

## PHARMACY POLICY STATEMENT

### Indiana Medicaid

<b>DRUG NAME</b>	<b>Fabrazyme (agalsidase beta)</b>
BILLING CODE	J0180
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.

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.**

DATE	ACTION/DESCRIPTION
06/17/2021	New policy for Fabrazyme created.
11/22/2022	Annual review; added reference.

#### References:

1. Fabrazyme (agalsidase beta) [package insert]. Cambridge, MA; Genzyme Corporation; Revised 03/2021.
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3. Hopkin RJ, Jefferies JL, Laney DA, et al. The management and treatment of children with Fabry disease: A United States-based perspective. *Mol Genet Metab*. 2016;117(2):104-113. doi:10.1016/j.ymgme.2015.10.007
4. Banikazemi M, Bultas J, Waldek S, et al. Agalsidase-beta therapy for advanced Fabry disease: a randomized trial. *Ann Intern Med*. 2007;146(2):77-86. doi:10.7326/0003-4819-146-2-200701160-00148
5. Eng CM, Guffon N, Wilcox WR, et al. Safety and efficacy of recombinant human alpha-galactosidase A replacement therapy in Fabry's disease. *N Engl J Med*. 2001;345(1):9-16. doi:10.1056/NEJM200107053450102
6. Lenders M, Brand E. Fabry Disease: The Current Treatment Landscape. *Drugs*. 2021;81(6):635-645. doi:10.1007/s40265-021-01486-1
7. Ortiz A, Germain DP, Desnick RJ, et al. Fabry disease revisited: Management and treatment recommendations for adult patients. *Mol Genet Metab*. 2018;123(4):416-427. doi:10.1016/j.ymgme.2018.02.014
8. Germain DP, Foulhoux A, Decramer S, et al. Consensus recommendations for diagnosis, management and treatment of Fabry disease in paediatric patients. *Clin Genet*. 2019;96(2):107-117. doi:10.1111/cge.13546

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