Clinical Policy: Agalsidase Beta (Fabrazyme)

Reference Number: LA.PHAR.158
Effective Date:
Last Review Date: 04.22
Line of Business: Medicaid

Coding Implications
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

Prior Authorization is required. 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 Louisiana Healthcare Connections 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®;**
5. **Dose does not exceed 1 mg/kg every 2 weeks.**

Approval duration:
Medicaid – 6 months

B. Other diagnoses/indications

1. **Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): LA.PMN.53 for Medicaid.**

II. Continued Therapy

A. Fabry Disease (must meet all):

1. **Currently receiving medication via Louisiana Healthcare Connections benefit 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/kg every 2 weeks.**

Approval duration:
Medicaid – 12 months

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

1. **Currently receiving medication via Louisiana Healthcare Connections benefit and documentation supports positive response to therapy.**
   Approval duration: Duration of request or 6 months (whichever is less); or
2. **Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): LA.PMN.53 for Medicaid.**

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 –LA.PMN.53 for Medicaid.

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**
- **Cornea 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 vials: 5 mg, 35 mg

VII. References

1. **Fabrazyme Prescribing Information. Cambridge, MA: Genzyme Corporation; March 2021. Available at http://www.fabrazyme.com. Accessed February 14, 2022.**
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.**

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	LDH Approval Date
Converted corporate to local policy.	04.22	

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. LHCC makes no representations and accepts no liability with respect to the content of any external

information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable LHCC administrative policies and procedures.

This clinical policy is effective as of the date determined by LHCC. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. LHCC retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. 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. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

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