Approved Revision: Do Not Implement Until 6/2/21

Laronidase (Aldurazyme®)

NDC CODE(S) 58468-0070-XX - ALDURAZYME 2.9MG/5ML Solution (GENZYME)

DESCRIPTION

Laronidase is a recombinant form of the human enzyme L-iduronidase (alpha-L-iduronidase). L-iduronidase is a lysosomal enzyme that is necessary for the degradation of glycosaminoglycans to its substrates dermatan sulfate and heparan sulfate. Without this enzyme these substrates accumulate throughout the body leading to widespread cellular, tissue and organ dysfunction.

Individuals with inherited deficiency of L-iduronidase have the lysosomal storage disease mucopolysaccharidosis type I. Treatment with laronidase reverses the metabolic and pathologic abnormalities outside the central nervous system. Mucopolysaccharidosis type I is classified into three distinct subgroups:

- Hurler's syndrome - most severe form, with neurologic, skeletal, and visceral involvement, including hepatosplenomegaly, cardiac disease, airway obstruction, mental retardation/development delay, corneal clouding, and severe skeletal abnormalities; death often occurs before the age of ten.
- Hurler-Scheie syndrome - intermediate form characterized by slower progression of same types of complications, but with minimal-to-no mental retardation; death is usually later (e.g., 20s).
- Scheie's syndrome - least severe with less extensive disease; some individuals may have a normal life span.

POLICY

- Laronidase for the treatment of Mucopolysaccharidosis type I is considered ***medically necessary*** if the medical appropriateness criteria are met. **(See Medical Appropriateness below.)**
- Laronidase for the treatment of other conditions/diseases is considered ***investigational.***

MEDICAL APPROPRIATENESS

INITIAL APPROVAL CRITERIA

- Patient is at least 6 months of age or older; **AND**
- Patient has absence of severe cognitive impairment; **AND**
- Documented baseline value for urinary glycosaminoglycan (uGAG) has been obtained; **AND**
- Documented baseline values for one or more of the following have been obtained:
  - Patients 6 years or greater: percent predicted forced vital capacity (FVC), 6-minute walk test, joint range of motion, left ventricular hypertrophy, growth, quality of life (CHAQ/HAQ/MPS HAQ); **OR**
  - Patients 6 months to less than 6 years: cardiac status, upper airway obstruction during sleep, growth velocity, mental development, FVC, and/or 6-minute walk test; **AND**

Mucopolysaccharidosis I (MPS I)

- Patient has a definitive diagnosis of MPS I confirmed by one of the following:
  - Detection of biallelic pathogenic mutations in the IDUA gene by molecular genetic testing; **OR**
  - Detection of deficient activity of the lysosomal enzyme α-L-iduronidase (IDUA); **AND**
- Patient has one of the following diagnoses:
  - Diagnosis of Hurler (severe) or Hurler-Scheie (attenuated) forms of disease; **OR**

- Diagnosis of Scheie (attenuated) form of disease with moderate to severe symptoms

RENEWAL CRITERIA

- Patient continues to meet indication-specific relevant criteria identified in the Initial Approval Criteria; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: anaphylaxis and severe hypersensitivity reactions, acute respiratory complications, acute cardiorespiratory failure, severe infusion reactions, etc.; **AND**
- Patient does not have progressive/irreversible severe cognitive impairment; **AND**
- Patient has a documented reduction in uGAG levels compared to pretreatment baseline; **AND**
- Patient has demonstrated a beneficial response to therapy compared to pretreatment baseline in one or more of the following:
  - Patients 6 years or greater: stability or improvement in percent predicted FVC and/or 6-minute walk test, increased joint range of motion, decreased left ventricular hypertrophy, improved growth, improved quality of life (clinically meaningful change in the CHAQ/HAQ/MPS HAQ disability index); **OR**
  - Patients 6 months to less than 6 years: stability or improvement in cardiac status, upper airway obstruction during sleep, growth velocity, mental development, FVC and/or 6-minute walk test

DOSAGE/ADMINISTRATION

INDICATION	DOSE
Mucopolysaccharidosis I (MPS I)	0.58 mg/kg of body weight administered once weekly as an intravenous infusion

LENGTH OF AUTHORIZATION

- Coverage will be provided for 12 months and may be renewed.

DOSING LIMITS

Max Units (per dose and over time) [HCPCS Unit]:

- 667 billable units every 7 days

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.

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, the express terms of the health plan will govern.

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).

SOURCES

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5. Clarke LA, Wraith JE, Beck M, et al. Long-term efficacy and safety of laronidase in the treatment of mucopolysaccharidosis I. Pediatrics. 2009 Jan;123(1):229-40. doi: 10.1542/peds.2007-3847.
6. Wraith JE, Beck M, Lane R, et al. Enzyme replacement therapy in patients who have mucopolysaccharidosis I and are younger than 5 years: results of a multinational study of recombinant human alpha-L-iduronidase (laronidase). Pediatrics. 2007 Jul;120(1):e37-46. Epub 2007 Jun 4.
7. Lexicomp Online. (2021). AHFS DI. Laronidase. Retrieved February 8, 2021 from Lexicomp Online with AHFS DI.
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EFFECTIVE DATE 6/2/2021

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