

Medical Policy Manual **Approved Rev: Do Not Implement until 12/1/26**

Pegunigalsidase Alfa-iwxj (Elfabrio®)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Elfabrio is indicated for the treatment of adults with confirmed Fabry disease.

All other indications are considered experimental/investigational and not medically necessary.

DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

Initial Requests:

- Alpha-galactosidase enzyme assay or genetic testing results supporting diagnosis. In the case of obligate carriers, the documentation must be submitted for the parent.
- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g., acroparesthesias, angiokeratomas, gastrointestinal symptoms, corneal opacities).

Continuation Requests:

- Chart notes or medical records documenting a positive response to therapy (e.g., reduction in plasma globotriaosylceramide [GL-3, Gb3], GL-3/Gb3 inclusions or plasma lyso-GL-3, improvement and/or stabilization in renal function, pain reduction).

PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

COVERAGE CRITERIA

Fabry Disease

Authorization of 12 months may be granted for treatment of Fabry disease when all of the following criteria are met:

- Member is 18 years of age or older.
- The diagnosis of Fabry disease was confirmed by enzyme assay demonstrating a deficiency of alpha-galactosidase enzyme activity or by genetic testing, or the member is a symptomatic obligate carrier.

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- The member exhibits clinical signs and symptoms of the disease at baseline (e.g., acroparesthesias, angiokeratomas, gastrointestinal symptoms, corneal opacities).
- The requested medication will not be used in combination with Galafold **or Fabrazyme**.

CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria who are responding to therapy (e.g., reduction in plasma globotriaosylceramide [GL-3, Gb3], GL-3/Gb3 inclusions **or plasma lyso-GL-3**, improvement and/or stabilization in renal function, pain reduction).

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. Elfabrio [package insert]. Cary, NC: Chiesi USA, Inc.; May 2024.
2. Biegstraaten M, Arngimsson R, Barbey F, et al. Recommendations for initiation and cessation of enzyme replacement therapy in patients with Fabry disease: the European Fabry Working Group consensus document. *Orphanet J Rare Dis*. 2015; 1036.
3. Ortiz A, Germain DP, Desnick RJ, et al. Fabry disease revisited: Management and treatment recommendations for adult patients. *Mol Genet Metab*. 2018;123(4):416-427.
4. **Mauer M, Wallace E, Schiffmann R. Fabry disease: Clinical features and diagnosis. UpToDate. Updated June 22, 2025. Accessed February 2, 2026.**

EFFECTIVE DATE 12/1/2026

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