



## PHARMACY POLICY STATEMENT TRICARE

<b>DRUG NAME</b>	<b>Enzyme Replacement Therapy (ERT) for Fabry Disease: Fabrazyme (agalsidase beta) and Elfabrio (pegunigalsidase alfa-iwxj)</b>
<b>BENEFIT TYPE</b>	Medical
<b>STATUS</b>	Prior Authorization Required

Fabrazyme is an enzyme replacement therapy (ERT) indicated for the treatment of confirmed Fabry disease, to replace the enzyme alpha-galactosidase A (alpha-Gal A).

Fabry disease, a lysosomal storage disorder, is a rare genetic disease caused by certain mutations of the *GLA* gene resulting in deficient alpha-Gal A. Normally this enzyme breaks down certain lipids in lysosomes, such as globotriaosylceramide (GL-3). Without it, GL-3 accumulates in blood vessels, the kidneys, heart, nerves, and other organs. The continuous build-up of GL-3 results in progressive cell damage and subsequent symptoms and manifestations in the affected organ systems.

Elfabrio is a "biobetter" of Fabrazyme and was designed to have an increased half-life and reduced immunogenicity.

ERT for Fabry Disease will be considered for coverage when the following criteria are met:

### Fabry Disease

For **initial** authorization:

1. For Fabrazyme: Member is at least 2 years of age OR for Elfabrio: Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a medical geneticist, nephrologist, cardiologist, neurologist, or metabolic specialist; AND
3. Member has a diagnosis of Fabry disease confirmed by genetic testing which identifies a pathogenic mutation of the *GLA* gene; AND
4. Member displays symptoms of Fabry disease (i.e., neuropathic pain, renal disease, cardiac disease, abdominal pain, impaired sweating)

NOTE: Exception-- Males with "classic" gene variants do not need to be symptomatic to qualify for treatment. Males with "non-classic" gene variants and asymptomatic females may be treated if there is evidence of injury to the heart, kidney, or central nervous system (CNS); AND

5. ERT will NOT be used in combination with Galafold.
6. **Dosage allowed/Quantity limit:** 1 mg/kg every 2 weeks as an IV infusion.

***If all the above requirements are met, the medication will be approved for 6 months.***



**For reauthorization:**

1. Chart notes must show positive clinical response such as stabilized kidney function (e.g., GFR, proteinuria), reduced plasma or tissue GL-3 levels, or other improved Fabry symptoms (such as neuropathic pain).

***If all the above requirements are met, the medication will be approved for an additional 12 months.***

**TRICARE Prime® Demo by CareSource Military & Veterans™ considers ERT for Fabry Disease not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.**

DATE	ACTION/DESCRIPTION
06/17/2021	New policy for Fabrazyme created.
11/22/2022	Annual review; added reference.
06/28/2023	Changed name of policy and added Elfabrio. Clarified note in #4.

**References:**

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11. Study of the Safety and Efficacy of PRX-102 Compared to Agalsidase Beta on Renal Function (BALANCE). ClinicalTrials.gov Identifier: NCT02795676. Updated 10/13/22. Accessed 6/16/23. Available at <https://clinicaltrials.gov/ct2/show/NCT02795676>



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