# Clinical Policy: Pegunigalsidase Alfa-iwxj (Elfabrio)

Reference Number: LA.PHAR.512
Effective Date: 05.10.24
Last Review Date: 07.10.25~~12.11.24~~ Coding Implications
Line of Business: Medicaid Revision Log

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

****Please note: This policy is for medical benefit****

**Description**
Pegunigalsidase alfa-iwxj (Elfabrio®) is a hydrolytic lysosomal neutral glycosphingolipid-specific enzyme.

**FDA Approved Indication(s)**
Elfabrio is indicated for the treatment of adults 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 Louisiana Healthcare Connections® that Elfabrio 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 ≥ 18 years;
4. Elfabrio is not prescribed concurrently with Fabrazyme® or Galafold®;
~~4.~~5. Documentation of member's current weight (in kg);
~~5.~~6. Dose does not exceed 1 mg/kg every 2 weeks.

**Approval duration: 6 months**

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

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to LA.PMN.255~~; or~~
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND

criterion 1 above does not apply, refer to the off-label use policy ~~for the relevant line of business:~~ LA.PMN.53 ~~for Medicaid~~.

**II. Continued Therapy**

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

~~1.~~a. Member is currently receiving medication via Louisiana Healthcare Connections benefit or member has previously met initial approval criteria;

~~2.~~1. Member is responding positively to therapy as evidenced by improvement in the individual member's Fabry disease manifestation profile *(see Appendix D for examples)*;

2. Elfabrio is not prescribed concurrently with Fabrazyme® or Galafold®;

3. Documentation of member's current weight (in kg);

~~3.~~4. 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. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to LA.PMN.255~~; or~~
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy ~~for the relevant line of business:~~ LA.PMN.53.

**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~~ies~~ – LA.PMN.53 ~~for Medicaid, 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*

- Contraindication(s): none
- Boxed warning(s): hypersensitivity reactions including anaphylaxis

*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:

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- 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

Injection solution in a single-dose vial: 20 mg/10 mL, 5 mg/2.5 mL

## VII. References

1. Elfabrio Prescribing Information. Parma, Italy: Chiesi Farmaceutici; May 2024. Available at: https://resources.chiesiusa.com/Elfabrio/ELFABRIO_PI.pdf. Accessed ~~June 6, 2024~~March 10, 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 Apr;123(4):416-27.
3. Linhart A, Nicholls K, West M, et al. Pegunigalsidase alfa for the treatment of Fabry disease – Phase III open label, switch-over study from agalsidase alfa – preliminary results. Poster abstract.
4. Schiffmann R, Goker-Alpan O, Holida M, et al. Pegunigalsidase alfa, a novel PEGylated enzyme replacement therapy for Fabry disease, provides sustained plasma concentrations and favorable pharmacodynamics: a 1-year Phase 1/2 clinical trial. J Inherit Metab Dis. 2019;42:534-44.
5. 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.
6. 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.
7. 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.

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## 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 |
|---|---|
| J2508 | Injection, pegunigalsidase alfa-iwxj, 1 mg |

| Reviews, Revisions, and Approvals | Date | LDH Approval Date |
|---|---|---|
| Converted corporate to local policy. | 04.04.24 | 05.10.24 |
| No significant changes; references reviewed and updated. | 12.11.24 | 02.24.25 |
| Annual review: added concomitant use exclusion to the Continued Therapy section to echo the exclusion which currently exists in the Initial Approval Criteria; added requirement for documentation of member's weight for dose calculation purposes; references reviewed and updated. | 07.10.25 | |

**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.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom LHCC has no control or right of control. Providers are not agents or employees of LHCC.

This clinical policy is the property of LHCC. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.