

PHARMACY POLICY STATEMENT

Mississippi Medicaid

DRUG NAME	Aldurazyme (laronidase)
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Aldurazyme is an enzyme replacement therapy (ERT) that was approved by the FDA in 2003 for the treatment of Mucopolysaccharidosis type I (MPS I), including patients with Hurler syndrome, Hurler-Scheie syndrome, and Scheie syndrome with moderate to severe symptoms. The risks and benefits of treating mildly affected patients with Scheie syndrome have not been established. It has not been evaluated for effects on central nervous system manifestations.

MPS I is a rare genetic lysosomal storage disease, with Hurler syndrome being the most severe and most common subtype and Scheie syndrome as the rarest and mildest of the attenuated forms. Pathogenic mutations of the IDUA gene cause the enzyme alpha-L-iduronidase (IDUA) to be deficient or absent. Normally this lysosomal enzyme breaks down glycosaminoglycans (GAGs) (previously known as mucopolysaccharides) but when it is reduced in MPS I, the GAG substrates heparan sulfate (HS) and dermatan sulfate (DS) accumulate throughout the body leading to widespread cellular, tissue, and organ dysfunction. Aldurazyme provides an exogenous form of the deficient enzyme.

Aldurazyme (laronidase) will be considered for coverage when the following criteria are met:

Mucopolysaccharidosis I (MPS I)

For **initial** authorization:

1. Medication must be prescribed by or in consultation with a geneticist, metabolic specialist, or pediatrician experienced with managing mucopolysaccharidoses; AND
2. Member has a documented diagnosis of ONE of the following forms of MPS I:
 - a) Hurler syndrome (severe),
 - b) Hurler-Scheie syndrome (attenuated), or
 - c) Scheie syndrome with moderate to severe symptoms (attenuated); AND
3. Member's clinical diagnosis of MPS I has been confirmed by at least one of the following:
 - a) Low IDUA enzyme activity (less than 10%), and/or
 - b) Molecular genetic testing identifies pathogenic IDUA gene mutations; AND
4. Documentation of elevated baseline urinary GAG (uGAG) level.
5. **Dosage allowed/Quantity limit:** 0.58 mg/kg IV infusion once weekly

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show improvement or stabilized signs and symptoms of disease such as improved functional capacity (e.g. 6-minute walk test, forced vital capacity (FVC)) compared to baseline, reduced hepatomegaly, and/or reduced uGAG levels.

If all the above requirements are met, the medication will be approved for an additional 12 months.



TrueCare considers Aldurazyme (laronidase) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
07/26/2021	New policy for Aldurazyme created.
01/02/2024	Removed age limit. Added the terms “severe” and “attenuated.”

References:

1. Aldurazyme [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; 2023.
2. Wraith JE, Clarke LA, Beck M, et al. Enzyme replacement therapy for mucopolysaccharidosis I: a randomized, double-blinded, placebo-controlled, multinational study of recombinant human alpha-L-iduronidase (laronidase). *J Pediatr*. 2004;144(5):581-588. doi:10.1016/j.jpeds.2004.01.046
3. Wang RY, Bodamer OA, Watson MS, Wilcox WR; ACMG Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genet Med*. 2011;13(5):457-484. doi:10.1097/GIM.0b013e318211a7e1
4. Jameson E, Jones S, Remington T. Enzyme replacement therapy with laronidase (Aldurazyme®) for treating mucopolysaccharidosis type I. *Cochrane Database Syst Rev*. 2019;6(6):CD009354. Published 2019 Jun 18. doi:10.1002/14651858.CD009354.pub5
5. Clarke LA. Mucopolysaccharidosis Type I. 2002 Oct 31 [Updated 2021 Feb 25]. In: Adam MP, Ardinger HH, Pagon RA, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2021. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK1162/>
6. Martins AM, Dualibi AP, Norato D, et al. Guidelines for the management of mucopolysaccharidosis type I. *J Pediatr*. 2009;155(4 Suppl):S32-S46. doi:10.1016/j.jpeds.2009.07.005
7. de Ru MH, Boelens JJ, Das AM, et al. Enzyme replacement therapy and/or hematopoietic stem cell transplantation at diagnosis in patients with mucopolysaccharidosis type I: results of a European consensus procedure. *Orphanet J Rare Dis*. 2011;6:55. Published 2011 Aug 10. doi:10.1186/1750-1172-6-55
8. Kubaski F, de Oliveira Poswar F, Michelin-Tirelli K, et al. Mucopolysaccharidosis Type I. *Diagnostics (Basel)*. 2020;10(3):161. Published 2020 Mar 16. doi:10.3390/diagnostics10030161

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