Prior Authorization Review Panel

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CHC-MCO Policy Submission

A separate copy of this form must accompany each policy submitted for review.
Policies submitted without this form will not be considered for review.

Plan: PA Health & Wellness	**Submission Date: 05/01/2020**
Policy Number: PA.CP.PHAR.152	**Effective Date: 01/2018** **Revision Date: 04/15/2020**
Policy Name: Laronidase (Aldurazyme)	

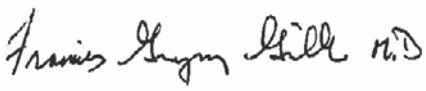
Type of Submission – Check all that apply:

- ☐ **New Policy**
- ✓ **Revised Policy***
- ☐ **Annual Review - No Revisions**
- ☐ **Statewide PDL -** *Select this box when submitting policies for Statewide PDL implementation and when submitting policies for drug classes included on the Statewide PDL.*

***All revisions to the policy must be highlighted using track changes throughout the document.**

Please provide any changes or clarifying information for the policy below:

2Q 2020 annual review: references reviewed and updated.

Name of Authorized Individual (Please type or print): **Francis G. Grillo, MD**	**Signature of Authorized Individual:** 

Clinical Policy: Laronidase (Aldurazyme)

Reference Number: PA.CP.PHAR.152
Effective Date: 01/18
Last Review Date: 04/2020

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 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
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.
- 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 21, 2020.

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

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	
2Q 2020 annual review: references reviewed and updated.	04/2020	