Medical Drug Clinical Criteria

Subject:	Elfabrio (pegunigalsidase alfa-iwxj)		
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Overview

This document addresses the clinical indications for Elfabrio (pegunigalsidase alfa-iwxj), a hydrolytic lysosomal neutral glycospingolipid-specific enzyme. Elfabrio (pegunigalsidase alfa-iwxj) is an enzyme replacement therapy (ERT) approved for the treatment of individuals with a lipid storage disorder called Fabry disease.

Fabry disease is an X-linked lysosomal (lipid) storage disorder related to a deficiency of the enzyme alpha-galactosidase A (α-Gal-A, also known as ceramide trihexosidase) required to metabolize lipids. Signs and symptoms of Fabry disease include burning sensations in the arms and legs (that worsens with exercise and hot weather), small, non-cancerous, raised reddish-purple blemishes on the skin, and clouding of the corneas. Other symptoms include decreased sweating, fever, and gastrointestinal difficulties. Lipid storage may lead to breathing and digestive problems, impaired circulation, and increased risk of cardiomyopathy, cerebrovascular accidents, and renal failure.

The American College of Medical Genetics (ACMG) (2011) and National Society of Genetic Counselors (NSGC) (2013) recommend screening for deficient α-Gal-A enzyme activity in males followed by confirmatory galactosidase alpha (*GLA*) gene sequencing. As α-Gal-A activity is unreliable in females, GLA gene sequencing should be performed for a confirmatory diagnosis.

ACMG states ERT is the standard of care for symptomatic individuals as it has shown improvements in the rate of renal dysfunction, pulmonary and gastrointestinal symptoms.

Elfabrio has a black box warning for hypersensitivity reactions, including anaphylaxis, and recommendations for appropriate medical support to be readily available during administration (i.e., cardiopulmonary resuscitation equipment). If severe hypersensitivity occurs, immediate discontinuation is recommended. A desensitization procedure may also be considered in these individuals.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Elfabrio (pegunigalsidase alfa-iwxj)

Initial requests for Elfabrio (pegunigalsidase alfa-iwxj) may be approved if the following criteria are met:

I. Documentation is provided that individual has a diagnosis of Fabry disease as defined with either of the following (ACMG, NSGC):
   A. Documentation of complete deficiency or less than 5% of mean normal alpha-galactosidase A (α-Gal A) enzyme activity in leukocytes, dried blood spots, or serum (plasma) analysis; **OR**
   B. Documented galactosidase alpha gene mutation by gene sequencing;

AND

II. The individual to be treated has one or more symptoms, or physical findings attributable to Fabry disease (ACMG), including, but not limited to:
   A. Burning pain in the extremities (acroparesthesias); **OR**
   B. Cutaneous vascular lesions (angiokeratomas); **OR**
   C. Corneal verticillata (whorls); **OR**
   D. Decreased sweating (anhidrosis or hypohidrosis); **OR**
   E. Personal or family history of exercise, heat, or cold intolerance; **OR**
   F. Personal or family history of kidney failure.

Continuation requests for Elfabrio (pegunigalsidase alfa-iwxj) may be approved if the following criteria are met:

I. Individual has had a positive therapeutic response to treatment.

Elfabrio (pegunigalsidase alfa-iwxj) may not be approved for the following:

I. Individual is using in combination with migalastat (Galafold) or Fabrazyme (agalsidase beta); **OR**
II. When the above criteria are not met and for all other indications.

Quantity Limits

Elfabrio (pegunigalsidase alfa-iwxj) Quantity Limits

Drug	Limit
Elfabrio (pegunigalsidase alfa-iwxj) 20 mg/10 mL vial	1 mg/kg every two weeks

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J3490	Unclassified drugs (when specified as [Elfabrio] (pegunigalsidase alfa-iwxj))
J3590	Unclassified biologics (when specified as [Elfabrio] (pegunigalsidase alfa-iwxj))

ICD-10 Diagnosis

All diagnoses pend

Document History

Reviewed: 09/11/2023
Document History:

- 09/11/2023 – Annual Review: No changes. Coding Reviewed: No changes.
- 06/12/2023 – Select Review: Add new clinical criteria document for Elfabrio. Coding Reviewed: Added HCPCS J3490, J3590. All diagnoses pend.

References

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7. Ortiz A, Germain D, Desnick R, et al. Fabry disease revisited: Management and treatment recommendations for adult patients. Mol Gen Metab. 2018;123(4):416-427. Accessed on September 7, 2023.
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Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.