Clinical Policy: Laronidase (Aldurazyme)

Reference Number: PA.CP.PHAR.152
Effective Date: 01/2018
Last Review Date: 04/2023

Revision Log
Coding Implications

Description
Laronidase (Aldurazyme®) is a hydrolytic lysosomal glycosaminoglycan-specific enzyme.

FDA Approved Indication(s)
Aldurazyme is indicated for 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.

Limitation(s) of use:
- The risks and benefits of treating mildly affected patients with the Scheie form have not been established.
- Aldurazyme has not been evaluated for effects on the central nervous system manifestations of the disorder

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness Corporation® that Aldurazyme is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. MPS I (mucopolysaccharidosis I) : Hurler, Hurler-Scheie and Scheie Forms (must meet all):

1. Diagnosis of MPS I: confirmed by one of the following:
   a. Enzyme assay demonstrating deficiency of alpha-L-iduronidase activity;
   b. DNA testing;
2. Member has one of the following (a or b):
   a. Hurler or Hurler-Scheie form of MPS I;
   b. Scheie form of MPS I with moderate to severe symptoms;
3. Age ≥ 6 months;
4. Documentation of member's current weight (in kg);
5. Dose does not exceed 0.58 mg/kg/week (rounded up to the nearest whole vial).

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued approval

A. MPS I: Hurler, Hurler-Scheie and Scheie Forms (must meet all):

1. Currently receiving medication via PA Health & Wellness benefit or member has previously met approval criteria or the Continuity of Care policy (PA.LTSS.PHAR.01) applies.;

2. Member is responding positively to therapy;
3. Documentation of member's current weight (in kg);
4. If request is for a dose increase, new dose does not exceed 0.58 mg/kg/week.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via PA Health & Wellness benefit or member has previously met initial approval criteria or the Continuity of Care policy (PA.LTSS.PHAR.01) applies.
2. Refer to PA.CP.PMN.53

III. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

6MWT: 6-minute walk test
FDA: Food and Drug Administration
FVC: forced vital capacity
MPS: mucopolysaccharidosis

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): none reported.
- Boxed warning(s): risk of life-threatening anaphylactic reactions with Aldurazyme infusions.

Appendix D: General Information

- The presenting symptoms and clinical course of MPS I can vary from one individual to another. Some examples, however, of improvement in MPS I disease as a result of Aldurazyme therapy may include improvement in:
  - Percent predicted forced vital capacity (FVC);
  - 6-minute walk test (6MWT);
  - Joint stiffness, Carpal Tunnel Syndrome;
  - Upper airway infection recurrence;
  - Hepatomegaly, splenomegaly;
  - Growth deficiencies.
- In the clinical trials of Aldurazyme in patients ≥ 6 years of age, the mean increase in percent of predicted forced vital capacity (FVC) observed corresponded to a 10% relative improvement over the baseline FVC, which is considered by the American Thoracic Society to be a clinically significant change and not due to week-to-week variability.
- In the clinical trials of Aldurazyme in patients ≥ 6 years of age, patients treated with Aldurazyme demonstrated a 19.7 meter mean increase in the 6MWT after 26 weeks.

IV. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
MPS I	0.58 mg/kg IV once weekly	0.58 mg/kg/week

V. Product Availability

Vial: 2.9 mg/5 mL

VI. References

1. Aldurazyme Prescribing Information. Cambridge, MA: Genzyme Corporation; December 2019. Available at https://www.aldurazyme.com. Accessed February 9, 2023.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.
3. Muenzer J, Wraith JE, Clarke LA. Mucopolysaccharidosis I: management and treatment guidelines. Pediatrics. 2009;123:19-29.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J1931	Injection, laronidase, 0.1 mg

Reviews, Revisions, and Approvals	Date	Approval Date
2Q 2018 annual review: no significant changes from previously approved policy; removed requirement for severity of MPS I Scheie form as this is a non-specific, non-actionable requirement; references reviewed and updated.	02/2018	
2Q 2019 annual review: added clarification on rounding the requested dose up to the nearest whole vial size to avoid inappropriate denials based on existing vial availability; references reviewed and updated.	04/2019	
2Q 2020 annual review: references reviewed and updated.	04/2020	
2Q 2021 annual review: clarified the covered subtypes of MPS I, to align with the FDA-approved indication; references reviewed and updated.	04/2021	
2Q 2022 annual review: added requirement for documentation of member's current weight for dose calculation purposes; references reviewed and updated.	04/2022	
2Q 2023 annual review: no significant changes; references reviewed and updated.	04/2023	