Reference number(s)
4811-A

STANDARD MEDICARE PART B MANAGEMENT

ALDURAZYME (laronidase)

POLICY

I. 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 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 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.*

All other indications will be assessed on an individual basis. Submissions for indications other than those enumerated in this policy should be accompanied by supporting evidence from Medicare approved compendia.

II. DOCUMENTATION

The following documentation must be available, upon request, for all submissions:
A. Initial requests: alpha-L-iduronidase enzyme assay and/or genetic testing results supporting diagnosis.
B. Continuation requests: chart notes documenting a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

III. CRITERIA FOR INITIAL APPROVAL

Mucopolysaccharidosis I (MPS I)
Authorization of 12 months may be granted for treatment of MPS I when both of the following criteria are met:
A. Diagnosis of MPS I was confirmed by enzyme assay demonstrating a deficiency of alpha-L-iduronidase enzyme activity and/or by genetic testing.
B. Member has the Hurler (i.e., severe MPS I) or Hurler-Scheie (i.e., attenuated MPS I) OR the member has 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).

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must be currently receiving therapy with the requested agent.

Authorization for 12 months may be granted when all of the following criteria are met:
A. The member is currently receiving therapy with Aldurazyme
B. Aldurazyme is being used to treat an indication enumerated in Section III
C. The member is receiving benefit from therapy. Benefit is defined as a clinically positive response to therapy, which shall include improvement, stabilization, or slowing of disease progression.

V. SUMMARY OF EVIDENCE

The contents of this policy were created after examining the following resources:
1. The prescribing information for Aldurazyme.
2. The available compendium
    a. National Comprehensive Cancer Network (NCCN) Drugs and Biologics Compendium
    b. Micromedex DrugDex
    c. American Hospital Formulary Service- Drug Information (AHFS-DI)
    d. Lexi-Drugs
    e. Clinical Pharmacology
3. International Consensus Panel on Management and Treatment of Mucopolysaccharidosis I. Mucopolysaccharidosis I: management and treatment guidelines.

After reviewing the information in the above resources, the FDA-approved indications listed in the prescribing information for Aldurazyme are covered.

VI. EXPLANATION OF RATIONALE

Support for FDA-approved indications can be found in the manufacturer's prescribing information.

Support for using enzyme assays or genetic testing prior to initiating Aldurazyme to treat MPSI can be found in a GeneReviews article (Clark LA). The diagnosis of MPSI can be established in one of two ways: detection of deficiency activity of the lysosomal enzyme α-L-iduronidase (IDUA) in combination with elevation of glycosaminoglycan levels; and/or identification of biallelic pathogenic variants in IDUA on molecular genetic testing.

VII. REFERENCES

1. Aldurazyme [package insert]. Cambridge, MA: Genzyme Corporation; December 2019.
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 2021 Feb 25]. In: Adam MP, Everman DB, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2023. Accessed January 5, 2022.