

Specialty Guideline Management

Elfabrio

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Elfabrio	pegunigalsidase alfa-iwxj

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 Indications¹

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 Disease¹⁻⁴

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.
- 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).

Reference number(s)
5934-A

References

1. Elfabrio [package insert]. Cary, NC: Chiesi USA, Inc.; May 2024.
2. Biegstraaten M, Arnglimsson 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.