BlueCross BlueShield Federal Employee Program

FABRAZYME
(agalsidase beta)

RATIONALE FOR INCLUSION IN PA PROGRAM

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).

Fabrazyme contains a boxed warning regarding hypersensitivity reactions including anaphylaxis. Anaphylaxis has occurred during the early course of enzyme replacement therapy and after extended duration of therapy. Prior to Fabrazyme administration, consider pretreating with antihistamines, antipyretics, and/or corticosteroids. Initiate Fabrazyme in a healthcare setting with appropriate medical monitoring and support measures, including access to cardiopulmonary resuscitation equipment (1).

The safety and effectiveness of Fabrazyme in pediatric patients less than 2 years of age have not been established).

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. Fabrazyme contains a boxed warning for hypersensitivity reactions including anaphylaxis. 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; July 2024.