RATIONAL FOR INCLUSION IN PA PROGRAM

Background
Aldurazyme is used to treat Mucopolysaccharidosis I (MPS I) a very rare disease that gets worse over time and can be life-threatening. It is an inherited disorder caused by a deficiency of an enzyme called alpha-L-iduronidase. This enzyme is needed for the breakdown of certain substances in the body commonly referred to as GAG (glycosaminoglycans). As more and more GAG builds up in a person’s body organs can become permanently damaged. That is why early diagnosis and treatment of MPS I is very important. MPS I has also been called Hurler, Hurler-Scheie, and Scheie syndromes (1).

Regulatory Status
FDA-approved indication: Aldurazyme is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for patients with Hurler and Hurler-Scheie forms of Mucopolysaccharidosis I (MPS I) and for patients with the Scheie form who have moderate to severe symptoms. The risks and benefits of treating mildly affected patients with the Scheie form have not been established (1).

Aldurazyme has been shown to improve pulmonary function and walking capacity. Aldurazyme has not been evaluated for effects on the central nervous system manifestations of the disorder (1).

The Aldurazyme label includes a boxed warning citing the risk of anaphylaxis. Anaphylaxis and severe allergic reactions have been observed in patients during or up to 3 hours after infusions. Appropriate medical support and monitoring measures should be readily available when Aldurazyme is administered as these reactions may be life-threatening (1).

Patients with an acute febrile or respiratory illness at the time of Aldurazyme infusion may be at greater risk for infusion reactions. Careful consideration should be given to the patient’s clinical status prior to administration of Aldurazyme and consider delaying Aldurazyme infusion (1).

Administration of Aldurazyme should be exercised with caution when administering to patients susceptible to fluid overload, or patients with acute underlying respiratory illness or compromised
cardiac and/or respiratory function for whom fluid restriction is indicated. These patients may be at risk of serious exacerbation of their cardiac or respiratory status during infusions. Prior to administration of Aldurazyme pretreatment is recommended to reduce the risk of infusion reactions. Patients should receive antipyretics and/or antihistamines prior to infusion (1).

The safety and effectiveness of Aldurazyme was assessed in patients with MPS I, ages 6 months to 5 years old, and was found to be similar to the safety and effectiveness of Aldurazyme in pediatric patients 6 to 18 years, and adults (1).

Summary

Aldurazyme (laronidase) is indicated for patients 6 months of age or older with Hurler and Hurler-Scheie forms of Mucopolysaccharidosis I (MPS I) and for patients with the Scheie form who have moderate to severe symptoms. Aldurazyme carries a boxed warning of the risk of anaphylaxis during infusion. Patients with an acute febrile or respiratory illness at the time of Aldurazyme infusion may cause greater risk for infusion reactions. Patients susceptible to fluid overload may be at risk of acute cardiorespiratory failure. Medical support should be readily available when Aldurazyme is administered with additional monitoring for patients with compromised respiratory function or acute respiratory disease. Patients should receive a pretreatment of antipyretics and/or antihistamines prior to infusion to reduce the risk of infusion reactions (1).

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

References