



## **RATIONALE FOR INCLUSION IN PA PROGRAM**

### **Background**

Naglazyme is indicated for Maroteaux-Lamy Syndrome (MPS VI), which is an inherited lysosomal storage disorder caused by the deficiency of N-acetylgalactosamine 4-sulfatase. N-acetylgalactosamine 4-sulfatase is an enzyme required for the breakdown of certain complex carbohydrates. The deficiency of this enzyme results in the accumulation of the glycosaminoglycan (GAG) substrate throughout the body. Naglazyme provides the needed enzyme that will be taken up into the lysosomes and increase the catabolism of GAG. Naglazyme has been shown to help people with MPS VI walk farther and climb more stairs (1).

### **Regulatory Status**

FDA-approved indication: Naglazyme is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for patients with Mucopolysaccharidosis VI (MPS VI; Maroteaux-Lamy syndrome). Naglazyme has been shown to improve walking and stair-climbing capacity (1).

Naglazyme contains a boxed warning for hypersensitivity reactions, including anaphylaxis. If a severe hypersensitivity reactions occurs, discontinue Naglazyme and immediately initiate appropriate medical treatment, including use of epinephrine (1).

Physicians should monitor patients for the development of immune complex mediated reactions while receiving the infusion. Appropriate medical monitoring and support measures should be available during infusion for the possible risk of acute cardio respiratory failure and respiratory support for acute respiratory complications. Pretreatment with antihistamines with or without antipyretics is recommended prior to the start of the infusion to reduce risk of reactions (1).

### **Summary**

Naglazyme is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for patients with Mucopolysaccharidosis VI (MPS VI; Maroteaux-Lamy syndrome). Naglazyme has been shown to improve walking and stair-climbing capacity. Observing of life threatening anaphylactic reactions should be done during infusions. Pretreatment with antihistamines with or without antipyretics is recommended prior to the start of infusion to reduce the risk of infusion-reactions (1).

Prior authorization is required to ensure the safe, clinically appropriate and cost effective use of



**BlueCross  
BlueShield**

Federal Employee Program.

**NAGLAZYME**  
(galsulfase)

Naglazyme while maintaining optimal therapeutic outcomes.

### **References**

1. Naglazyme [package insert]. Novato, CA. BioMarin Pharmaceutical Inc. July 2024.