Mepsevii

Description

Mepsevii (vestronidase alfa-vjbk)

Background
Mepsevii is an enzyme used to treat patients with Mucopolysaccharidosis Type VII (Sly syndrome). Sly syndrome is an inherited, rare genetic condition that causes various skeletal abnormalities that become more pronounced with age, including short stature. Affected individuals can also develop heart valve abnormalities, enlarged liver and spleen, and narrowed airways which can lead to lung infections and trouble breathing. Mepsevii is intended to replace the missing enzyme called beta-glucuronidase, which when missing causes an abnormal buildup of toxic materials in the body’s cells (1).

Regulatory Status
FDA-approved indication: Mepsevii is a recombinant human lysosomal beta glucuronidase indicated in pediatric and adult patients for the treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome) (1).

Limitations of Use:
The effect of Mepsevii on the central nervous system manifestations of MPS VII has not been determined (1).

Mepsevii has a boxed warning for life-threatening anaphylactic reaction that has occurred in some patients during infusions. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation due to infusion reactions, and
require additional monitoring. Appropriate medical support should be readily available when Mepsevii is administered. Closely observe patients during and after Mepsevii administration and be prepared to manage anaphylaxis. Inform patients of the signs and symptoms of anaphylaxis and have them seek immediate medical care should symptoms occur (1).

The safety and effectiveness of Mepsevii have been established in pediatric patients less than 18 years of age (1).

**Related policies**
Aldurazyme, Elaprase, 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.*

Mepsevii may be considered **medically necessary** in patients with Mucopolysaccharidosis Type VII (MPS VII) (Sly syndrome) and if the conditions indicated below are met.

Mepsevii may be considered **investigational** for all other indications.

**Prior-Approval Requirements**

**Diagnosis**

Patient must have the following:

Mucopolysaccharidosis Type VII (MPS VII) (Sly syndrome)

**AND** at least **ONE** of the following:

1. Documented signs and symptoms of MPS VII such as skeletal abnormalities and decreased level of beta-glucuronidase activity in blood
2. Genetic testing confirming diagnosis of MPS VII

**Prior – Approval Renewal Requirements**

**Diagnosis**
Patient must have the following:

Mucopolysaccharidosis Type VII (MPS VII) (Sly syndrome)

Policy Guidelines

Pre - PA Allowance
None

Prior - Approval Limits
Duration 2 years

Prior – Approval Renewal Limits
Same as above

Rationale

Summary
Mepsevii is an enzyme used to treat patients with Mucopolysaccharidosis Type VII (Sly syndrome). Sly syndrome is an inherited, rare genetic condition that causes various skeletal abnormalities that become more pronounced with age, including short stature. Anaphylaxis has been reported to occur during Mepsevii infusions, regardless of duration of the course of treatment. Patients with compromised respiratory function or acute respiratory disease may be at risk of serious acute exacerbation due to infusion reactions, and require additional monitoring. Appropriate medical support should be readily available when Mepsevii is administered (1).

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

References

Policy History

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June 2018  
December 2019  
Annual editorial review. Changed approval duration from lifetime to 2 years

**Keywords**

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