Elaprase (idursulfase)

**Background**
Hunter Syndrome (mucopolysaccharidosis type II or MPS II) is an X-linked recessive disease caused by the body’s inability to break down certain elements in the body called mucopolysaccharides, also known as glycosaminoglycans (GAG) due to insufficient levels of the lysosomal enzyme iduronate-2-sulfatase (I2S). The missing or defective enzyme causes mucopolysaccharides to accumulate in a variety of cells, leading to cellular growth, organ enlargement, tissue destruction, and organ system dysfunction. Elaprase replaces the deficient or absent enzyme to breakdown the excess buildup within the cells, returning the cells to normal size (1).

**Regulatory Status**
FDA-approved indication: Elaprase is indicated for patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II). Elaprase has shown to improve walking capacity in patients 5 years or older (1).

Elaprase carries a boxed warning regarding the risk of life-threatening anaphylaxis reactions during infusions. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to infusion reactions, and require additional monitoring. Appropriate medical support should be readily available when Elaprase is administered (1).

Safety and efficacy have not been established in pediatric patients less than five years of age (1).
Related policies
Aldurazyme, Mepsevii, Naglazyme, Vimizim

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Elaprase may be considered **medically necessary** for the treatment of Hunter syndrome in patients 5 years of age or older.

Elaprase may be considered **investigational** in patients younger than 5 years of age and for all other indications.

Prior-Approval Requirements

Age
5 years of age or older

Diagnosis

Patient must have the following:

Hunter Syndrome (mucopolysaccharidosis type II)

Prior – Approval *Renewal* Requirements

Same as above

Policy Guidelines

Pre - PA Allowance
None

Prior - Approval Limits

Duration 2 years

Prior – Approval *Renewal* Limits

Same as above
Rationale

Summary
Elaprase is indicated for patients with Hunter syndrome (Mucopolysaccharidosis II, MPS II). Elaprase carries a boxed warning regarding the risk of life-threatening anaphylaxis reactions during infusions. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation of their respiratory compromise due to infusion reactions, and require additional monitoring. Appropriate medical support should be readily available when Elaprase is administered. Safety and efficacy have not been established in pediatric patients less than five years of age (1).

Prior approval is required to ensure the safe, clinically appropriate and cost effective use of Elaprase while maintaining optimal therapeutic outcomes.

References

Policy History

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<td>September 2011</td>
<td>New Policy aligned with MPRM.</td>
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<td>September 2012</td>
<td>Annual editorial review and reference update.</td>
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<td>Changed approval duration from lifetime to 2 years</td>
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Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 6, 2019 and is effective on January 1, 2020.