 Approved Revision: Do Not Implement until 8/31/21

Agalsidase Beta (Fabrazyme®)

NDC CODE(S) 58468-0041-XX FABRAZYME 5MG Solution Reconstituted (GENZYME)
58468-0040-XX FABRAZYME 35MG Solution Reconstituted (GENZYME)

DESCRIPTION

Agalsidase beta is a recombinant DNA origin form of human α-galactosidase A, a lipid degrading enzyme. It has the same amino acid sequence as the native enzyme. It is used to treat Fabry disease, deficiency of the enzyme α-galactosidase A.

Fabry disease is an X-linked recessive inborn error of glycosphingolipid metabolism with an estimated frequency of about 1 in 50,000 births. The result of this mutation is progressive accumulation of glycosphingolipids in cellular lysosomes of multiple body tissues. Clinical manifestations typically begin in childhood and may include abdominal or flank pain simulating appendicitis or renal colic, angiokeratomas, hypohidrosis, corneal and lenticular opacities, vascular disease of the kidney, heart, and brain, intolerance to heat, cold, and exercise, mild proteinuria, gastrointestinal problems, and acroparesthesias. Fabry crises, lasting from minutes to several days, consist of agonizing, burning pain in the hands, feet, and proximal parts of the extremities. Affected individuals have a lifespan of 30 to 50 years, typically resultant from renal failure, hypertrophic cardiomyopathy, myocardial infarction or cerebrovascular accidents. Female carriers may be asymptomatic or may exhibit severe manifestations similar to males with classic disease.

Enzyme replacement therapy for Fabry disease with agalsidase beta has been shown to provide an exogenous source of α-galactosidase A. It can reverse histologic abnormalities as well as improve some clinical manifestations of the disease.

POLICY

- Agalsidase beta for the treatment of Fabry disease is considered ***medically necessary*** if the medical appropriateness criteria are met **(See Medical Appropriateness below.)**
- Agalsidase beta for the treatment of other conditions/diseases is considered ***investigational.***

MEDICAL APPROPRIATENESS

INITIAL APPROVAL CRITERIA

- Patient is at least 2 years of age; **AND**

Universal Criteria

- Must not be used in combination with migalastat; **AND**

Fabry Disease (alpha-galactosidase A deficiency)

- Documented diagnosis of Fabry disease with biochemical/genetic confirmation by one of the following:
  - α-galactosidase A (α-Gal A) activity in plasma, isolated leukocytes, and/or cultured cells (males only); **OR**
  - Plasma or urinary globotriaosylceramide(Gb3/GL-3) or globotriaosylsphingosine (lyso-Gb3); **OR**
  - Detection of pathogenic mutations in the GALA/GLA gene by molecular genetic testing; **AND**
- Baseline value for plasma GL-3 and/or GL-3 inclusions

RENEWAL CRITERIA

- Patient continues to meet universal and other indication-specific relevant criteria such as concomitant therapy requirements (not including prerequisite therapy), performance status, etc. identified in Initial Approval Criteria; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include the following: anaphylaxis and severe hypersensitivity reactions, severe infusion-associated reactions, compromised cardiac function, etc.; **AND**
- Disease response with treatment as defined by a reduction in plasma GL-3 and/or GL-3 inclusions compared to pre-treatment baseline

DOSAGE/ADMINISTRATION

INDICATION	DOSE
Fabry Disease	1 mg/kg of body weight infused every two weeks as an intravenous (IV) infusion.

LENGTH OF AUTHORIZATION

Coverage will be provided for 12 months and may be renewed.

DOSING LIMITS

Max Units (per dose and over time) [HCPCS Unit]:

- 115 billable units every 14 days

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the Medical Policy and a health plan, the express terms of the health plan will govern.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

SOURCES

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EFFECTIVE DATE 8/31/2021

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