Clinical Policy: Laronidase (Aldurazyme)
Reference Number: CP.PHAR.152
Effective Date: 02.01.16
Last Review Date: 05.19
Line of Business: Commercial, HIM-Medical Benefit, Medicaid

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 (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 ≥ 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 and CP.PMN.53 for Medicaid and HIM-Medical Benefit.

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 and CP.PMN.53 for Medicaid and HIM-Medical Benefit.

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 and CP.PMN.53 for Medicaid and HIM-Medical Benefit, or evidence of coverage documents.

IV. Appendices/General Information
   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 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.

V. Dosage and Administration

<table>
<thead>
<tr>
<th>Indication</th>
<th>Dosing Regimen</th>
<th>Maximum Dose</th>
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<tr>
<td>MPS I</td>
<td>0.58 mg/kg IV once weekly</td>
<td>0.58 mg/kg/week</td>
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VI. Product Availability

Vial: 2.9 mg/5 mL

VII. References


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.

<table>
<thead>
<tr>
<th>HCPCS Codes</th>
<th>Description</th>
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<tbody>
<tr>
<td>J1931</td>
<td>Injection, laronidase, 0.1 mg</td>
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Reviews, Revisions, and Approvals

<table>
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<tr>
<th>Description</th>
<th>Date</th>
<th>P&amp;T Approval Date</th>
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<tbody>
<tr>
<td>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 ≤80% of predicted normal; re-authorization criteria added.</td>
<td>01.16</td>
<td>02.16</td>
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<tr>
<td>Age restriction removed; Allergy history removed; Initial approval duration extended to 6 months; Positive response to therapy added. Background section converted to new template.</td>
<td>12.16</td>
<td>02.17</td>
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<tr>
<td>Policy converted to newer template; added age restriction.</td>
<td>09.05.17</td>
<td>11.17</td>
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**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.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

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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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