Clinical Policy: Laronidase (Aldurazyme)

Reference Number: PA.CP.PHAR.152
Effective Date: 01/18
Last Review Date: 04/19

Revision Log
Coding Implications

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

FDA Approved Indication(s)
Aldurazyme is indicated for the treatment of 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. Aldurazyme has been shown to improve pulmonary function and walking capacity.

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
It is the policy of health plans affiliated with Pennsylvania Health and 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. Age ≥ 6 months;
3. 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 Pennsylvania Health and 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. 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 Pennsylvania Health and 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

FDA: Food and Drug Administration
FVC: forced vital capacity
GAG: glycosaminoglycan
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;
- Joint stiffness, Carpal Tunnel Syndrome;
- Upper airway infection recurrence;
- Hepatomegaly, splenomegaly;
- Growth deficiencies.

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; April 2013. Available at https://www.aldurazyme.com. Accessed February 28, 2019.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.

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

3.

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.	02.05.18	
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.	04/19	