Reference number
2054-A

SPECIALTY GUIDELINE MANAGEMENT

FABRAZYME (agalsidase beta)

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
Fabrazyme is indicated for the treatment of adult and pediatric patients 2 years of age and older with confirmed Fabry disease.

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: lab results or chart notes documenting a positive response to therapy.

III. CRITERIA FOR INITIAL APPROVAL

Fabry disease
Authorization of 12 months may be granted for treatment of Fabry disease when both 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. Fabrazyme will not be used in combination with Galafold.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III who are responding 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).

V. REFERENCES

1. Fabrazyme [package insert]. Cambridge, MA: Genzyme Corporation; March 2023.
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.

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