

# SmartPA Criteria Proposal

<b>Drug/Drug Class:</b>	Galafold® (migalastat) Clinical Edit
<b>First Implementation Date:</b>	May 23, 2019
<b>Proposed Date:</b>	March 19, 2020
<b>Prepared for:</b>	MO HealthNet
<b>Prepared by:</b>	MO HealthNet/Conduent
<b>Criteria Status:</b>	<input checked="" type="checkbox"/> Existing Criteria <input type="checkbox"/> Revision of Existing Criteria <input type="checkbox"/> New Criteria

## Executive Summary

**Purpose:** Ensure appropriate utilization and control of Galafold® (migalastat)

**Why 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. Fabry disease is a rare, progressive genetic disorder characterized by a defective gene, GLA, that causes a deficiency of the enzyme, alpha-galactosidase A (alpha-Gal A). This enzyme is responsible for breaking down specific lipids in lysosomes, including globotriaosylceramide (GL-3). The accumulation of GL-3 in blood vessels, kidneys, heart, nerves and other organs leads to cell damage with consequences from mild-to-severe symptoms including kidney failure, myocardial infarctions, and strokes that can be fatal. Galafold binds to and stabilizes specific mutant forms of alpha-Gal A, thereby facilitating proper trafficking of the enzyme to lysosomes and increasing enzyme activity. 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.

### Program-Specific Information:

Date Range FFS 1-1-2019 to 12-31-2019				
Drug	Claims	Spend	Cost per unit	Cost per month
GALAFOLD 123MG CAP	0	-	\$1,784.45 MAC	\$24,982.35 MAC

**Type of Criteria:**  Increased risk of ADE  Preferred Drug List  
 Appropriate Indications  Clinical Edit

**Data Sources:**  Only Administrative Databases  Databases + Prescriber-Supplied

## Setting & Population

- Drug class for review: Galafold® (migalastat)
- Age range: All appropriate MO HealthNet participants aged 18 years or older

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## Approval Criteria

- Participant is aged 18 years or older **AND**
- Documented diagnosis of Fabry disease **AND**
- Documented genetic testing confirming participant has an amenable GLA variant

## Denial Criteria

- Therapy will be denied if no approval criteria are met
- Claim exceeds 14 capsules for 28 days of therapy

## Required Documentation

Laboratory Results:  
MedWatch Form:

X

Progress Notes:  
Other:


## Disposition of Edit

Denial: Exception code "0682" (Clinical Edit)  
Rule Type: CE

## Default Approval Period

6 months

## References

- GALAFOLD (migalastat) capsules, [package insert]. Cranbury, NJ: Amicus Therapeutics U.S., Inc.; June 2019.
- IPD Analytics. New Drug Approval: Galafold (migalastat). September 2018.
- 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

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