



Smart

PA

Clinical Edit Criteria

Drug/Drug Class: Galafold™ (migalastat) Clinical Edit

Date: May 23, 2019

Prepared for:

Prepared by: MO HealthNet

New Criteria

Revision of Existing Criteria

Executive Summary

Purpose: Ensure appropriate utilization and control of Galafold™ (migalastat).

Why was this Issue Selected:

Galafold™ is an alpha-galactosidase 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. Galafold™ reversibly binds to the active site of the alpha-galactosidase A protein (encoded by the GLA), which is deficient in Fabry disease. By binding at the active site, migalastat stabilizes alpha-galactosidase A allowing its transfer from the endoplasmic reticulum into the lysosome where it exerts its action. Fabry disease affects approximately 3,000 people in the United States and has only one other current treatment option, Fabrazyme. Galafold™ is unlike Fabrazyme, an enzyme replacement therapy, in that it increases the activity of the deficient enzyme rather than replacing it and it's an oral option. Due to the highly specific patient population that would benefit from treatment with Galafold™ and high cost, MO HealthNet recommends adding a clinical edit to ensure appropriate patient selection.

Date Range FFS 1-1-2018 to 4-1-2019

Program-specific information:	Drug	Claims	MAC
	Galafold™	0	\$1749.46 per capsule (\$24,492.50 per month)

Type of Criteria:

Increased risk of ADE Non-Preferred Agent

Appropriate Indications

Data Sources: Only administrative databases Databases + Prescriber-supplied

Setting & Population

- Drug for review: Galafold™ (migalastat)
- Age range: all appropriate MO HealthNet participants aged 18 years and older

Approval Criteria

- 18 years of age or older
- Documented diagnosis of Fabry disease
- Adherence to prescribing within the protocol:
 - Genetic testing documentation verifying participant has an amenable *GLA* variant
 - Quantity limit: 14 caps for 28 days

Initial approval duration of 6 months in order to reevaluate therapy and ensure proper monitoring has occurred with regards to hepatic transaminase levels (AST, ALT). If criteria are met to continue therapy, 6-month renewal PA can be given with re-review required again in 6 months.

Denial Criteria

Therapy will be denied if approval criteria not met

References

1. Galafold (migalastat) [prescribing information]. Cranbury, NJ: Amicus Therapeutics U.S. Inc; August 2018.
2. Germain DP, Hughes DA, Nicholls K, et al. Treatment of Fabry's disease with the pharmacologic chaperone migalastat. *N Engl J Med*. 2016;375(6):545-555
3. Galafold (migalastat)
<https://secure.ipdanalytics.com/User/Pharma/RxStrategy/Recent/NovelBrandApprovals>