, 2022 and is effective on July 1, 2022.