Aldurazyme is an enzyme replacement therapy that was approved by the FDA in 2003 for the treatment of Mucopolysaccharidosis type I (MPS I), including patients with Hurler syndrome, Hurler-Scheie syndrome, and Scheie syndrome with moderate to severe symptoms. The risks and benefits of treating mildly affected patients with Scheie syndrome have not been established. MPS I is a rare genetic lysosomal storage disease, with Hurler syndrome being the most severe and most common subtype and Scheie syndrome as the rarest and mildest of the attenuated forms. Pathogenic mutations of the IDUA gene cause the enzyme alpha-L-iduronidase (IDUA) to be deficient or absent. Normally this lysosomal enzyme breaks down glycosaminoglycans (GAGs) (previously known as mucopolysaccharides) but when it is reduced in MPS I, the GAG substrates heparan sulfate (HS) and dermatan sulfate (DS) accumulate throughout the body leading to widespread cellular, tissue, and organ dysfunction. Aldurazyme provides an exogenous form of the deficient enzyme.

Aldurazyme (laronidase) will be considered for coverage when the following criteria are met:

**Mucopolysaccharidosis I (MPS I)**

For initial authorization:
1. Member is at least 6 months of age; AND
2. Medication must be prescribed by or in consultation with a geneticist, metabolic specialist, or pediatrician experienced with managing mucopolysaccharidoses; AND
3. Member has a documented diagnosis of ONE of the following forms of MPS I:
   a) Hurler syndrome,
   b) Hurler-Scheie syndrome, or
   c) Scheie syndrome with moderate to severe symptoms; AND
4. Member’s clinical diagnosis of MPS I has been confirmed by at least one of the following:
   a) Low IDUA enzyme activity (less than 10%), and/or
   b) Molecular genetic testing identifies pathogenic IDUA gene mutation; AND
5. Documentation of elevated baseline urinary GAG (uGAG) level.
6. Dosage allowed/Quantity limit: 0.58 mg/kg IV infusion once weekly

*If all the above requirements are met, the medication will be approved for 6 months.*
For **reauthorization**:

1. Chart notes must show improvement or stabilized signs and symptoms of disease such as improved functional capacity (e.g. 6-minute walk test, forced vital capacity (FVC)) compared to baseline, reduced liver size, and/or reduced uGAG levels.

   **If all the above requirements are met, the medication will be approved for an additional 12 months.**

**CareSource considers Aldurazyme (laronidase) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.**

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<tr>
<th>DATE</th>
<th>ACTION/DESCRIPTION</th>
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<tbody>
<tr>
<td>07/26/2021</td>
<td>New policy for Aldurazyme created.</td>
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**References:**


Effective date: 01/01/2022
Revised date: 07/26/2021