Effective Date: 3/1/2021
Reviewed: 12/2020, 06/2021, 04/2022, 04/2023, 05/2024 Scope: Medicaid

GALAFOLD (migalastat)

POLICY

I. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

a. Initial requests: laboratory confirmation of an amenable galactosidase alpha (GLA) gene variant.
b. Continuation requests: lab results or chart notes documenting a positive response to therapy (e.g., reduction in plasma globotriaosylceramide [GL-3, Gb3] or GL-3/Gb3 inclusions, improvement and/or stabilization in renal function, pain reduction).

II. CRITERIA FOR INITIAL APPROVAL

Fabry disease with an amenable galactosidase alpha gene (*GLA*) variant
Authorization of 6 months may be granted for treatment of Fabry disease with an amenable galactosidase alpha gene (*GLA*) variant when all of the following criteria are met:

A. Patient is 18 years old or older; AND
B. The diagnosis of Fabry disease was confirmed by enzyme assay demonstrating a deficiency of alpha-galactosidase enzyme activity or by genetic testing, or the member is a symptomatic obligate carrier; AND
C. Patient has an amenable galactosidase alpha gene *(GLA)* variant based on in vitro assay data; AND
D. Galafold will not be used in combination with Fabrazyme (agalsidase beta) or Elfabrio (pegunigalsidase alfa-iwxj); AND
E. Patient does not have severe renal impairment or end-stage renal disease requiring dialysis.

III. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for Fabry disease with an amenable galactosidase alpha gene (*GLA*) variant who are responding to therapy (e.g., reduction in plasma globotriaosylceramide [GL-3] or GL-3 inclusions).

IV. QUANTITY LIMIT

- 14 capsules per 28 days

V. COVERAGE DURATION

- Initial Approval: 6 months
- Continuation Approval: 12 months

VI. REFERENCES

1. Galafold [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; June 2023.