

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. REQUIRED 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 (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**

Authorization for 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.
- 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 Fabry disease 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. Fabrazyme [package insert]. Cambridge, MA: Genzyme Corporation; March 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.

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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.