Laronidase (Aldurazyme®)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
Aldurazyme is indicated for the treatment of 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.

Limitations of Use
- The safety and effectiveness of treating mildly affected patients with the Scheie form have not been established.
- The effect of Aldurazyme on central nervous system manifestations of the disorder has not been determined.

All other indications are considered experimental/investigational and not medically necessary.

DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
Initial Requests:
- Alpha-L-iduronidase enzyme assay or genetic testing results supporting diagnosis.
- Chart notes or medical records documenting clinical signs and symptoms of disease at baseline (e.g., coarse facial features, frequent upper respiratory infections, inguinal or umbilical hernia, hepatosplenomegaly, characteristic skeletal findings, noninflammatory arthropathy, corneal clouding, hydrocephalus, developmental delay).

Continuation Requests:
- Chart notes or medical records documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression (e.g., improvement, stabilization, or slowing of disease progression for forced vital capacity, 6-minute walk test, apnea/ hypoapnea index (AHI), liver volume, active joint range of motion, urinary glycosaminoglycan (GAG) levels).

PRESCRIBER SPECIALITIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

COVERAGE CRITERIA

Mucopolysaccharidosis I (MPS I)

Authorization of 12 months may be granted for treatment of MPS I when all of the following criteria are met:

- Diagnosis of MPS I was confirmed by enzyme assay demonstrating a deficiency of alpha-L-iduronidase enzyme activity or by genetic testing.
- Member has one of the following:
  - The Hurler form (i.e., severe MPS I).
  - The Hurler-Scheie form (i.e., attenuated MPS I).
  - The Scheie form (Scheie syndrome; i.e., attenuated MPS I) with moderate to severe symptoms (e.g., normal intelligence, less progressive physical problems, corneal clouding, joint stiffness, valvular heart disease).
- Member exhibits clinical signs and symptoms of disease at baseline (e.g., coarse facial features, frequent upper respiratory infections, inguinal or umbilical hernia, hepatosplenomegaly, characteristic skeletal findings, noninflammatory arthropathy, corneal clouding, hydrocephalus, developmental delay).

CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the Coverage Criteria section who have a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression (e.g., improvement, stabilization, or slowing of disease progression in forced vital capacity, 6-minute walk test, apnea/hypoapnea index (AHI), liver volume, active joint range of motion, urinary glycosaminoglycan (GAG) levels).

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. Aldurazyme [package insert]. Cambridge, MA: Genzyme Corporation; December 2023.

2. Wraith JE, Clarke LA, Beck M, et al. Enzyme replacement therapy for mucopolysaccharidosis I: a randomized, double-blinded, placebo-controlled, multinational study of recombinant human alpha-L-iduronidase (laronidase). J Pediatr. 2004;144:581-588.
3. Muenzer J, Wraith JE, Clarke LA; International Consensus Panel on Management and Treatment of Mucopolysaccharidosis I. Mucopolysaccharidosis I: management and treatment guidelines. Pediatrics. 2009 Jan;123(1):19-29.
4. Clarke LA. Mucopolysaccharidosis Type I. 2002 Oct 31 [Updated 2025 Dec 4]. In: Adam MP, Everman DB, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2023. Accessed Jan 13, 2025.

EFFECTIVE DATE 9/30/2026

ID_CHS_2026