

# SmartPA Criteria Proposal

<b>Drug/Drug Class:</b>	Fabry Disease Clinical Edit
<b>First Implementation Date:</b>	May 23, 2019
<b>Proposed Date:</b>	March 17, 2022
<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<sup>®</sup>, 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<sup>®</sup>. Galafold is unlike Fabrazyme, an enzyme replacement therapy, in that it increases the activity of the deficient enzyme rather than replacing it and is an oral medication.

Due to high cost and specific approved indication, MO HealthNet will impose clinical criteria to ensure appropriate utilization of agents for Fabry Disease.

**Program-Specific Information:**

Date Range FFS 1-1-2021 to 12-31-2021			
Drug	Claims	Spend	Avg Spend per Claim
FABRAZYME 5 MG VIAL	59	\$254,977.98	\$4,321.66
FABRAZYME 35 MG VIAL	70	\$993,603.58	\$14,194.34
GALAFOLD 123 MG CAP	4	\$102,595.85	\$25,648.96

**Type of Criteria:**  Increased risk of ADE  
 Appropriate Indications

Preferred Drug List  
 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 **2** years or older

## Approval Criteria

- Documented diagnosis of Fabry disease **AND**
- For Fabrazyme: participant is aged **2** 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:

<input checked="" type="checkbox"/>
<input type="checkbox"/>

Progress Notes:  
Other:

<input type="checkbox"/>
<input type="checkbox"/>

## 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.; December 2021.
- FABRAZYME® (agalsidase beta) [package insert]. Cambridge, MA: Genzyme Corporation; March 2021.
- IPD Analytics. New Drug Approval: Galafold (migalastat). September 2018.
- IPD Analytics. Endocrine and Metabolic Agents: Fabry Disease. Accessed February 2, 2022.
- 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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