Clinical Policy: Laronidase (Aldurazyme)

Reference Number: ERX.SPA.102
Effective Date: 10.01.16
Last Review Date: 11.17 Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

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
Provider must submit documentation (which may include office chart notes and lab results) supporting that member has met all approval criteria.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ that Aldurazyme is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Mucopolysaccharidosis I: Hurler, Hurler-Scheie, and Scheie Forms (must meet all)**:**
1. Diagnosis of MPS I (Hurler, Hurler-Scheie, or Scheie form) confirmed by one of the following (a or b):
   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.

Approval duration: 6 months

B. Other diagnoses/indications
1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

A. Mucopolysaccharidosis I: Hurler, Hurler-Scheie, and Scheie Forms (must meet all)**:**
1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
2. Member is responding positively to therapy as evidenced by improvement in the individual member's MPS I disease manifestation profile *(see Appendix B for examples)*;
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 a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
   Approval duration: Duration of request or 6 months (whichever is less); or

2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off-label use policy – ERX.PA.01 or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

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

Appendix B: General Information

- 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 6-minute walk test (6MWT) after 26 weeks.
- 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 FVC;
  - 6MWT;
  - Joint stiffness, carpal tunnel syndrome;
  - Upper airway infection recurrence;
  - Hepatomegaly, splenomegaly;
  - Growth deficiencies.

Appendix C: Therapeutic Alternatives

N/A

V. Dosage and Administration

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

VI. Product Availability

Vial: 2.9 mg/5 mL

VII. References

1. Aldurazyme Prescribing Information. Cambridge, MA: Genzyme Corporation; April 2013. Available at https://www.aldurazyme.com. Accessed June 27, 2017.
2. Muenzer J. The mucopolysaccharidoses: a heterogeneous group of disorders with variable pediatric presentations. J Pediatr. 2004; 144(5 Suppl): S27-S34.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy split from USS.SPMN.33 Lysosomal Storage Disorders and converted to new template. Added age restriction per PI. Changed requirement for FVC ≤ 80% of predicted normal to attestation of moderate to severe symptoms for Scheie form of MPS I. Modified approval duration to 6 months for initial and 12 months for re-auth.	08.16	09.16

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Converted to new template. Added prescriber requirement. Added max dose criteria. Added requirement for positive response to therapy.	06.17	08.17
4Q17 Annual Review Removed prescriber requirement. Removed subjective requirement for moderate-to-severe symptoms for MPS I Scheie form.	09.11.17	11.17

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.

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