

When requesting Aldurazyme® (Iaronidase), 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

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

Coverage Guidelines

Mucopolysaccharidosis Type I (MPS I)

The individual must meet **all** of the following criteria for approval:

- Diagnosis is confirmed by one of the following:
 - Laboratory test demonstrating deficient α -L-iduronidase activity in leukocytes, fibroblasts, plasma, or serum; OR
 - Molecular genetic test demonstrating α -L-iduronidase gene mutation
- Aldurazyme is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Approval duration: 12 months

Dosing Recommendation

Approve up to 0.58 mg/kg administered intravenously no more frequently than once weekly.

References

1. Aldurazyme® solution for intravenous infusion [prescribing information]. Novato, CA: BioMarin Pharmaceutical Inc.; December 2019.
2. Muenzer J, Wraith JE, Clarke LA, et al. Mucopolysaccharidosis I: Management and treatment guidelines. *Pediatrics*. 2009;123:19-29.
3. Clarke LA, Atherton AM, Burton BK, et al. Mucopolysaccharidosis type I newborn screening: Best practices for diagnosis and management. *J Pediatr*. 2017;182:363-370.
4. Giugliani R, Federhen A, Munoz Rojas MV, et al. Mucopolysaccharidosis I, II, and VI: Brief review and guidelines for treatment. *Genet Mol Biol*. 2010;33:589-604.
5. Martins AM, Dualibi AP, Norato D, et al. Guidelines for the management of mucopolysaccharidosis type I. *J Pediatr*. 2009;155(Suppl 2):S32-S46.