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

Scope: Medicaid, Commercial, Medicare-Medicaid Plan (MMP)

I. Length of Authorization

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

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC unit]:

- Aldurazyme 2.9 mg/5ml vial: 92 vials every 28 days

B. Max Units (per dose and over time) [HCPCS Unit]:

- 667 billable units every 7 days

III. Summary of Evidence

Clinical trials have demonstrated the efficacy and safety of Aldurazyme in patients with MPS I. Treatment with Aldurazyme has been shown to improve endurance, walking capacity, and pulmonary function, as well as reduce urinary glycosaminoglycan (GAG) levels and liver volume. Long-term studies have also indicated a stabilization or slowing of disease progression with continued treatment.

IV. Initial Approval Criteria[1,2,3,4,5,6]

Coverage is provided in the following conditions:

MMP 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**
- Patient has absence of severe 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.; May 2023. Accessed October 2023.
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 INTEGRITY (Medicare-Medicaid Plan) 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.