

Mepsevii® (vestronidase alfa-vjbk)

When requesting Mepsevii® (vestronidase alfa-vjbk), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved Indication

Mepsevii is indicated for the treatment of individuals with mucopolysaccharidosis type VII (MPS VII; Sly syndrome).

Coverage Guidelines

Mucopolysaccharidosis Type VII (MPS VII; Sly syndrome)

The individual must meet all of the following criteria for approval:

- Diagnosis is confirmed by one of the following:
 - Laboratory test demonstrating deficient beta-glucuronidase activity in leukocytes, fibroblasts, or serum; OR
 - Molecular genetic test demonstrating biallelic pathogenic or likely pathogenic beta-glucuronidase gene variants;
- Mepsevii is prescribed by or in consultation with a geneticist, an endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Approval duration: 12 months

Dosing Recommendation

The recommended dosage of Mepsevii is 4 mg/kg administered by intravenous infusion every two weeks.

References

1. Mepsevii injection [prescribing information]. Novato, CA: Ultragenyx Pharmaceutical; December 2020.
2. Montano AM, Lock-Hock N, Steiner RD, et al. Clinical course of sly syndrome (mucopolysaccharidosis type VII). *J Med Genet.* 2016;53:403-418.
3. Tomatsu S, Montano AM, Dung VC, et al. Mutations and polymorphisms in GUSB gene in mucopolysaccharidosis VII (Sly syndrome). *Hum Mutat.* 2009;30:511-519.