

**Aldurazyme® (laronidase)
(Intravenous)**

Effective Date: 02/01/2020**Review Date:** 01/15/2020, 1/28/2021, 1/5/2022, 1/5/2023, 12/07/2023, 01/10/2024, 5/21/2025**Scope:** Medicaid, Commercial, Medicare**I. Length of Authorization**

Coverage will be provided for 12 months and may be renewed.

II. Dosing Limits**A. Max Units (per dose and over time) [HCPCS Unit]:**

- 2668 billable units every 28 days

III. Summary of Evidence

Aldurazyme (laronidase) is an enzyme replacement therapy indicated for the treatment of patients with Hurler-Scheie, and moderate to severe Scheie forms of Mucopolysaccharidosis I (MPSI), a progressive, multisystem lysosomal storage disorder caused by α -L-iduronidase deficiency. Its approval is based on a 26-week randomized, double-blind, placebo-controlled trial in 45 patients aged 6 to 43 weeks with MPS I. Patients receiving Aldurazyme (0.58 mg/kg IV weekly) demonstrated significant improvement in forced vital capacity (+1% vs. -3% with placebo; $p=0.02$) and a 38-meter improvement in 6-minute walk test versus placebo ($p=0.07$). All patients completing the trial continued into a 182-week open-label extension study, which demonstrated sustained improvements in functional outcomes and continued urinary GAG reduction. The most common adverse reactions are infusion related events, including rash, pyrexia, headache, and flushing. Life-threatening hypersensitivity reactions including anaphylaxis have been reported.

IV. Initial Approval Criteria^{1,2,3,4,5,6}

Coverage is provided in the following conditions:

Medicare members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements.

- Patient is 6 months of age or older; **AND**

- Therapy is being used to treat non-central nervous system manifestations of the disease and patient does not have severe, irreversible cognitive impairment; ; **AND**
- Documented baseline value for urinary glycosaminoglycan (uGAG) has been obtained; **AND**
- Documented baseline age-appropriate values for one or more of the following have been obtained:
 - Patients 6 years or greater: percent predicted forced vital capacity (FVC), 6-minute walk test, joint range of motion, left ventricular hypertrophy, growth, quality of life (CHAQ/HAQ/MPS HAQ); **OR**
 - Patients 6 months to less than 6 years: cardiac status, upper airway obstruction during sleep, growth velocity, mental development, FVC, and/or 6-minute walk test; **AND**

Mucopolysaccharidosis I (MPS I) †

- Patient has a definitive diagnosis of MPS I confirmed by one of the following:
 - Detection of biallelic pathogenic mutations in the *IDUA* gene by molecular genetic testing; **OR**
 - Detection of deficient activity of the lysosomal enzyme α -L-iduronidase (IDUA); **AND**
- Patient has one of the following diagnoses:
 - Hurler (severe) or Hurler-Scheie (attenuated) forms of disease; **OR**
 - Scheie (attenuated) form of disease with moderate to severe symptoms

† FDA approved indication(s) **Φ** Orphan Drug

V. Renewal Criteria^{1,2,5,6}

Authorizations can be renewed based on the following criteria:

- Patient continues to meet indication-specific relevant criteria identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: anaphylaxis and severe hypersensitivity reactions, acute respiratory complications, acute cardiorespiratory failure, severe infusion reactions, etc.; **AND**
- Patient has a documented reduction in uGAG levels compared to pretreatment baseline; **AND**

- Patient has demonstrated a beneficial response to therapy compared to pretreatment baseline in one or more of the following:
 - Patients 6 years or greater: stability or improvement in percent predicted FVC and/or 6-minute walk test, increased joint range of motion, decreased left ventricular hypertrophy, improved growth, improved quality of life (clinically meaningful change in the CHAQ/HAQ/MPS HAQ disability index); **OR**
 - Patients 6 months to less than 6 years: stability or improvement in cardiac status, upper airway obstruction during sleep, growth velocity, mental development, FVC and/or 6-minute walk test

VI. Dosage/Administration^{1,2,5,6}

Indication	Dose
Mucopolysaccharidosis I (MPS I)	0.58 mg/kg of body weight administered once weekly as an intravenous infusion

VII. Billing Code/Availability Information

HCPCS Code:

J1931 – Injection, laronidase, 0.1 mg; 1 billable unit = 0.1 mg

NDC:

Aldurazyme 2.9 mg/5 mL single-dose vial: 58468-0070-xx

VIII. References

1. Aldurazyme [package insert]. Cambridge, MA; Genzyme Corporation.; October 2024. Accessed May 2025.
2. Clark LA. Mucopolysaccharidosis Type I. GeneReviews®. www.ncbi.nlm.nih.gov/books/NBK1162/. Initial Posting: October 31, 2002; Last Update: February 11, 2016. Accessed on August 24, 2018.
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. doi: 10.1542/peds.2008-0416.
4. Martins AM, Dualibi AP, Norato D, et al. 1.Guidelines for the Management of Mucopolysaccharidosis Type I. JPeds. 155 (4): S32 - S46.

5. Clarke LA, Wraith JE, Beck M, et al. Long-term efficacy and safety of laronidase in the treatment of mucopolysaccharidosis I. *Pediatrics*. 2009 Jan;123(1):229-40. doi: 10.1542/peds.2007-3847.
6. Wraith JE, Beck M, Lane R, et al. Enzyme replacement therapy in patients who have mucopolysaccharidosis I and are younger than 5 years: results of a multinational study of recombinant human alpha-L-iduronidase (laronidase). *Pediatrics*. 2007 Jul;120(1):e37-46. Epub 2007 Jun 4.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
E76.01	Hurler's syndrome
E76.02	Hurler-Scheie syndrome
E76.03	Scheie's syndrome

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Determinations (LCDs), and Articles may exist and compliance with these policies is required where applicable. They can be found at: <http://www.cms.gov/medicare-coverage-database/search/advanced-search.aspx>. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCD/Article): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	Applicable State/US Territory	Contractor
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT,	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	Applicable State/US Territory	Contractor
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC

Policy Rationale:

Aldurazyme was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Aldurazyme according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For Medicare members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.