Mepsevii

(vestronidase alfa-vjbk)

NEW PRODUCT SLIDESHOW

MPR

Introduction

- **Brand name:** Mepsevii
- **Generic name:** Vestronidase alfa-vjbk
- **Pharmacological class:** Recombinant human lysosomal beta glucuronidase
- **Strength and Formulation:** 2mg/mL; soln for IV infusion after dilution; preservative-free
- **Manufacturer:** Ultragenyx Pharmaceutical Inc.
- **How supplied:** Single-dose vial (5mL)—1
- **Legal Classification:** Rx

Mepsevii


NDC 69794-001-01
Rx only
Mepsevii™
(vestronidase alfa-vjbk)
10 mg/5 mL (2 mg/mL)
For Intravenous Infusion
Must dilute before use

Indications

- Treatment of Mucopolysaccharidosis VII (MPS VII, Sly syndrome)
- **Limitations of use:** Effect on the CNS manifestations of MPS VII has not been determined

Dosage & Administration

- Premedicate with a non-sedating antihistamine with or without an antipyretic 30–60mins prior to infusion
- Give as IV infusion over 4hrs (infuse first 2.5% of total volume over 1hr, then increase rate as tolerated over next 3hrs); see full labeling
- 4mg/kg every 2 weeks

Considerations for Special Populations

- **Pregnancy:** No available data in pregnant women to establish drug-associated risk
- **Nursing mothers:** Consider mother's need and potential adverse effects on child

Warnings/Precautions

- Have appropriate medical support readily available
- Should be administered under supervision of healthcare professional
- Monitor during and for ≥60mins post-infusion for **anaphylaxis**; discontinue immediately if a severe systemic reaction occurs

Adverse Reactions

- Infusion site extravasation
- Diarrhea
- Rash
- Anaphylaxis
- Infusion site swelling
- Peripheral swelling
- Pruritus

Mechanism of Action

- **Vestronidase alfa-vjbk** provides exogenous beta-glucuronidase (GUS) enzyme for uptake into cellular lysosomes
- Mannose-6-phosphate (M6P) residues on the oligosaccharide chains allow binding of the enzyme to cell surface receptors, leading to cellular uptake of the enzyme, targeting to lysosomes and subsequent catabolism of accumulated GAGs in affected tissues

Clinical Studies

- The Mepsevii clinical program included 23 patients with MPS VII, 17 of whom were evaluable for efficacy, 20 for safety, and 23 for immunogenicity
- Patients received doses up to 4mg/kg once every 2 weeks for up to 164 weeks

Clinical Studies

- **Study 301** was a randomized start trial in patients with MPS VII (N=12)
- Motor function, forced vital capacity, and visual acuity were assessed after 24 weeks of treatment and measured against pre-specified minimal important differences

Clinical Studies

- Repeated assessments of the 6 minute walk test (6MWT) were feasible in 10 of 12 patients
- The mean difference in 6MWT between Mepsevii and placebo were:
  - -11 meters at 8 weeks
  - 13 meters at 16 weeks
  - 18 meters at 24 weeks

Clinical Studies

- **Study 201** was a single-arm, open-label, dose exploration trial (N=3)
- After 120 weeks of exposure to Mepsevii, 1 patient showed a 21% improvement over baseline in forced vital capacity on pulmonary function testing as well as a 105 meters improvement in the 6MWT

Clinical Studies

- The remaining patients with hepatosplenomegaly showed a reduction in liver volume and spleen volume after 36 weeks of treatment
- For more clinical trial data, see full labeling

New Product Monograph

- For more information view the product monograph available at:

  http://www.empr.com/mepsevii/drug/34770/