BlueCross BlueShield Federal Employee Program

VIMIZIM
(elosulfase alfa)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Vimizim is an enzyme used to treat patients with Mucopolysaccharidosis Type IVA (Morquio A syndrome). Morquio A syndrome is a rare autosomal recessive lyosomal storage disease caused by a deficiency in N-acetylgalactosamine-6-sulfate sulfatase (GALNS). Vimizim is intended to replace the missing GALNS enzyme involved in an important metabolic pathway. Absence of this enzyme leads to problems with bone development, growth and mobility (1).

Regulatory Status

FDA-approved indication: Vimizim is a hydrolytic lyosomal glycosaminoglycan (GAG)-specific enzyme indicated for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome) (1).

Life-threatening anaphylactic reactions have occurred in some patients during Vimizim infusions. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to infusion reactions, and require additional monitoring. Appropriate medical support should be readily available when Vimizim is administered. Closely observe patients during and after Vimizim administration and be prepared to manage anaphylaxis. Inform patients of the signs and symptoms of anaphylaxis and have them seek immediate medical care should symptoms occur (1).

Sleep apnea is common in MPS IVA patients. Evaluation of airway patency should be considered prior to initiation of treatment with Vimizim. Patients using supplemental oxygen or continuous positive airway pressure (CPAP) during sleep should have these treatments readily available during infusion in the event of an acute reaction, or extreme drowsiness/sleep induced by antihistamine use (1).

Safety and effectiveness in patients below 5 years of age have not been established (1).

Summary

Vimizim is an enzyme preparation for patients diagnosed with Mucopolysaccharidosis type IVA (MPS IVA). Vimizim is intended to replace the missing GALNS enzyme involved in an important

metabolic pathway. Anaphylaxis has been reported to occur during Vimizim infusions, regardless of duration of the course of treatment. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to infusion reactions, and require additional monitoring. Appropriate medical support should be readily available when Vimizim is administered. Safety and efficacy have not been established in pediatric patients less than five years of age (1).

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

References

1. Vimizim [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; December 2019.