Overview
Vestronidase alfa is a recombinant human beta-glucuronidase (GUS), which provides exogenous 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 glycosaminoglycans (GAGs) in affected tissues.

Coverage Guidelines
Authorization may be granted for members who are currently receiving treatment with Mepsevii, excluding when the product is obtained as samples or via manufacturer’s patient assistance programs. OR
Authorization may be granted if the member meets all following criteria and documentation has been submitted:
1. The member is diagnosed with mucopolysaccharidosis VII (MPS VII, Sly syndrome)
2. An assay of enzyme activity results from genetic testing showing mutation in the beta glucuronidase gene is submitted
3. The member’s current weight is provided.

Limitations
1. Authorization will be granted for 6 months

References
1. Mepsevii (vestronidase Alfa-vjbk) [prescribing information]. Novato, CA: Ultragenyx Pharmaceutical Inc; November 2017

3. First FDA approved treatment for pediatric and adult patients with MPS VII. https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm585308.htm

Review History
09/18/19 – Reviewed
07/22/20 – Reviewed July P&T Mtg; no clinical updates
09/16/20 – Reviewed at P&T

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