Clinical Policy: Agalsidase Beta (Fabrazyme)

Reference Number: PA.CP.PHAR.158
Effective Date: 01/18
Last Review Date: 04/19

Coding Implications
Revision Log

Description
Agalsidase beta (Fabrazyme®) is a recombinant human alpha-galactosidase A enzyme.

FDA Approved Indication
Fabrazyme is indicated for the treatment of Fabry disease.

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;
2. Dose does not exceed 1 mg/kg every 2 weeks.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

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 as evidenced by improvement in the individual member's Fabry disease manifestation profile;
3. If request is for a dose increase, new dose does not exceed 1 mg/kg every 2 weeks.

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.PMN.53

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings
- Contraindication(s): none reported.
- Boxed warning(s): none reported.

Appendix D: General Information
The presenting symptoms and clinical course of Fabry disease can vary from one individual to another. As such, there is not one generally applicable set of clinical criteria that can be used to determine appropriateness of continuation of therapy. Some examples, however, of improvement in Fabry disease as a result of Fabrazyme therapy may include improvement in:
- Fabry disease signs such as pain in the extremities, hypohidrosis or anhidrosis, or angiokeratomas
- Diarrhea, abdominal pain, nausea, vomiting, and flank pain
- Renal function
- Neuropathic pain, heat and cold intolerance, vertigo and diplopia
- Fatigue

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Fabry disease	1 mg/kg IV every 2 weeks	1 mg/kg/2 weeks

V. Product Availability
Single-use vial: 5 mg, 35 mg

VI. References
1. Fabrazyme Prescribing Information. Cambridge, MA: Genzyme Corporation; May 2010. Available at http://www.fabrazyme.com. Accessed February 27, 2019.
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.

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
2Q 2018 annual review:; added age limit; added requirement for documentation of positive response to therapy for reauthorization; changed approval durations from length of benefit to 6/12 months; references reviewed and updated.	2.26.18	
2Q 2019 annual review: references reviewed and updated.	04/19	