

## **SPECIALTY GUIDELINE MANAGEMENT**

### **ALDURAZYME (Iaronidase)**

#### **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. REQUIRED DOCUMENTATION**

The following information is necessary to initiate the prior authorization review: enzyme assay or genetic testing results supporting diagnosis.

##### **III. CRITERIA FOR INITIAL APPROVAL**

###### **A. Mucopolysaccharidosis I (MPS I)**

Authorization for 12 months 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.

##### **IV. CONTINUATION OF THERAPY**

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

##### **V. DOSAGE AND ADMINISTRATION**

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

##### **VI. REFERENCES**

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 (Iaronidase). *J Pediatr.* 2004;144:581-588.