

PHARMACY POLICY STATEMENT

Ohio Medicaid

DRUG NAME	Fabrazyme (agalsidase beta)
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 and OAC.

References:

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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
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9. Ohio Administrative Code. (2022, February 23). 5160-1-01 (C) Medicaid medical necessity: definitions and principles. Retrieved February 22 2023 from codes.ohio.gov.
10. Ohio Administrative Code. (2022, July 18). 5160-26-03 Managed care: covered services. Retrieved February 22, 2023 from codes.ohio.gov.
11. Ohio Administrative Code. (2020, January 1). 5160-9-03 Pharmacy services: covered drugs and associated limitations. Retrieved February 22, 2023 from codes.ohio.gov.

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