Clinical Policy Title:	**laronidase**
Policy Number:	RxA.10
Drug(s) Applied:	Aldurazyme®
Original Policy Date:	02/07/2020
Last Review Date:	03/09/2021
Line of Business Policy Applies to:	All lines of business

Background

Laronidase (Aldurazyme®) is a hydrolytic lysosomal glycosaminoglycan-specific enzyme. It is indicated for the treatment of 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.

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
laronidase (Aldurazyme®)	MPS I	0.58 mg/kg IV once weekly	0.58 mg/kg/week

Dosage Forms

- Vial: 2.9 mg/5 mL (0.58mg/mL)

Clinical Policy

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

I. Initial Approval Criteria

A. 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 per kg per week (rounded up to the nearest whole vial).

Approval Duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

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

1. Member is currently receiving medication that has been authorized by RxAdvance or member has previously met initial approval criteria listed in this policy;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS I disease manifestation profile (see Appendix D for examples);
3. If request is for a dose increase, new dose does not exceed 0.58 mg per kg per week (rounded up to the nearest whole vial).

Approval Duration
Commercial: 6 months
Medicaid: 12 months

III. Appendices

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.

- 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.

References

1. Aldurazyme Prescribing Information. Cambridge, MA: Genzyme Corporation; December 2019. Available at https://www.aldurazyme.com. Accessed January 20, 2021.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34. Accessed January 20, 2021.

Review/Revision History	Review/Revised Date	P&T Approval Date
Policy established.	01/2020	02/07/2020
Policy was reviewed: 1) Continuation therapy criteria II.A.1. rephrased to "Member is currently receiving medication that has been authorized by RxAdvance or the member has met initial approval criteria listed in this policy." 2) Initial therapy and continued therapy approval updated from duration of request or 6 months (whichever is less) to "6 months". 3) References were updated.	01/20/2021	03/09/2021