Fabrazyme (agalsidase beta)

Override(s)	Approval Duration
Prior Authorization	1 year

Medications	Dosing Limit
Fabrazyme (agalsidase beta) 5 mg, 35 mg vial	1 mg/kg every two weeks

APPROVAL CRITERIA

Initial requests for Fabrazyme (agalsidase beta) may be approved when the following criteria are met:

I. Documentation is provided that individual has a diagnosis of Fabry disease as defined with either of the following (ACMG, NSGC):
   A. Complete deficiency or less than 5% of mean normal alpha-galactosidase A (α-Gal A) enzyme activity in leukocytes, dried blood spots, or serum (plasma) analysis; **OR**
   B. Galactosidase alpha gene mutation by gene sequencing; **AND**

II. The individual to be treated has one or more symptoms or physical findings attributable to Fabry disease, including, but not limited to:
   A. Burning pain in the extremities (acroparesthesias); **OR**
   B. Cutaneous vascular lesions (angiokeratomas); **OR**
   C. Corneal verticillata (whorls); **OR**
   D. Decreased sweating (anhidrosis or hypohidrosis); **OR**
   E. Personal or family history of exercise, heat, or cold intolerance; **OR**
   F. Personal or family history of kidney failure.

Continuation requests for Fabrazyme (agalsidase beta) may be approved if the following criteria are met:

I. Individual has had a positive therapeutic response to treatment.

Fabrazyme (agalsidase beta) may not be approved for the following:

I. Individual is using in combination with migalastat (Galafold) or pegunigalsidase alfa-iwxj; **OR**
II. When the above criteria are not met and for all other indications.

Key References:

1. Biegstraaten M, Arngrímsson 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; 10:36. Available at http://www.ncbi.nlm.nih.gov/pmc/articles/PMC4383065/pdf/13023_2015_Article_253.pdf. Accessed: September 7, 2023.
2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: September 7, 2023.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Gal A, Hughes DA, Winchester B. Toward a consensus in the laboratory diagnostics of Fabry disease - recommendations of a European expert group. J Inherit Metab Dis. 2011;34(2):509-514. Accessed September 7, 2023.
5. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; Updated periodically.
6. Laney DA, Bennett RL, Clarke V, et al. Fabry disease practice guidelines: recommendations of the National Society of Genetic Counselors (NSGC). J Genet Couns. 2013;22(5):555-564. Focused Revision Sept. 2020. Accessed September 7, 2023.
7. Ortiz A, Germain D, Desnick R, et al. Fabry disease revisited: Management and treatment recommendations for adult patients. Mol Gen Metab. 2018;123(4):416-427. Accessed on September 7, 2023.
8. Schiffmann R, Hughes D, Linthorst G, et al. Screening, diagnosis, and management of patients with Fabry disease: conclusions from a “Kidney Disease: Improving Global Outcomes” (KDIGO) Controversies Conference. Kidney Intl. 2017;91:284-293. Accessed September 7, 2023.
9. Wang RY, Bodamer OA, Watson MS, Wilcox WR; American College of Medical Genetics (ACMG) Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. Genet Med. 2011;13(5):457-484. Accessed September 7, 2023.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.