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POLICY Document for ALDURAZYME (laronidase)

The overall objective of this policy is to support the appropriate and cost-effective use of the medication, specific to use of preferred medication options, lower cost site of care and overall, clinically appropriate use. This document provides specific information to each of the three sections of the overall policy.

Section 1: Site of Care

- Policy information specific to site of care (outpatient, hospital outpatient, home infusion)

Section 2: Clinical Criteria

- Policy information specific to the clinical appropriateness for the medication

Section 1: Site of Care

Site of Care Criteria
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.

Brand Name	Generic Name	Dosage Form
Aldurazyme	laronidase	intravenous

Criteria for Approval for Administration in Outpatient Hospital Setting

This policy provides coverage for administration of Aldurazyme in an outpatient hospital setting for up to 54 days when a member is new to therapy or is reinitiating therapy after not being on therapy for at least 6 months.

This policy provides coverage for administration of Aldurazyme in an outpatient hospital setting for a longer course of treatment when ANY of the following criteria are met:

- The member has experienced an adverse reaction to the drug that did not respond to conventional interventions (e.g., acetaminophen, steroids, diphenhydramine, fluids, other

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pre-medications or slowing of infusion rate) or a severe adverse event (anaphylaxis, anaphylactoid reactions, myocardial infarction, thromboembolism, or seizures) during or immediately after infusion (up to 3 hours post infusion).

- The member has developed laboratory confirmed laronidase IgE antibodies which increases the risk for infusion related reactions.
- The member is medically unstable (e.g., respiratory, cardiovascular, or renal conditions).
- The member has severe venous access issues that require the use of special interventions only available in the outpatient setting.
- The member has significant behavioral issues and/or physical or cognitive impairment that would impact the safety of the infusion therapy AND the patient does not have access to a caregiver.
- Alternative infusion sites (pharmacy, physician office, ambulatory care, etc.) are greater than 30 miles from the member's home.
- The member is less than 14 years of age.

For situations where administration of Aldurazyme does not meet the criteria for outpatient hospital infusion, coverage for Aldurazyme is provided when administered in alternative sites such as; physician office, home infusion or ambulatory care.

Required Documentation

The following information is necessary to initiate the site of care prior authorization review (where applicable):

- Medical records supporting the member has experienced an adverse reaction that did not respond to conventional interventions or a severe adverse event during or immediately after an infusion
- Medical records supporting the member has developed laronidase IgE antibodies
- Medical records supporting the member is medically unstable
- Medical records supporting the member has severe venous access issues that requires specialized interventions only available in the outpatient hospital setting
- Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver

Records supporting alternative infusion sites are greater than 30 miles from the member's home
Medical records supporting the member is new to therapy

Section 2: Clinical Criteria

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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 Indications[1]

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.

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- 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

SECTION 1

1. Aldurazyme [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; December 2023.
2. Giugliani R, Rojas VM, Martins AM, et al. A dose-optimization trial of laronidase (Aldurazyme) in patients with mucopolysaccharidosis I. *Mol Genet Metab*. 2009;96(1):13-19.
3. Clarke LA, Wraith JE, Beck M, et al. Long-term efficacy and safety of laronidase in the treatment of mucopolysaccharidosis I. *Pediatrics.* 2009;123(1):229-240.

SECTION 2

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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.

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