Medical Drug Clinical Criteria

Subject:	Aldurazyme (laronidase)		
Document #:	CC-0025	**Publish Date**:	10/23/2024
Status:	Reviewed	**Last Review Date**:	09/09/2024

Table of Contents

Overview

This document addresses Aldurazyme (laronidase), an enzyme replacement therapy approved by the Food and Drug Administration (FDA) to treat individuals with Mucopolysaccharidosis I. Mucopolysaccharidosis I has historically been divided into three groups based on severity of symptoms: Hurler syndrome, Hurler-Scheie syndrome and Scheie syndrome. Aldurazyme is approved to treat all three syndromes with the exception of individuals exhibiting only mild symptoms of Scheie syndrome.

The mucopolysaccharidoses are a group of inherited metabolic diseases caused by the deficiency of lysosomal enzymes needed to breakdown mucopolysaccharides or glycosaminoglycan (GAGs). The progressive accumulation of GAGs in lysosomes leads to respiratory, cardiac, skeletal and connectivity, neurologic and ophthalmologic complications. There are seven distinct types of mucopolysaccharidosis (I, II, III, IV, VI, VII and IX). Accurate diagnosis is important to provide disease-specific enzyme replacement therapy. Diagnosis is confirmed through urinary GAG concentration measurement, enzymatic activity measurement or genetic testing.

Aldurazyme has a black box warning for hypersensitivity reactions. Life-threatening anaphylaxis has occurred during Aldurazyme infusions. Appropriate medical support should be available during Aldurazyme administration. Individuals with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory disease and require additional monitoring.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Aldurazyme (laronidase)

Initial requests for Aldurazyme (laronidase) may be approved if the following criteria are met:

I. Individual has a diagnosis of any of the following Mucopolysaccharidosis I (MPS I) syndromes:
   A. Hurler syndrome; **OR**
   B. Hurler-Scheie syndrome; **OR**
   C. Documentation is provided for Scheie syndrome, moderate to severe symptoms including any of the following (Thomas 2010, Wang 2011):
      1. Cardiac valve abnormalities (including aortic or mitral valve regurgitation, with or without insufficiency or stenosis); **OR**
      2. Corneal clouding, open-angle glaucoma, and retinal degeneration, progressive; **OR**
      3. Craniofacial or growth retardation; **OR**
      4. Frequent, moderate to severe upper respiratory infections; **OR**
      5. Hepatosplenomegaly; **OR**
      6. Hernias (such as hiatal, inguinal, or umbilical); **OR**
      7. Neurological symptoms resulting from cervical instability or cervical spinal cord compression; **OR**
      8. Skeletal and joint involvement, progressive (including arthropathy, back pain, joint stiffness, lumbar spondylolisthesis, lumbar spinal compression, osteopenia, or osteoporosis); **AND**

II. Documentation is provided that diagnosis is demonstrated by one of the following (Clarke 2021, Lehman 2011):
   A. Documented deficiency in alpha-L-iduronidase enzyme activity as measured in fibroblasts or leukocytes; **OR**
   B. Documented alpha-L-iduronidase gene mutation.

Continuation requests for Aldurazyme (laronidase) may be approved if the following criterion is met:

I. Documentation is provided that there is clinically significant improvement or stabilization in clinical signs and symptoms of disease (including but not limited to reduction in urinary GAG excretion, reduction in hepatosplenomegaly, improvement in pulmonary function, improvement in walking distance and/or improvement in fine or gross motor function) compared to the predicted natural history trajectory of disease.

Requests for Aldurazyme (laronidase) may not be approved for the following:

I. Individual with the Scheie syndrome of MPS I with mild symptoms; **OR**
II. May not be approved when the above criteria are not met and for all other indications.

Quantity Limits

Aldurazyme (laronidase) Quantity Limit

Drug	Limit
Aldurazyme (laronidase) 2.9 mg vial	0.58 mg/kg once a week

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J1931	Injection, laronidase, 0.1 mg (Aldurazyme)
S9357	Home infusion therapy, enzyme replacement intravenous therapy, (e.g., Imiglucerase); administrative services, professional pharmacy services, care coordination, and all necessary supplies and equipment (drugs and nursing visits coded separately), per diem

ICD-10 Diagnosis

E76.01	Hurler's syndrome
E76.02	Hurler-Scheie syndrome
E76.03	Scheie's syndrome
E76.3	Mucopolysaccharidosis, unspecified

Document History

Reviewed: 9/9/2024
Document History:

- 9/9/2024 – Annual Review: No changes. Coding Reviewed: No changes.
- 9/11/2023 – Annual Review: Wording and formatting changes. Update references. Coding Reviewed: No changes.
- 9/12/2022 – Annual Review: Wording and formatting changes. Coding Reviewed: No changes.
- 9/13/2021 – Annual Review: Wording and formatting changes. Coding reviewed: No changes.
- 08/01/2021 – Administrative update to add documentation.
- 09/14/2020 – Annual Review: Addition of continuation criteria. Coding Reviewed: No changes.
- 09/23/2019 – Administrative update to add drug specific quantity limit.
- 09/09/2019 – Annual Review: Wording and formatting changes. Coding reviewed: Added ICD-10-DX –E76.3
- 08/17/2018 – Annual Review: Update enzyme activity diagnosis criteria for consistency with other MPS agents. Add references for non-label based criteria elements. Minor wording and formatting updates.

References

1. Clarke LA. Mucopolysaccharidosis I. 2002 Oct 31 [Updated 2021 Feb 25]. In: Pagon RA, Adam MP, Bird TD, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2016. Available at: https://www.ncbi.nlm.nih.gov/books/NBK1162/. Accessed: September 7, 2024.
2. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: September 7, 2024.
3. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
4. Lehman TJ, Miller N, Norquist B, Underhill L, Keutzer J. Diagnosis of the mucopolysaccharidoses. *Rheumatology (Oxford)*. 2011;50 Suppl 5:v41-v48. doi:10.1093/rheumatology/ker390.
5. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. Updated periodically.
6. Muenzer J, Wraith HE, Clark LA, International Consensus Panel on Management and Treatment of Mucopolysaccharidosis I. Mucopolysaccharidosis I: management and treatment guidelines. *Pediatrics.* 2009 Jan;123(1):19-29.
7. Thomas JA, Beck M, Clarke JTR, Cox GF. Childhood onset of Scheie syndrome, the attenuated form of mucopolysaccharidosis I. *Journal of Inherited Metabolic Disease*. 2010; 33(4):421-427.
8. Wang RY, Bodamer OA, Watson MS, Wilcox WR. American College of Medical Genetics (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.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.