CVS Caremark®

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| Reference number(s) |
| 2049-A |

# Specialty Guideline Management Aldurazyme

## Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name |
| --- | --- |
| Aldurazyme | laronidase |

## Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

### FDA-Approved Indications1

Aldurazyme is indicated for the treatment of adult and pediatric 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

* The safety and effectiveness of treating mildly affected patients with the Scheie form have not been established.
* The effect of Aldurazyme on central nervous system manifestations of the disorder has not been determined.

All other indications are considered experimental/investigational and not medically necessary.

## Documentation

Submission of the following information is necessary to initiate the prior authorization review:

* Initial requests: alpha-L-iduronidase enzyme assay or genetic testing results supporting diagnosis.
* Continuation requests: chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

## Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

## Coverage Criteria

### Mucopolysaccharidosis I (MPS I)1-4

Authorization of 12 months may be granted for treatment of MPS I when both of the following criteria are met:

* Diagnosis of MPS I was confirmed by enzyme assay demonstrating a deficiency of alpha-L-iduronidase enzyme activity or by genetic testing.
* Member has one of the following:
  + The Hurler form (i.e., severe MPS I).
  + The Hurler-Scheie form (i.e., attenuated MPS I).
  + The Scheie form (Scheie syndrome; i.e., attenuated MPS I) with moderate to severe symptoms (e.g., normal intelligence, less progressive physical problems, corneal clouding, joint stiffness, valvular heart disease).

## Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the Coverage Criteria section who have a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

## References

1. Aldurazyme [package insert]. Cambridge, MA: Genzyme Corporation; December 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;144:581-588.
3. Muenzer J, Wraith JE, Clarke LA; International Consensus Panel on Management and Treatment of Mucopolysaccharidosis I. Mucopolysaccharidosis I: management and treatment guidelines. Pediatrics. 2009 Jan;123(1):19-29.
4. Clarke LA. Mucopolysaccharidosis Type I. 2002 Oct 31 [Updated 2024 Apr 11]. In: Adam MP, Everman DB, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2023. Accessed Jan 10, 2025.