Elaprase (idursulfase)

Override(s)	Approval Duration
Prior Authorization	1 year

Medications	Dosing Limit
Elaprase (idursulfase) 6 mg vial	0.5 mg/kg once per week

APPROVAL CRITERIA

Initial requests for Elaprase (idursulfase) may be approved if the following criteria are met:

I. Individual has a diagnosis of Mucopolysaccharidosis II (MPS II, Hunter syndrome) confirmed by (Scarpa 2011, Wraith 2008):
   A. Deficiency in iduronate 2-sulfatase enzyme activity as measured in fibroblasts or leukocytes combined with normal enzyme activity level of another sulfatase, and documentation is provided; **OR**
   B. Pathologic iduronate 2-sulfatase gene mutation, and documentation is provided;

AND

II. The individual has symptoms attributable to MPS II including (Muenzer 2012, Wraith 2008):
   A. Developmental delay or cognitive impairment; **OR**
   B. Frequent infections; **OR**
   C. Hearing loss; **OR**
   D. Hepatosplenomegaly; **OR**
   E. Hernias; **OR**
   F. Impaired respiratory function; **OR**
   G. Joint pain; **OR**
   H. Skeletal deformities; **OR**
   I. Sleep apnea; **OR**
   J. Valvular heart disease.

Continuation requests for Elaprase (idursulfase) may be approved if the following criterion is met

I. Documentation is provided to show clinically significant improvement or stabilization in clinical signs and symptoms of disease (including but not limited to reduction in urinary GAG excretion, reduction in hepatosplenomegaly, improvement in pulmonary function, improvement in walking distance and/or improvement in fine or gross motor function) compared to the predicted natural history trajectory of disease.

Elaprase (idursulfase) is may not be approved when the criteria above are not met and for all other indications.

Note:
Elaprase has a black box warning for risk of anaphylaxis. Life-threatening anaphylactic reactions have occurred and up to 24 hours after Elaprase infusions. Appropriate medical support should be available during Elaprase administration, and individuals should be educated on the signs and symptoms of anaphylaxis and to seek immediate medical care should they occur. Individuals with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to hypersensitivity reactions, and require additional monitoring.

Key References:

1. DailyMed. Package inserts. U.S. National Library of Medicine, National Institutes of Health website. http://dailymed.nlm.nih.gov/dailymed/about.cfm. Accessed: September 11, 2020.
2. DrugPoints® System [electronic version]. Truven Health Analytics, Greenwood Village, CO. Updated periodically.
3. Lehman TJA, Miller Nicole, Norquist B, et al. Diagnosis of the mucopolysaccharidoses. *Rheumatology*. 2011; 50:V41-V46.
4. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc.; 2020; Updated periodically.
5. Muenzer J, Bodamer O, Burton B, et al. The role of enzyme replacement therapy in severe Hunter syndrome-an expert panel consensus. *Eur J Pediatr*. 2012; 171(1):181-188.
6. Scarpa M, Almássy Z, Beck M, et al. Mucopolysaccharidosis type II: European recommendations for the diagnosis and multidisciplinary management of a rare disease. *Orphanet J Rare Dis*. 2011; 6:72.
7. Wang RY, Bodamer OA, Watson MS, Wilcox WR. American College of Medical Genetics (ACMG) Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genet Med*. 2011; 13(5):457-484.
8. Wraith JE, Scarpa M, Beck M, et al. Mucopolysaccharidosis type II (Hunter syndrome): a clinical review and recommendations for treatment in the era of enzyme replacement therapy. *Eur J Pediatr*. 2008; 167(3):267-277.