Clinical Policy Title:	agalsidase beta
Policy Number:	RxA.117
Drug(s) Applied:	Fabrazyme®
Original Policy Date:	02/07/2020
Last Review Date:	03/09/2021
Line of Business Policy Applies to:	All Line of Business

Background

Fabrazyme® is a recombinant human alpha-galactosidase A enzyme. It is indicated for the treatment of Fabry disease.

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
agalsidase beta (Fabrazyme®)	Fabry disease	1 mg/kg body weight IV every 2 weeks	1 mg/kg/2 weeks

Dosage Forms

- Single-use vial: 5 mg, 35 mg

Clinical Policy

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

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. Age 8 years of years or age;
3. Dose does not exceed 1 mg/kg body weight every 2 weeks.

Approval Duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

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

1. Member is currently receiving medication that has been authorized by RxAdvance or the member has met initial approval criteria listed in this policy;
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/kg body weight every 2 weeks.

Approval Duration
Commercial: 6 months
Medicaid: 6 months

III. Appendices

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

References

1. Fabrazyme® Prescribing Information. Cambridge, MA: Genzyme Corporation; December 2018. Available at http://www.fabrazyme.com. Accessed January 20, 2021.
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. Accessed January 20, 2021.
3. Desnick RJ, Brady RO. Fabry disease in childhood. J Pediatr. 2004; 144(5 Suppl): S20-S26. Accessed January 20, 2021.
4. Ortiz A, Germain DP, Desnick RJ, Politei J, Mauer M, Burlina A, Eng C, Hopkin RJ, Laney D, Linhart A, Waldek S, Wallace E, Weidemann F, Wilcox WR. Fabry disease revisited: Management and treatment recommendations for adult patients. Mol Genet Metab. 2018 Apr;123(4):416-427. doi: 10.1016/j.ymgme.2018.02.014. Epub 2018 Feb 28. Review. PubMed PMID: 29530533. Accessed January 20, 2021.

Review/Revision History	Review/Revised Date	P&T Approval Date
Policy was established	01/2020	02/07/2020
Updated references	04/30/2020	05/20/2020

Policy was reviewed: 1. Clinical policy title table was updated. 2. Drug(s) applied was updated. 3. Line of Business Policy Applies to was update to all lines of business. 4. Continued Therapy criteria II.A.1 was rephrased to "Currently receiving medication that has been authorized by RxAdvance..." 5. Initial Approval criteria: Commercial and Medicaid approval duration were updated from length of benefit to 6 months. 6. Continued Approval criteria: Commercial and Medicaid approval duration were updated from length of benefit to 6 months. 7. References were reviewed and updated. 8. Updated dosing regimen to add body weight. 9. Updated Initial and Continued Therapy to include body weight.	01/20/2021	03/09/2021