

Clinical Policy: Laronidase (Aldurazyme)

Reference Number: CP.PHAR.152

Effective Date: 02/16

Last Review Date: 02/17

[Revision Log](#)
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See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

The intent of the criteria is to ensure that patients follow selection elements established by Centene® medical policy for laronidase (Aldurazyme®).

Policy/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. 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. If diagnosis of MPS I Scheie form, symptoms are moderate to severe.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to CP.PHAR.57 - Global Biopharm Policy

II. Continued approval

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

1. Currently receiving medication via Centene benefit or member has previously met all initial approval criteria;
2. Member is responding positively to therapy.

Approval duration: 12 months

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

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy; or
2. Refer to CP.PHAR.57 - Global Biopharm Policy.

Background

Description/Mechanism of Action:

Mucopolysaccharide storage disorders are caused by the deficiency of specific lysosomal enzymes required for the catabolism of glycosaminoglycans (GAG). Mucopolysaccharidosis I (MPS I) is characterized by the deficiency of alpha-L-iduronidase, a lysosomal hydrolase which catalyzes the hydrolysis of terminal alpha-L-iduronic acid residues of dermatan sulfate and

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heparan sulfate. Reduced or absent alpha-L-iduronidase activity results in the accumulation of the GAG substrates, dermatan sulfate and heparan sulfate, throughout the body and leads to widespread cellular, tissue, and organ dysfunction. The rationale of Aldurazyme therapy in MPS I is to provide exogenous enzyme for uptake into lysosomes and increase the catabolism of GAG. Aldurazyme uptake by cells into lysosomes is most likely mediated by the mannose-6-phosphate-terminated oligosaccharide chains of laronidase binding to specific mannose-6-phosphate receptors. Because many proteins in the blood are restricted from entry into the central nervous system (CNS) by the blood brain barrier, effects of intravenously administered Aldurazyme on cells within the CNS cannot be inferred from activity in sites outside the CNS. The ability of Aldurazyme to cross the blood brain barrier has not been evaluated in animal models or in clinical studies.

Formulations:

Aldurazyme (laronidase): Solution for reconstitution; for intravenous use

- 2.9 mg/5 mL vial; 0.58 mg/mL (172 units/mg)

FDA Approved Indications:

Aldurazyme (laronidase) is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme/intravenous formulation indicated for:

- 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.
 - The risks and benefits of treating mildly affected patients with the Scheie form have not been established.
 - Aldurazyme has been shown to improve pulmonary function and walking capacity.
 - Aldurazyme has not been evaluated for effects on the central nervous system manifestations of the disorder.

Appendices

Appendix A: Abbreviation Key

GAG: Glycosaminoglycan

MPS: Mucopolysaccharidosis

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
Policy converted to new template.	01/16	02/16

Reviews, Revisions, and Approvals	Date	Approval Date
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.		
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

References

1. Aldurazyme prescribing information. Cambridge, MA: Genzyme Corporation; April 2013. Available at <https://www.aldurazyme.com/healthcare/about-aldurazyme/clinical-pharmacology.aspx>. Accessed December 14, 2016.
2. Muenzer J. The mucopolysaccharidoses: A heterogeneous group of disorders with variable pediatric presentations. *J Pediatr.* 2004; 144(5 Suppl): S27-S34.

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.

Note: For Medicare members, to ensure consistency with the Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), all applicable NCDs, LCDs and Medicare Coverage Articles should be reviewed prior to applying the criteria set forth in this clinical policy. Refer to the CMS website at <http://www.cms.gov> for additional information.

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