BlueCross BlueShield
Federal Employee Program
Federal Employee Program®
750 9th St NW
Washington, D.C. 20001
202.942.1000
Fax 202.942.1125

5.30.035

Section:	Prescription Drugs	**Effective Date:**	April 1, 2024
Subsection:	Endocrine and Metabolic Drugs	**Original Policy Date:**	September 9, 2008
Subject:	Fabrazyme	**Page:**	1 of 4

Last Review Date: March 8, 2024

Fabrazyme

Description

Fabrazyme (agalsidase beta)

Background
Fabry disease is an X-linked genetic disorder of glycosphingolipid metabolism. Deficiency of the lysosomal enzyme α-galactosidase A leads to progressive accumulation of glycosphingolipids, predominantly GL-3, in many body tissues, starting early in life and continuing over decades. Clinical manifestations of Fabry disease include neuropathy, renal failure, cardiomyopathy, and cerebrovascular accidents. Accumulation of GL-3 in renal endothelial cells may play a role in renal failure (1).

Regulatory Status
FDA-approved indication: Fabrazyme is a hydrolytic lysosomal neutral glycosphingolipid-specific enzyme indicated for the treatment of adult and pediatric patients 2 years of age and older with confirmed Fabry disease (1).

Life-threatening anaphylactic and severe allergic reactions have been observed in some patients during Fabrazyme infusions. If severe allergic or anaphylactic reactions occur, immediately discontinue administration of Fabrazyme and provide necessary emergency treatment. Patients with advanced Fabry disease may have compromised cardiac function, which may predispose them to a higher risk of severe complications from infusion reactions. Appropriate medical support measures should be readily available when Fabrazyme is administered because of the potential for severe infusion reactions (1).

The safety and effectiveness of Fabrazyme in pediatric patients less than 2 years of age have

not been established (1).

Related policies
Elfabrio, Galafold

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Fabrazyme may be considered **medically necessary** if the conditions indicated below are met.

Fabrazyme may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 2 years of age or older

Diagnosis

Patient must have the following:

Fabry disease

Prior – Approval *Renewal* Requirements

Same as above

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Duration 2 years

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary
Fabrazyme is indicated for use in patients with Fabry disease. Fabrazyme reduces globotriaosylceramide (GL-3) deposition in capillary endothelium of the kidney and certain other cell types. Life-threatening anaphylactic and severe allergic reactions have been observed in some patients during Fabrazyme infusions. The safety and effectiveness of Fabrazyme in pediatric patients less than 2 years of age have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Fabrazyme while maintaining optimal therapeutic outcomes.

References

1. Fabrazyme [package insert]. Cambridge, MA: Genzyme Corporation; March 2023.

Policy History

Date	Action
March 2010	Age updated to current package insert recommendations. The safety and efficacy of Fabrazyme were assessed in a multi-national, multi-center, uncontrolled, open-label study in 16 pediatric patients with Fabry disease, ages 8 to 16 years. Patients younger than 8 years of age were not included in clinical studies. The safety and efficacy in patients younger than 8 years of age have not been evaluated. No new safety concerns were identified in pediatric patients in this study, and the overall safety and efficacy profile of Fabrazyme treatment in pediatric patients was found to be consistent with that seen in adults.
September 2011	Annual editorial review and reference update
September 2012	Annual editorial review and reference update
June 2013	Annual editorial review and reference update
September 2014	Annual editorial review and reference update
September 2015	Annual review
September 2016	Annual editorial review Policy number change from 5.08.07 to 5.30.35
December 2017	Annual editorial review
November 2018	Annual review
December 2019	Annual editorial review and reference update. Changed approval duration from lifetime to 2 years

December 2020	Annual review
March 2021	Annual review. Age requirement reduced from 8 years and older to 2 years and older
March 2022	Annual review
March 2023	Annual review. Changed policy number to 5.30.035
September 2023	Annual review and reference update. Per SME, added clinical manifestations of Fabry disease including neuropathy to background section
March 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 8, 2024 and is effective on April 1, 2024.