



# SmartPA Criteria Proposal

<b>Drug/Drug Class:</b>	Fabry Disease Clinical Edit ( <i>formerly Galafold Clinical Edit</i> )
<b>First Implementation Date:</b>	May 23, 2019
<b>Proposed Date:</b>	March 18, 2021
<b>Prepared for:</b>	MO HealthNet
<b>Prepared by:</b>	MO HealthNet/Conduent
<b>Criteria Status:</b>	<input type="checkbox"/> Existing Criteria <input checked="" type="checkbox"/> Revision of Existing Criteria <input type="checkbox"/> New Criteria

## Executive Summary

**Purpose:** Ensure appropriate utilization and control of agents for Fabry Disease

**Why Issue Selected:** Fabry disease is a rare, progressive genetic disorder characterized by a defective gene, galactosidase alpha 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. Treatment of Fabry disease primarily focuses upon replacing the missing or deficient enzyme (alpha-Gal A) with enzyme replacement therapy as well as treating the various symptoms and disease complications. Galafold®, an alpha-galactosidase A pharmacological chaperone, was FDA approved in 2018 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 (present in 35% to 50% of patients). 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 and high cost, MO HealthNet recommends adding a clinical edit to ensure appropriate patient selection.

**Program-Specific Information:**

Date Range FFS 1-1-2020 to 12-31-2020			
Drug	Claims	Spend	Avg Spend per Claim
FABRAZYME 5 MG VIAL	26	\$210,748.90	\$8,105.72
FABRAZYME 35 MG VIAL	63	\$1,389,851.21	\$22,061.13
GALAFOLD 123 MG CAP	0	-	-

**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: Agents for Fabry Disease
- Age range: All appropriate MO HealthNet participants aged **8 years or older**

## Approval Criteria

- Documented diagnosis of Fabry disease **AND**
- **For Fabrazyme: participant is aged 8 years or older**
- For Galafold:
  - Participant is aged 18 years or older **AND**
  - Documented genetic testing confirming participant has an amenable *GLA* variant **AND**
  - Claim does not exceed 14 capsules for 28 days of therapy

## Denial Criteria

- Therapy will be denied if all approval criteria are not met

## 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.; February 2021.
- FABRAZYME® (agalsidase beta) [package insert]. Cambridge, MA: Genzyme Corporation; December 2018.
- IPD Analytics. New Drug Approval: Galafold (migalastat). September 2018.
- IPD Analytics. Endocrine and Metabolic Agents: Fabry Disease. Accessed February 11, 2021.
- 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

*SmartPA Clinical Proposal Form*

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