Elfabrio (pegunigalsidase alfa-iwxj)
PAM – 071

Iowa Medicaid Program	Prior Authorization	**Effective Date**	01/01/2024
Revision Number	3	**Last Reviewed**	04/17/2026
Reviewed By	Medicaid Medical Director	**Next Review**	04/16/2027
Approved By	Medicaid Clinical Advisory Committee	**Approved Date**	04/19/2024

Overview

Medication: [1]	pegunigalsidase alfa-iwxj
Brand Name:	Elfabrio®
Pharmacologic Category:	Hydrolytic lysosomal neutral glycosphingolipid-specific enzyme
FDA-Approved Indication(s):	Treatment of adults with confirmed Fabry disease
How Supplied:	• Single-dose vial, 20 mg/10 mL (provided in carton of 1, 5, or 10 vials) • Single-dose vial, 5 mg/2.5 mL (provided in carton of 1 vial)
Dosage and Administration:	Intravenous (IV) infusion: 1 mg/kg every 2 weeks (based on actual body weight)
Benefit Category:	Medical

BOXED WARNING: HYPERSENSITIVITY REACTIONS INCLUDING ANAPHYLAXIS

Patients treated with Elfabrio have experienced hypersensitivity reactions, including anaphylaxis. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during Elfabrio administration. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, discontinue Elfabrio immediately and initiate appropriate medical treatment. In patients with severe hypersensitivity reaction, a desensitization procedure to Elfabrio may be considered.

Descriptive Narrative

Lysosomal storage diseases are inherited metabolic diseases that are characterized by an abnormal build-up of various toxic materials in the body's cells as a result of enzyme deficiencies. There are nearly 50 of these disorders altogether, and they may affect different parts of the body, including the skeleton, brain, skin, heart, and central nervous system. There is no cure for lysosomal storage disorders, and there are not yet specific treatments for many of these diseases. However, progress is being made in the search for therapies, and there are treatments available such as enzyme replacement therapy (ERT)

which have been proven effective for some lysosomal storage disorders that greatly improve the quality of life for those affected.

Although the signs and symptoms vary from disease to disease in this group, symptoms occur in each case because of an enzyme deficiency that inhibits the ability of the lysosomes present in each of the body's cells to perform their normal function. The lysosomes function as the primary digestive units within cells. Their function is to break down complex components into simpler ones. Each cell has hundreds of lysosomes that degrade complex cellular components such as proteins (substrates) into simpler components. When this process does not take place, the substrate begins to accumulate in the cells (hence the name "storage diseases"). The symptoms of lysosomal storage disorders are generally progressive over a period of time.[2]

Fabry Disease

Fabry disease is a rare X-linked recessive lysosomal storage disorder and is the second most prevalent lysosomal disease after Gaucher disease. It is caused by decreased activity of alpha-galactosidase A and results in lysosomal accumulations of neutral glycosphingolipids and globotriaosylceramide GL-3. The major debilitating manifestations of Fabry disease result from the progressive accumulation of globotriaosylceramide in the vascular endothelium, leading to ischemia and infarction, especially in the kidney, heart and brain.

Patients with Fabry disease may present with a spectrum of clinical manifestations, ranging from the severe classic phenotype in males to asymptomatic disease in some females, with a variety of clinical presentations in between. The classic form of Fabry disease is the most severe clinical phenotype and occurs predominantly in males. Clinical manifestations begin in childhood or adolescence and include severe neuropathic or limb pain, telangiectasias and angiokeratomas, gastrointestinal symptoms, corneal opacities, kidney manifestations, and other nonspecific manifestations such as heat, cold, and exercise intolerance, dry mouth, hearing loss, tinnitus, and vertigo. In adulthood, there may be progressive cardiac and cerebrovascular involvement, and cardiac complications account for the majority of deaths associated with Fabry disease.[3]

Guidelines

In the United States, the National Society of Genetic Counselors (NSGC) published a practice resource for Fabry disease in 2013, with a focused revision published in 2020.[4,5] The resource states, "The progressive nature of Fabry disease requires at least annual evaluation and revision of management based on clinical and lab assessments by the appropriate medical professionals."

Once a diagnosis of Fabry disease is made, the following steps are recommended:

1. Referrals by appropriate medical professionals to a metabolic specialist and genetic counselor for discussion of diagnosis, recurrence risk, construction of a detailed family history, identification of other at-risk family members, and development of a comprehensive monitoring and treatment plan.
2. Baseline evaluations to be ordered by and under the supervision of appropriate medical professionals as recommended for age group.
3. Discussion with appropriate medical professionals of treatment with enzyme replacement therapy (ERT) treatment practices vary widely in recommended timing of beginning ERT. The decision to initiate therapy should be determined based on the clinical judgment of the managing metabolic specialist after reviewing baseline evaluations in conjunction with the patient or patient's family in affected minors.

The focused revision published in 2020 discusses the first oral therapy approved for Fabry disease (migalastat), approved by the FDA in August 2018 under the proprietary name Galafold®. Newborn screening is also discussed.

Criteria

Prior authorization is required.

Elfabrio® is considered medically necessary when **ALL** of the following are met:

1. Diagnosis of Fabry disease confirmed by one of the following (a or b):
   a. Enzyme assay demonstrating a deficiency of alpha-galactosidase activity; or
   b. Genetic testing; **AND**
2. Member is 18 years of age or older; **AND**
3. Elfabrio® is not prescribed concurrently with agalsidase beta (Fabrazyme®) or migalastat (Galafold®); **AND**
4. Prescribed by, or in consultation with, a clinical geneticist, cardiologist, endocrinologist, nephrologist, neurologist, lysosomal disease specialist, or Fabry disease specialist; **AND**
5. Request meets one of the following (a or b):
   a. Regimen prescribed does not exceed 1 mg/kg every 2 weeks; or
   b. Regimen is supported by clinical practice guidelines. Supporting clinical documentation must be provided with any request for which regimen prescribed does not align with FDA-approved labeling.

Elfabrio® is considered medically necessary for continuation of therapy when **ALL** of the following are met:

1. Member is currently receiving medication through the Iowa Medicaid benefit or has previously met initial approval criteria; **AND**
2. Documentation of positive clinical response to therapy (e.g., improvement and/or stabilization in renal function, pain reduction, or other positive therapeutic response); **AND**
3. Elfabrio® is not prescribed concurrently with agalsidase beta (Fabrazyme®) or migalastat (Galafold®); **AND**
4. Prescribed by, or in consultation with, a clinical geneticist, cardiologist, endocrinologist, nephrologist, neurologist, lysosomal disease specialist, or Fabry disease specialist; **AND**
5. Request meets one of the following (a or b):
    a. Regimen prescribed does not exceed 1 mg/kg every 2 weeks; or
    b. Regimen is supported by clinical practice guidelines. Supporting clinical documentation must be provided with any request for which regimen prescribed does not align with FDA-approved labeling.

Approval Duration and Quantity Limits

	Initial Authorization	Subsequent Authorization(s)
Approval Duration	12 months	12 months
Quantity Limits	1 mg/kg every 2 weeks (based on actual body weight)	

Coding and Product Information

The following list(s) of codes and product information are provided for reference purposes only and may not be all inclusive. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment, nor does the exclusion of a code imply that its association to the HCPCS code is inappropriate.

HCPCS	Description
J2508	Injection, pegunigalsidase alfa-iwxj, 1 mg

ICD-10	Description
E75.21	Fabry (-Anderson) disease

NDC (Strength)	Labeler	Dosage	Pkg Size	Pkg Qty	Units/ Pkg
10122-0160-02 (20 mg/10 mL single-dose vial; carton contains 1 vial)	Chiesi USA, Inc. (10122)	1 mg	1	EA	20
10122-0160-05 (20 mg/10 mL single-dose vial; carton contains 5 vials)	Chiesi USA, Inc. (10122)	1 mg	1	EA	100
10122-0160-10 (20 mg/10 mL single-dose vial; carton contains 10 vials)	Chiesi USA, Inc. (10122)	1 mg	1	EA	200
10122-0165-02 (5 mg/2.5 mL single-dose vial; contain contains 1 vial)	Chiesi USA, Inc. (10122)	1 mg	1	EA	5

Compliance

1. Should conflict exist between the policy and applicable statute, the applicable statute shall supersede.
2. Federal and State law, as well as contract language, including definitions and specific contract provisions or exclusions, take precedence over medical policy and must be considered first in determining eligibility for coverage.
3. Medical technology is constantly evolving, and Iowa Medicaid reserves the right to review and update medical policy on an annual or as-needed basis.

Medical necessity guidelines have been developed for determining coverage for member benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. Medical necessity guidelines are developed for selected physician-administered medications found to be safe and proven to be effective in a limited, defined population or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. Criteria are revised and updated annually, or more frequently if new evidence becomes available that suggests needed revisions.

References

[1] Elfabrio® prescribing information (05/2024). Chiesi USA, Inc.: Cary, NC. Available online: hcp.elfabrio.com. Accessed January 27, 2026.

[2] Sutton VR. Inborn errors of metabolism: Classification. Kremen J, ed. UpToDate. Waltham, MA: UpToDate Inc. www.uptodate.com. Accessed February 26, 2025.

[3] Mauer M, Wallace E, Schiffmann R. Fabry disease: Clinical features and diagnosis. Lam AQ, ed. UpToDate. Waltham, MA: UpToDate Inc. www.uptodate.com. Accessed February 26, 2025.

[4] Laney DA, Bennett RL, et al. Fabry disease practice guidelines: recommendations of the National Society of Genetic Counselors. J Genet Couns. 2013 Oct;22(5):555-64. Epub 2013 Jul 17. PMID: 23860966.

[5] Henderson N, Berry L, Laney DA. Fabry Disease practice resource: Focused revision. J Genet Couns. 2020 Oct;29(5):715-717. Epub 2020 Sep 3. PMID: 32885538.

Development of utilization management criteria may also involve research into other state Medicaid programs, other payer policies, consultation with experts and review by the Medicaid Clinical Advisory Committee (CAC). These sources may not be referenced individually unless they are specifically published and are otherwise applicable to the criteria at issue.

Criteria Change History

Change Date	Changed By	Description of Change	Version
[mm/dd/yyyy]	CAC		
Signature			

Change Date	Changed By	Description of Change	Version
[mm/dd/yyyy]	CAC		
Signature			

Change Date	Changed By	Description of Change	Version
04/17/2026	CAC	Annual review. No changes.	3
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/18/2025	CAC	Annual review. New vial size available; updated Coding and Product Information section.	2
Signature William (Bill) Jagiello, DO			

Change Date	Changed By	Description of Change	Version
04/19/2024	CAC	Criteria implementation.	1
Signature William (Bill) Jagiello, DO			

CAC = Medicaid Clinical Advisory Committee