Reference number(s)
5935-A

Standard Medicare Part B 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 Indications[1]

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

All other indications will be assessed on an individual basis. Submissions for indications other than those listed in this criteria should be accompanied by supporting evidence from Medicare approved compendia.

Documentation

The following documentation must be available, upon request, for all submissions:

- 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.
- Continuation requests: lab results or chart notes documenting a benefit from therapy.

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Coverage Criteria

Fabry Disease[1-3]

Authorization of 12 months may be granted for treatment of Fabry disease when both 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.

Continuation of Therapy

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization of 12 months may be granted when all of the following criteria are met:

- The member is currently receiving therapy with the requested medication.
- The requested medication is being used to treat an indication listed in the coverage criteria.
- The member is receiving benefit from therapy (e.g., reduction in plasma globotriaosylceramide [GL-3, Gb3] or GL-3/Gb3 inclusions, improvement and/or stabilization in renal function, pain reduction).

Summary of Evidence

The contents of this policy were created after examining the following resources:

- The prescribing information for Elfabrio.
- The available compendium
    - National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    - Micromedex DrugDex
    - American Hospital Formulary Service- Drug Information (AHFS-DI)
    - Lexi-Drugs
    - Clinical Pharmacology
- Recommendations for initiation and cessation of enzyme replacement therapy in patients with Fabry disease: the European Fabry Working Group consensus document.
- Fabry disease revisited: Management and treatment recommendations for adult patients.

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After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Elfabrio are covered.

Explanation of Rationale

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

The diagnosis of Fabry disease can be confirmed using several methods. According to Biegstraaten et al, a definite diagnosis of Fabry disease is made when the patient has a GLA mutation, deficiency of alpha-enzyme activity in leukocytes and one of the following: one or more characteristic sign(s) of Fabry disease (Fabry neuropathic pain, cornea verticillata or clustered angiokeratoma), an increase in plasma (lyso)Gb3, or a family member with a definite Fabry disease diagnosis carrying the same GLA mutation.

Additionally, Ortiz et al report a larger group of patients have later-onset phenotypes and varying levels of residual alpha-galactosidase enzyme activity. Severe clinical manifestations have been reported in at least 43% of obligate carrier women. Enzyme replacement therapy should be considered in these cases if there is laboratory, histological, or imaging evidence of injury to the kidney, heart or central nervous system regardless of the alpha-galactosidase enzyme activity.

References

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

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