Reference number
2650-A

SPECIALTY GUIDELINE MANAGEMENT

GALAFOLD (migalastat)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Galafold is 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.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. Initial requests: alpha- galactosidase enzyme assay or genetic testing results supporting diagnosis. In the case of obligate carriers, the documentation must be submitted for the parent.
B. Continuation requests: chart notes documenting a positive response to therapy (e.g., reduction in plasma globotriaosylceramide [GL-3] or GL-3 inclusions, improvement and/or stabilization in renal function, pain reduction).

III. CRITERIA FOR INITIAL APPROVAL

Fabry disease with an amenable galactosidase alpha gene (*GLA*) variant
Authorization of 12 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. 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
B. Member has an amenable galactosidase alpha gene *(GLA)* variant based on in vitro assay data; and
C. Galafold will not be used in combination with enzyme replacement therapy (ERT) for the treatment of Fabry disease.

IV. 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, improvement and/or stabilization in renal function, pain reduction).

V. REFERENCES

1. Galafold [package insert]. Cranbury, NJ: Amicus Therapeutics U.S., Inc.; September 2020.

2. Biegstraaten M, Arngrimsson R, Barbey F, et al. Recommendations for initiation and cessation of enzyme replacement therapy in patients with Fabry disease: the European Fabry Working Group consensus document. *Orphanet J Rare Dis.* 2015; 1036.
3. Ortiz A, Germain DP, Desnick RJ, et al. Fabry disease revisited: Management and treatment recommendations for adult patients. Mol Genet Metab. 2018;123(4):416-427.