Medical Drug Clinical Criteria

Subject:	Elaprase (idursulfase)		
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Overview

This document addresses Elaprase (idursulfase), an enzyme replacement therapy approved by the Food and Drug Administration (FDA) to treat individuals with Mucopolysaccharidosis II (Hunter syndrome).

The mucopolysaccharidoses are a group of inherited metabolic diseases caused by the deficiency of lysosomal enzymes needed to breakdown mucopolysaccharides or glycosaminoglycan (GAGs). The progressive accumulation of GAGs in lysosomes leads to respiratory, cardiac, skeletal and connectivity, neurologic and ophthalmologic complications. There are seven distinct types of mucopolysaccharidosis (I, II, III, IV, VI, VII, and IX). Accurate diagnosis is important to provide disease-specific enzyme replacement therapy. Diagnosis is confirmed through urinary GAG concentration measurement, enzymatic activity measurement or genetic testing.

Elaprase has a black box warning for anaphylaxis. Life-threatening anaphylactic reactions have occurred during and up to 24 hours after Elaprase infusions. Appropriate medical support should be available during Elaprase administration. 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 disease and require additional monitoring.

Clinical Criteria

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

Elaprase (idursulfase)

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) demonstrated 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 that there is 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) may not be approved when the above criteria are not met and for all other indications.

Quantity Limits

Elaprase (idursulfase) Quantity Limit

Drug	Limit
Elaprase (idursulfase) 6 mg vial	0.5 mg/kg once a week

Coding

The following codes for treatments and procedures applicable to this document are included below for informational purposes. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Please refer to the member's contract benefits in effect at the time of service to determine coverage or non-coverage of these services as it applies to an individual member.

HCPCS

J1743	Injection, idursulfase, 1 mg [Elaprase]
S9357	Home infusion therapy, enzyme replacement intravenous therapy, (e.g., Imiglucerase); administrative services, professional pharmacy services, care coordination, and all necessary supplies and equipment (drugs and nursing visits coded separately), per diem

ICD-10 Diagnosis

E76.1	Mucopolysaccharidosis, type II (Hunter's syndrome)

Document History

Reviewed: 9/9/2024
Document History:

- 9/9/2024 – Annual Review: No changes. Coding Reviewed: No changes.
- 9/11/2023 – Annual Review: Wording and formatting changes. Coding Reviewed: No changes.9/12/2022 – Annual Review: No changes. Coding Reviewed: No changes.
- 9/13/2021 – Annual Review: Wording and formatting changes. Coding reviewed: No changes.
- 08/01/2021 – Administrative update to add documentation.
- 09/14/2020 – Annual Review: Addition of continuation criteria. Coding Reviewed: No changes.
- 09/23/2019 – Administrative update to add drug specific quantity limit.
- 09/9/2019 – Annual Review: Wording and formatting changes. Coding reviewed: No changes.
- 08/17/2018 – Annual Review: Add references for non-label based criteria elements.

References

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3. Lehman TJ, Miller N, Norquist B, Underhill L, Keutzer J. Diagnosis of the mucopolysaccharidoses. *Rheumatology (Oxford)*. 2011;50 Suppl 5:v41-v48. doi:10.1093/rheumatology/ker390.
4. Lexi-Comp ONLINE™ with AHFS™, Hudson, Ohio: Lexi-Comp, Inc. 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.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.