SmartPA Criteria Proposal

Drug/Drug Class:	Fabry Disease Clinical Edit
First Implementation Date:	May 23, 2019
Revised Date:	July 29, 2021
Prepared for:	MO HealthNet
Prepared by:	MO HealthNet/Conduent
Criteria Status:	☐Existing Criteria ☒Revision of Existing Criteria ☐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**
☒ **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:	X	Progress Notes:	
MedWatch Form:		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; March 2021.
- IPD Analytics. New Drug Approval: Galafold (migalastat). September 2018.
- IPD Analytics. Endocrine and Metabolic Agents: Fabry Disease. Accessed July 8, 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