

Market Applicability						
Market	GA	KY	MD	NJ	NY	WA
Applicable	X	X	X	NA	X	X

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

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

- I. Individual has a diagnosis of Fabry disease is confirmed with either of the following (ACMG, NSGC):
 - A. Documentation of 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. Documented 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.

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

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

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Applicable	X	X	X	NA	X	X

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: August 10, 2020.
2. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2020. URL: <http://www.clinicalpharmacology.com>. Updated periodically.
3. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. <http://dailymed.nlm.nih.gov/dailymed/about.cfm>. Accessed: August 10, 2020.
4. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
5. Gal A, Hughes DA, Winchester B. Toward a consensus in the laboratory diagnostics of Fabry disease - recommendations of a European expert group. *J Inher Metab Dis.* 2011;34(2):509-514. Accessed August 10, 2020.
6. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2020; Updated periodically.
7. 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. Accessed August 10, 2020.
8. 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 August 10, 2020.
9. 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.
10. 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 August 10, 2020.

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

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