POLICY Document for Aldurazyme

The overall objective of this policy is to support the appropriate and cost effective use of the medication, specific to use of lower cost site of care and overall clinically appropriate use. This document provides specific information to each section of the overall policy.

Section 1: Site of Care
- Policy information specific to site of care (outpatient, hospital outpatient, home infusion)

Section 2: Clinical Criteria
- Policy information specific to the clinical appropriateness for the medication

Section 1: Site of Care

Site of Care Criteria
Administration of Intravenous Aldurazyme

POLICY

I. CRITERIA FOR APPROVAL FOR ADMINISTRATION IN OUTPATIENT HOSPITAL SETTING

This policy provides coverage for administration of Aldurazyme in an outpatient hospital setting for up to 8 doses when a member is new to therapy.

This policy provides coverage for administration of Aldurazyme in an outpatient hospital setting for a longer course of treatment when ANY of the following criteria are met:

A. The member has experienced an adverse reaction that did not respond to conventional interventions (eg, acetaminophen, steroids, diphenhydramine, fluids or other pre-medications) or a severe adverse event (anaphylaxis, anaphylactoid reactions, myocardial infarction, thromboembolism, or seizures) during or immediately after an infusion.
B. The member has developed laboratory confirmed laronidase IgE antibodies which increases the risk for infusion related reactions.
C. The member is medically unstable (eg respiratory, cardiovascular, or renal conditions).
D. The member has severe venous access issues that require the use of a special intervention.
E. The member has significant behavioral issues and/or physical or cognitive impairment that would impact the safety of the infusion therapy AND the patient does not have access to a caregiver.
F. Alternative infusion sites are not available.
G. The member is less than 21 years of age or 65 years of age or older.

For situations where administration of Aldurazyme does not meet the criteria for outpatient hospital infusion, coverage for Aldurazyme is provided when administered in alternative sites such as; physician office, home infusion or ambulatory care.

II. REQUIRED DOCUMENTATION

The following information is necessary to initiate the site of care prior authorization review (where applicable):

A. Medical records supporting the member has experienced an adverse reaction that did not respond to conventional interventions or a severe adverse event during or immediately after an infusion
B. Medical records supporting the member has developed laronidase IgE antibodies
C. Medical records supporting the member is medically unstable
D. Medical records supporting the member has severe venous access issues

Aldurazyme_Specialty Medical SOC 2019

E. Medical records supporting the member has behavioral issues and/or physical or cognitive impairment and no access to a caregiver
Records supporting alternative infusion sites are not available

Section 2: Clinical Criteria

SPECIALTY GUIDELINE 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 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. The risks and benefits of treating mildly affected patients with the Scheie form have not been established.

All other indications are considered experimental/investigational and are not a covered benefit.

II. CRITERIA FOR INITIAL APPROVAL

Mucopolysaccharidosis I (MPS I)
Indefinite authorization may be granted for treatment of MPS I when both of the following criteria are met:
1. Diagnosis of MPS I was confirmed by enzyme assay demonstrating a deficiency of alpha-L-iduronidase enzyme activity or by genetic testing.
2. Member has the Hurler or Hurler-Scheie form of MPS I OR the member has the Scheie form (Scheie syndrome) with moderate to severe symptoms.

III. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

Aldurazyme_Specialty Medical SOC 2019

REFERENCES:

SECTION 1

1. Aldurazyme [package insert]. Cambridge, MA: Genzyme Corporation.; April 2013.
2. Giugliani R, Rojas VM, Martins AM, et al. A dose-optimization trial of laronidase (Aldurazyme) in patients with mucopolysaccharidosis I. *Mol Genet Metab.* 2009;96(1):13-19.
3. Clarke LA, Wraith JE, Beck M, et al. Long-term efficacy and safety of laronidase in the treatment of mucopolysaccharidosis I. *Pediatrics.* 2009;123(1):229-240.

SECTION 2

1. Aldurazyme [package insert]. Cambridge, MA: Genzyme Corporation; April 2013.
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.

Aldurazyme_Specialty Medical SOC 2019