BlueCross BlueShield Federal Employee Program

ELAPRASE
(idursulfase)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Hunter Syndrome (mucopolysaccharidosis type II or MPS II) is an X-linked recessive disease caused by the body's inability to break down certain elements in the body called mucopolysaccharides, also known as glycosaminoglycans (GAG) due to insufficient levels of the lysosomal enzyme iduronate-2-sulfatase (I2S). The missing or defective enzyme causes mucopolysaccharides to accumulate in a variety of cells, leading to cellular growth, organ enlargement, tissue destruction, and organ system dysfunction. Elaprase (idursulfase) replaces the deficient or absent enzyme to breakdown the excess buildup within the cells, returning the cells to normal size (1).

Regulatory Status

FDA-approved indication: Elaprase is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II) is indicated for patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II). Elaprase has been shown to improve walking capacity in patients 5 years or older. In patients 16 months to 5 years of age, no data are available to demonstrate improvement in disease-related symptoms or long term clinical outcome; however, treatment with Elaprase has reduced spleen volume similarly to that of adults and children 5 years of age and older (1).

Elaprase carries a boxed warning regarding the risk of life-threatening anaphylaxis reactions during 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 Elaprase is administered (1).

Safety and efficacy have not been established in pediatric patients less than 16 months of age of age (1).

Summary

Elaprase (idursulfase) is indicated for patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II). Elaprase carries a boxed warning regarding the risk of life-threatening anaphylaxis reactions during 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 Elaprase is administered. Safety and efficacy have not been established in pediatric patients less than 16 months of age (1).

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

References

1. Elaprase [package insert].Lexington, MA. Shire Human Genetic Therapies, Inc. September 2021.