



RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Aldurazyme (laronidase) is used to treat Mucopolysaccharidosis I (MPS I) 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. MPS I has also been called Hurler, Hurler-Scheie, and Scheie syndromes (1).

Regulatory Status

FDA-approved indications: (1)

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.

Limitations of use: (1)

- The risks and benefits of treating mildly affected patients with the Scheie form have not been established.
- Aldurazyme has not been evaluated for effects on the central nervous system manifestations of the disorder

Aldurazyme has been shown to improve pulmonary function and walking capacity (1).

Aldurazyme includes a boxed warning citing the risk of hypersensitivity reactions including anaphylaxis, and acute respiratory complications associated with administration. Patients treated with Aldurazyme have experienced life-threatening hypersensitivity reactions, including anaphylaxis. Appropriate medical monitoring and support measures, including cardiopulmonary resuscitation equipment, should be readily available during Aldurazyme administration. 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 (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



ALDURAZYME (laronidase)

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

1. Aldurazyme [package insert]. Cambridge, MA: Genzyme Corporation; December 2023.