Fabrazyme (agalsidase beta) 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.

Fabrazyme (agalsidase beta) will be considered for coverage when the following criteria are met:

**Fabry Disease**

For **initial** authorization:

1. Member is at least 2 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 8 years of age or older 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 documentation of symptoms noted above that warrant treatment with ERT; AND
5. Fabrazyme will NOT be used in combination with Galafold.
6. **Dosage allowed/Quantity limit:** 1 mg/kg body weight infused every two 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.

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

CareSource considers Fabrazyme (agalsidase beta) 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.
<table>
<thead>
<tr>
<th>DATE</th>
<th>ACTION/DESCRIPTION</th>
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<tbody>
<tr>
<td>06/17/2021</td>
<td>New policy for Fabrazyme created.</td>
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<tr>
<td>11/22/2022</td>
<td>Annual review; added reference and OAC.</td>
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References:


Effective date: 04/01/2023
Revised date: 11/22/2022