Clinical Policy: Agalsidase Beta (Fabrazyme)

Reference Number: PA.CP.PHAR.158
Effective Date: 01/18
Last Review Date: 02/17

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for agalsidase beta (Fabrazyme®).

Policy/Criteria
It is the policy of health plans affiliated with Pennsylvania Health and Wellness that Fabrazyme is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Fabry Disease (must meet all):

1. Diagnosis of Fabry disease confirmed by one of the following:
   a. Enzyme assay demonstrating a deficiency of alpha-galactosidase activity;
   b. DNA testing.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Fabry Disease (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies; or
2. Refer to PA.CP.PHAR.57 - Global Biopharm Policy**.**

Background
Description/Mechanism of Action:
Fabry disease is an X-linked genetic disorder of glycosphingolipid metabolism. Deficiency of the lysosomal enzyme alpha- galactosidase A leads to progressive accumulation of glycosphingolipids, predominantly GL-3, in many body tissues, starting early in life and continuing over decades. Clinical manifestations of Fabry disease include renal failure, cardiomyopathy, and cerebrovascular accidents. Accumulation of GL-3 in renal endothelial cells may play a role in renal failure. Fabrazyme is intended to provide an exogenous source of alpha-galactosidase A in Fabry disease patients. Nonclinical and clinical studies evaluating a limited

number of cell types indicate that Fabrazyme will catalyze the hydrolysis of glycosphingolipids, including GL-3.

Formulations:
Fabrazyme (agalsidase beta): Lyophilized product for reconstitution; for intravenous use
- 5 mg/1 mL vial (70 units/mg)
- 35 mg/7 mL vial; 5 mg/mL (70 units/mg)

FDA Approved Indications:
Fabrazyme (agalsidase beta) is a recombinant human alpha-galactosidase A enzyme/intravenous formulation 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.

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J0180	Injection, agalsidase beta, 1 mg

Reviews, Revisions, and Approvals	Date	Approval Date

References
1. Fabrazyme prescribing information. Cambridge, MA: Genzyme Corporation; May 2010. Available at http://www.fabrazyme.com. Accessed December 14, 2016.
2. Desnick RJ, Brady R, Barranger J, et al. Fabry disease, an under-recognized multisystemic disorder: Expert recommendations for diagnosis, management, and enzyme replacement therapy. Ann Intern Med. 2003; 138(4): 338-346.
3. Desnick RJ, Brady RO. Fabry disease in childhood. J Pediatr. 2004; 144(5 Suppl): S20-S26.