Clinical Policy: Agalsidase Beta (Fabrazyme)

Reference Number: ERX.SPA.104
Effective Date: 10.01.16
Last Review Date: 05.21
Line of Business: Commercial, Medicaid

Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

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

FDA Approved Indication(s)
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.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ 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;
3. Age ≥ 2 years;
4. Fabrazyme is not prescribed concurrently with Galafold;
5. Dose does not exceed 1 mg per kg every 2 weeks.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

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

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
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. If request is for a dose increase, new dose does not exceed 1 mg per kg every 2 weeks.

Approval duration: 12 months

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

I. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
Approval duration: Duration of request or 6 months (whichever is less); or

II. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off-label use policy – ERX.PA.01 or evidence of coverage documents.

IV. 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;
- Corneal verticillata.

V. Dosage and Administration

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

VI. Product Availability

Single-use vial: 5 mg, 35 mg

VII. References

1. Fabrazyme Prescribing Information. Cambridge, MA: Genzyme Corporation; March 2021. Available at http://www.fabrazyme.com. Accessed March 15, 2021.
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.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Converted to new template. Added prescriber requirement. Modified age restriction from > 8 years to ≥ 8 years per PI. Added max dose criteria. Added requirement for positive response to therapy.	06.17	08.17
4Q17 Annual Review Removed prescriber requirement.	09.11.17	11.17
2Q 2018 annual review: No significant changes. References reviewed and updated.	02.26.18	05.18
2Q 2019 annual review: no significant changes; references reviewed and updated.	02.27.19	05.19

Reviews, Revisions, and Approvals	Date	P&T Approval Date
2Q 2020 annual review: no significant changes; references reviewed and updated.	02.04.20	05.20
2Q 2021 annual review: added a requirement for a clinical geneticist specialist and no concomitant use with Galafold, in line with the previously P&T-approved approach for Fabry disease for Galafold; RT4: updated age limit to ≥ 2 years of age per newly FDA-approved pediatric extension; references reviewed and updated.	03.15.21	05.21

Important Reminder
This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.