Original Effective Date: 09/06/2023
Current Effective Date: 09/19/2026
Last P&T Approval/Version: 07/29/2026
Next Review Due By: 07/2027
Policy Number: C25604-A

Elfabrio (pegunigalsidase alfa)

PRODUCTS AFFECTED

Elfabrio (pegunigalsidase alfa)

COVERAGE POLICY

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Coverage Guideline must be read in its entirety to determine coverage eligibility, if any. This Coverage Guideline provides information related to coverage determinations only and does not imply that a service or treatment is clinically appropriate or inappropriate. The provider and the member are responsible for all decisions regarding the appropriateness of care. Providers should provide Molina Healthcare complete medical rationale when requesting any exceptions to these guidelines.

Documentation Requirements:
Molina Healthcare reserves the right to require that additional documentation be made available as part of its coverage determination; quality improvement; and fraud; waste and abuse prevention processes. Documentation required may include, but is not limited to, patient records, test results and credentials of the provider ordering or performing a drug or service. Molina Healthcare may deny reimbursement or take additional appropriate action if the documentation provided does not support the initial determination that the drugs or services were medically necessary, not investigational or experimental, and otherwise within the scope of benefits afforded to the member, and/or the documentation demonstrates a pattern of billing or other practice that is inappropriate or excessive.

DIAGNOSIS:
Fabry disease

REQUIRED MEDICAL INFORMATION:
This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. This clinical policy will be reviewed along with state and federal requirements, the benefit being administered, and formulary preferencing. If a drug within this policy receives an updated FDA label within the last 180 days, medical necessity for the member will be reviewed using the updated FDA label information. Coverage will be determined on a case-by-case basis until the criteria can be updated through Molina Healthcare, Inc. clinical governance. Additional information may be required on a case-by-case basis to allow for adequate review. When the requested drug product for coverage is dosed by weight, body surface area or other member specific measurement, this data element is required as part of the medical necessity review. The Pharmacy and Therapeutics Committee has determined that the drug benefit shall be a mandatory generic and that generic drugs will be dispensed whenever available. The Pharmacy and Therapeutics Committee has determined that biosimilars may be preferred.

A. FABRY DISEASE:
1. (a) Documented diagnosis of classic Fabry disease with typical clinical manifestations confirmed by documented deficient α-galactosidase A (α-Gal A) enzyme activity in plasma, isolated leukocytes,

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and/or cultured cells using alpha galactosidase A enzyme assay (Males with classic Fabry disease have less than 1% α-Gal A enzyme activity; Males with atypical Fabry disease have residual enzyme activity that is greater than 1% of normal) OR Molecular genetic testing that identifies a GLA mutation providing additional confirmation of the diagnosis [DOCUMENTATION REQUIRED]
OR
(b) Diagnosed as a carrier of Fabry disease with significant clinical manifestations, confirmed by documented decrease α-Gal A enzyme activity in plasma and/or isolated leukocytes confirms the carrier state in a female OR documented molecular genetic testing to clarify genetic status [DOCUMENTATION REQUIRED]
AND

2. (a) Documentation of baseline status by Mainz Severity Score Index (MSSI) or FOS Mainz Severity Score Index
   OR
   (b) Documentation of objective/subjective clinical information, including signs/symptoms, with sufficient clinical manifestations to justify treatment and supported by at least one of the following: pain in the extremities, hypohidrosis, corneal opacities, kidney dysfunction, cardiac dysfunction, cerebrovascular disorders
   OR
   (c) Documentation of baseline plasma globotriaosylceramide (GL3 or Gb3) level
   AND
3. Documentation of treatment plan that includes goals of therapy
   AND
4. Not intended for concurrent therapy with Galafold (migalastat) or other enzyme replacement therapy for Fabry disease [agalsidase beta (Fabrazyme)]

CONTINUATION OF THERAPY:

A. FABRY DISEASE:

1. Documentation of ANY of the following:
   a. Improvement or stabilization of Mainz Severity Score Index (MSSI) or FOS Mainz Severity Score Index
      OR
   b. A marked clinical improvement in or a stabilization in disease progression from baseline disease manifestations (e.g., kidney dysfunction, cardiac dysfunction, etc.)
      OR
   c. Disease response as evidenced by a reduction in plasma GL-3 and/or GL-3 inclusions compared to pre-treatment baseline

   AND
2. The member is not receiving concurrent therapy with Galafold (migalastat) or other enzyme replacement therapy for Fabry disease [agalsidase beta (Fabrazyme)]
   AND
3. Prescriber attests to or clinical reviewer has found no evidence of intolerable adverse effects or drug toxicity (e.g., severe hypersensitivity reactions, severe infusion site reactions)

DURATION OF APPROVAL:

Initial authorization: 12 months, Continuation of Therapy: 12 months

PRESCRIBER REQUIREMENTS:

Prescribed by or in consultation with a board-certified nephrologist, cardiologist, neurologist, endocrinologist, clinical geneticist, clinical biochemical geneticist, or physician experienced in the management of Fabry disease. [If prescribed in consultation, consultation notes must be submitted with initial request and reauthorization requests]

AGE RESTRICTIONS:

18 year of age and older

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HIGH RISK ALERT

QUANTITY:
1 mg/kg actual body weight every 2 weeks

PLACE OF ADMINISTRATION:
The recommendation is that infused medications in this policy will be for pharmacy or medical benefit coverage administered in a place of service that is a non-inpatient hospital facility-based location as per the Molina Health Care Site of Care program.

Note: Site of Care Utilization Management Policy applies for Elfabrio (pegunigalsidase alfa) to channel to the prescription drug benefit for member self-administration as appropriate. For information on site of care, see Specialty Medication Administration Site of Care Coverage Criteria (molinamarketplace.com)

DRUG INFORMATION

ROUTE OF ADMINISTRATION:
Intravenous

DRUG CLASS:
Fabry Disease - Agents

FDA-APPROVED USES:
Indicated for the treatment of adults with confirmed Fabry disease

E75.21 Fabry-Anderson disease

COMPENDIAL APPROVED OFF-LABELED USES:
None

APPENDIX

APPENDIX:
None

BACKGROUND AND OTHER CONSIDERATIONS

BACKGROUND:
Fabry disease is an X-linked lysosomal storage disorder that is caused by deficient activity of the α–galactosidase–A enzyme, resulting in the accumulation of globotriaosylceramide (GL-3) within lysosomes in a wide variety of cells. This accumulation of GL-3 in blood vessels, kidneys, heart, nerves, and other organs leads to cell damage, with consequences ranging from episodes of pain and impaired peripheral sensation to end-stage organ failure. Fabry disease is characterized by a mutation in the GLA gene.

Elfabrio (pegunigalsidase alfa-iwxj) injection is a pegylated enzyme replacement therapy (ERT). The approval of Elfabrio was based on safety, tolerability, and efficacy results from a comprehensive clinical development program, which included more than 140 patients with up to 7.5 years of follow-up treatment (the BALANCE, BRIDGE, AND BRIGHT trials). Elfabrio was studied in both ERT-naïve and ERT-experienced patients. A head-to-head trial versus agalsidase beta (Fabrazyme) in ERT-experienced patients met its primary efficacy endpoint, demonstrating that Elfabrio was noninferior to agalsidase beta in controlling estimated glomerular filtration rate (eGFR) decline. In this study, most common adverse reactions (≥15%) reported with Elfabrio therapy are infusion-associated reactions, nasopharyngitis, headache, diarrhea, fatigue, nausea, back pain, pain in extremities, and sinusitis.

CONTRAINDICATIONS/EXCLUSIONS/DISCONTINUATION:

HIGH RISK ALERT

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All other uses of Elfabrio (pegunigalsidase alfa) are considered experimental/investigational and therefore, will follow Molina's Off- Label policy. Contraindications to Elfabrio (pegunigalsidase alfa) include: No labeled contraindications.

OTHER SPECIAL CONSIDERATIONS:
Elfabrio has a Black Box Warning for hypersensitivity reactions including anaphylaxis. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available. If a severe hypersensitivity reaction occurs, discontinue Elfabrio immediately and initiate appropriate medical treatment.

If one or more doses are missed, restart Elfabrio treatment as soon as possible, maintaining the 2 week interval between infusions thereafter. Do not double a dose to compensate for a missed dose.

CODING/BILLING INFORMATION

CODING DISCLAIMER. *Codes listed in this policy are for reference purposes only and may not be all-inclusive or applicable for every state or line of business. Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement. Listing of a service or device code in this policy does not guarantee coverage. Coverage is determined by the benefit document. Molina adheres to Current Procedural Terminology (CPT®), a registered trademark of the American Medical Association (AMA). All CPT codes and descriptions are copyrighted by the AMA; this information is included for informational purposes only. Providers and facilities are expected to utilize industry-standard coding practices for all submissions. Molina has the right to reject/deny the claim and recover claim payment(s) if it is determined it is not billed appropriately or not a covered benefit. Molina reserves the right to revise this policy as needed.*

HCPCS CODE	DESCRIPTION
J2508	Injection, pegunigalsidase alfa-iwxj, 1 mg

AVAILABLE DOSAGE FORMS:
Elfabrio SOLN 20MG/10ML single-dose vial
Elfabrio SOLN 5MG/2.5ML single-dose vial

REFERENCES

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SUMMARY OF REVIEW/REVISIONS	DATE
ANNUAL REVIEW COMPLETED- No coverage criteria changes with this annual review	Q3 2026
REVISION- Notable revisions: Required Medical Information Continuation of Therapy Place of Administration References	Q3 2025
REVISION- Notable revisions: Required Medical Information Continuation of Therapy Coding/Billing Information Available Dosage Forms	Q3 2024
NEW CRITERIA CREATION	Q3 2023

HIGH RISK ALERT

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