Mucopolysaccharidoses comprise a group of lysosomal storage disorders caused by the deficiency of specific lysosomal enzymes required for the catabolism of glycosaminoglycans (GAG). Mucopolysaccharidosis IVA (MPS IVA, Morquio A Syndrome) is characterized by the absence or marked reduction in N-acetylglactosamine-6-sulfatase activity. The sulfatase activity deficiency results in the accumulation of the GAG substrates, KS and C6S, in the lysosomal compartment of cells throughout the body. The accumulation leads to widespread cellular, tissue, and organ dysfunction. Vimizim is intended to provide the exogenous enzyme N-acetylgalactosamine-6-sulfatase that will be taken up into the lysosomes and increase the catabolism of the GAGs KS and C6S. Elosulfase alfa uptake by cells into lysosomes is mediated by the binding of mannose-6-phosphate-terminated oligosaccharide chains of elosulfase alfa to mannose-6-phosphate receptors.

(vimizim™) is indicated for patients with Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome).

Elosulfase alfa hypersensitivity can cause anaphylaxis. Patients with acute febrile or respiratory illness at the time of Vimizim infusion may be at higher risk of life-threatening complications from hypersensitivity reactions. Careful consideration should be given to the patient's clinical status prior to administration of Vimizim and consider delaying the Vimizim infusion.

Cost ☒ Potential for misuse ☒ Toxicity

1. Patient is 5 years of Age or older
2. Patient has a diagnosis of Mucopolysaccharidosis type IVA (MPS IVA; Morquio A syndrome)
3. Dosing is 2 mg per kg given IV once every week.

1. The patient does not meet the above criteria

Initial approval will be for 3 months; renewals will be issued for 12 months as well based on patient’s clinical response.