CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Marketplace

DRUG NAME	Enzyme Replacement Therapy (ERT) for Fabry Disease: Fabrazyme (agalsidase beta) and Elfabrio (pegunigalsidase alfa-iwxj)
BENEFIT TYPE	Medical
STATUS	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.

CareSource 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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