

When requesting Aldurazyme® (laronidase), the individual requiring treatment must be diagnosed with the following FDA-approved indications and meet the specific coverage guidelines and applicable safety criteria for the covered indications.

## FDA-approved indications<sup>1</sup>

- Aldurazyme® (laronidase) is indicated for the treatment of individuals with the Hurler or Hurler-Scheie forms of mucopolysaccharidosis, type I.
- Aldurazyme® (laronidase) is indicated for the treatment of individuals with the Scheie form of mucopolysaccharidosis, type I and have moderate to severe symptoms.

## Approved Off-label Compendial use<sup>2</sup>

- No off-label uses meet evidence standards

## Coverage Guidelines

### Mucopolysaccharidosis, Type I (MPS I)

- Has a diagnosis of MPS I confirmed by enzyme assay demonstrating a deficiency of alpha-L-iduronidase enzyme activity or by genetic testing<sup>1,3</sup> AND
- Has Hurler or Hurler-Scheie form of MPS I <sup>1,3</sup> OR
- Has Scheie form of MPS I with moderate to severe symptoms<sup>1,3</sup>

**Approval duration: 12 months**

## Additional Information for Prescribers<sup>1</sup>

A registry for pregnant women is available. Pregnant women with MPS I should be encouraged to enroll in the MPS I Registry.

Our prescribers are alerted to the following safety concerns as educational points/reminders:

- Life-threatening anaphylactic reactions have been observed in some individuals during Aldurazyme® (laronidase) infusion and up to 3 hours after infusion.
- If anaphylactic or other severe allergic reactions occur, immediately discontinue the infusion and initiate appropriate treatment, which may include ventilator support, treatment with inhaled beta-adrenergic agonists, epinephrine, and IV corticosteroids.
- Individuals with acute febrile or respiratory illness at the time of Aldurazyme® (laronidase) infusion may be at greater risk for infusion reactions. Consider delaying Aldurazyme® (laronidase) infusion.
- Pretreatment is recommended prior to the infusion to reduce the risk of infusion reactions and may include antihistamines, antipyretics, or both. If infusion reactions occur, decreasing the infusion rate, temporarily stopping the infusion, or

- administering additional antipyretics and/or antihistamines may ameliorate the symptoms.

## References

1. Aldurazyme® prescribing information. Novato, CA: BioMarin Pharmaceutical Inc.: Revised April 2013. <https://aldurazyme.com/patients.aspx>. Accessed February 15, 2017.
2. Klasco RK (Ed): DRUGDEX® system. Thomson Micromedex, Greenwood Village, CO. Accessed February 15, 2017. <https://www.micromedexsolutions.com>
3. Lehman T, Miller N, Norquist B, et al. Diagnosis of the mucopolysaccharidoses. *Rheumatology*. 2011;50:v41-v48.