Clinical Policy: Agalsidase Beta (Fabrazyme)

Reference Number: PA.CP.PHAR.158
Effective Date: 01/2018
Last Review Date: 04/2025

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

FDA Approved Indication
Fabrazyme is indicated for the treatment of adult and pediatric patients 2 years of age and older with confirmed Fabry disease.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & 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 or b):
   a. Enzyme assay demonstrating a deficiency of alpha-galactosidase activity;
   b. DNA testing;
2. Prescribed by or in consultation with a clinical geneticist, cardiologist, nephrologist, neurologist, lysosomal disease specialist, or Fabry disease specialist;
3. Age ≥ 2 years;
4. Fabrazyme is not prescribed concurrently with Galafold or Elfabrio;
5. Documentation of member's current weight (in kg);
6. 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 PA Health & Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.PHARM.01) applies;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's Fabry disease manifestation profile *(see Appendix D for examples)*;
3. Fabrazyme is not prescribed concurrently with Galafold or Elfabrio;
4. Documentation of member's current weight (in kg);
5. 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 PA Health & Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.PHARM.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
- 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
- Cornea verticillata

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; July 2024. Available at http://www.fabrazyme.com. Accessed January 8, 2025.
2. Ortiz A, Germain DP, Desnick RJ, et al. Fabry disease revisited: management and treatment recommendations for adult patients. Molecular Genetics and Metabolism 2018;123:416-27.
3. Hopkin RJ, Jeffries JL, Laney DA, et al. The management and treatment of children with Fabry disease: A United States-based perspective. Molecular Genetics and Metabolism 2016;117:104-13.

4. Germain DP, Fouilhoux A, Decramer S, et al. Consensus recommendations for diagnosis, management and treatment of Fabry disease in paediatric patients. Clinical Genetics. 2019;96:107-17.
5. Germain DP, Altarescu G, Barriales-Villa R, et al. An expert consensus on practical clinical recommendations and guidance for patients with classic Fabry disease. Molecular Genetics and Metabolism. July 2022;137:49-61.

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
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.	02/2018
2Q 2019 annual review: references reviewed and updated.	04/2019
2Q 2020 annual review: references reviewed and updated.	04/2020
2Q 2021 annual review: added a requirement for a clinical geneticist specialist and no concomitant use with Galafold; references reviewed and updated.	04/2021
Added other specialist types who might be involved in a Fabry patient's care, in line with the previously P&T-approved approach to specialists in Fabry disease.	10/2021
2Q 2022 annual review: updated age limit to ≥ 2 years of age per FDA-approved pediatric extension; references reviewed and updated.	04/2022
2Q 2023 annual review: no significant changes; references reviewed and updated.	04/2023
2Q 2024 annual review: added exclusion for concomitant use with Elfabrio to align with the Elfabrio criteria; references reviewed and updated.	04/2024
2Q 2025 annual review: no significant changes; added requirement for documentation of member's weight for dose calculation purposes; references reviewed and updated.	04/2025