Market Applicability							
Market	**DC**	**GA**	**KY**	**MD**	**NJ**	**NY**	**WA**
Applicable	X	X	X	X	X	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.

Requests for Fabrazyme (agalsidase beta) may not be approved when the criteria above are not met and for all other indications.

Market Applicability							
Market	**DC**	**GA**	**KY**	**MD**	**NJ**	**NY**	**WA**
Applicable	X	X	X	X	X	X	X

State Specific Mandates		
State name	Date effective	Mandate details (including specific bill if applicable)
N/A	N/A	N/A

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 29, 2019.
2. Clinical Pharmacology [database online]. Tampa, FL: Gold Standard, Inc.: 2019. 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 26, 2019.
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 Inherit Metab Dis. 2011;34(2):509-514.
6. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2019; Updated periodically.
7. Laney DA, Bennett RL, Clarke V, et al. Fabry disease practice guidelines: recommendations of the National Society of Genetic Counselors. J Genet Couns. 2013;22(5):555-564.
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.
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.

This policy does not apply to health plans or member categories that do not have pharmacy benefits, nor does it apply to Medicare. Note that market specific restrictions or transition-of-care benefit limitations may apply.