BlueCross BlueShield Federal Employee Program

GALAFOLD (migalastat)

RATIONALE FOR INCLUSION IN PA PROGRAM

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).

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

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1. Galafold [package insert]. Cranbury, NJ: Amicus Therapeutics U.S., Inc.; October 2024.