

Clinical Policy: Laronidase (Aldurazyme)

Reference Number: CP.PHAR.152

Effective Date: 02.01.16

Last Review Date: 05.20

Line of Business: Commercial, HIM, Medicaid

[Coding Implications](#)
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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 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 Centene Corporation[®] 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: confirmed by one of the following:
 - a. Enzyme assay demonstrating deficiency of alpha-L-iduronidase activity;
 - b. DNA testing;
2. Age \geq 6 months;
3. Dose does not exceed 0.58 mg per kg per week (rounded up to the nearest whole vial).

Approval duration:

Medicaid/HIM – 6 months

Commercial – 6 months or to the member's renewal date, whichever is longer

B. Other diagnoses/indications

1. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is

NOT authorized): CP.CPA.09 for commercial, HIM.PHAR.21 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

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

1. Currently receiving medication via Centene benefit 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 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:

Medicaid/HIM – 12 months

Commercial – 6 months or to the member's renewal date, whichever is longer

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

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy.

Approval duration: Duration of request or 6 months (whichever is less); or

2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PHAR.21 for health insurance marketplace, and CP.PMN.53 for Medicaid

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 policies – CP.CPA.09 for commercial, HIM.PHAR.21 for health insurance marketplace, CP.PMN.53 for Medicaid, or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

FDA: Food and Drug Administration

MPS: mucopolysaccharidosis

FVC: forced vital capacity

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.

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; 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	P&T Approval Date
Policy converted to new template; Criteria: age criteria added; moderate to severe symptoms in regard to patients with the Scheie form of MPS I changed to attestation rather than FVC $\leq 80\%$ of predicted normal; re-authorization criteria added.	01.16	02.16

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Age restriction removed; Allergy history removed; Initial approval duration extended to 6 months; Positive response to therapy added. Background section converted to new template.	12.16	02.17
Policy converted to newer template; added age restriction.	09.05.17	11.17
2Q 2018 annual review: no significant changes from previously approved corporate policy; policies combined for commercial, HIM, and Medicaid lines of business; Commercial: simplified policy requirements to align with previously approved policy for Medicaid; 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	05.18
2Q 2019 annual review: no significant changes; 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.	02.28.19	05.19
2Q 2020 annual review: no significant changes; revised HIM-Medical Benefit to HIM line of business; references reviewed and updated.	02.21.20	05.20

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. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or

regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. 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. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

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Note:

For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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