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1. Clinical Pharmacology Compendia. Tampa FL: Gold Standard, Inc. 2020. Vestronidase alfa.
2. Mepsevii™ (vestronidase alfa-vjvk) for intravenous infusion [package insert]. Ultragenyx Pharmaceutical, Inc. Novato, CA. Revised 12/2019.
3. Montano A, Lock-Hock N, Steiner R, et al. Clinical course of sly syndrome (Mucopolysaccharidosis Type VII). *J Med Genet*. 2016;53(6):403-418.
4. Fox J, Volpe L, Bullaro J, et al. Treatment with investigational rhGUS enzyme replacement therapy in an advance stage MPS VII patient. *Mol Genet Metab*. 2015;114(2):203-208.
5. Qi Y, McKeever K, Taylor J, et al. Pharmacokinetic and pharmacodynamic modeling to optimize the dose of vestronidase alfa, an enzyme replacement therapy for treatment of patients with mucopolysaccharidosis type VII: results from three trials. *Clin Pharmacokinet* 2019;58(5):673-83.
6. Harmatz P, Whitley CB, Wang RY, et al. A novel blind start study design to investigate vestronidase alfa for mucopolysaccharidosis VII, an ultra-rare genetic disease. *Mol Genet Metab*. 2018;123(4):488–94.
7. MCG™ Care Guidelines, 22nd edition, 2018, Home Infusion Therapy, CMT: CMT-0009(SR).
8. Polinski JM, Kowal MK, Gagnon M, et al. Home infusion: safe clinically effective, patient preferred, and cost saving. *Healthcare*. 2016.
9. Morrison A, Oussoren E, Friedel T, et al. Pathway to diagnosis and burden of illness in mucopolysaccharidosis type VII—a european caregiver survey. *Orphanet J Rare Dis*. 2019;14 (1):254
10. McCafferty EH, Scott LJ. Vestronidase alfa: a review in mucopolysaccharidosis VII. *BioDrugs*. 2019;11;33(2):233-240.