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 use in patients with Fabry disease. Fabrazyme reduces globotriaosylceramide (GL-3) deposition in capillary endothelium of the kidney and certain other cell types.

All other indications are considered experimental/investigational and are not a covered benefit.

II. REQUIRED DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review: alpha-galactosidase enzyme assay or genetic testing results supporting diagnosis. In the case of obligate carriers, the documentation must be submitted for the parent.

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).

V. REFERENCES

1. Fabrazyme [package insert]. Cambridge, MA: Genzyme Corporation; December 2018.
2. Desnick RJ, Brady RO. Fabry disease in childhood. *J Pediatr*. 2004;144(5 Suppl):S20-S26.
3. 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.
4. Desnick RJ, Brady R, Barranger J, et al. Fabry disease, an under-recognized multisystemic disorder: expert recommendations for diagnosis, and enzyme replacement therapy. *Ann Intern Med.* 2003; 138(4):338.

5. Sirrs, S, Bichet DG, Iwanochko RM, et al. Canadian Fabry disease treatment guidelines 2016.