

11. Clinicaltrials.gov. An open-label phase 1/2 study to assess the safety, efficacy, and dose of UX003 rhGUS enzyme replacement therapy in patients with mucopolysaccharidosis type 7 (MPS 7) (NCT01856218). Available at: <https://clinicaltrials.gov/ct2/show/NCT01856218>. Accessed on June 29, 2020.
12. Montañó AM, Lock-hock N, Steiner RD, et al. Clinical course of sly syndrome (mucopolysaccharidosis type VII). J Med Genet. 2016; 53 (6): 403-18.
13. National Organization for Rare Disorders. Mucopolysaccharidosis Type VII. Available at: <https://rarediseases.org/rare-diseases/sly-syndrome>. Accessed on November 21, 2017.
14. Wang RW, Bodamer OA, Watson MS, et al. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. Genetic Med. 2011 May; 13 (3): 457 – 484.
15. Scarpa M, Almásy Z, Beck M, et al. Mucopolysaccharidosis type II: European recommendations for the diagnosis and multidisciplinary management of a rare disease. Orphanet J of Rare Dis. 2011; 6: 72 – 90.
16. National Organization for Rare Disorders. Mucopolysaccharidosis IV. 2019. Available at: <https://rarediseases.org/rare-diseases/morquio-syndrome/>. Accessed on June 29, 2020.
17. Tomatsu S, Yasuda E, Patel P, et al. Morquio a syndrome: diagnosis and current and future therapies. Pediatr Endocrinol Rev. 2014 Sep; 12 (1): 141 – 51.
18. National Organization for Rare Disorders. Mucopolysaccharidosis Type VII. 2017. Available at: <https://rarediseases.org/rare-diseases/sly-syndrome/>. Accessed on June 29, 2020.

| Policy/UM Medical Management System Update History | | |
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| # | Date | Change Description |
| 1. | Effective Date: 10/0/202 | Annual review – no changes to the criteria at this time |
| 1. | Effective Date: 10/07/2021 | Annual review – no changes to the criteria at this time |
| 1. | Effective Date: 10/08/2020 | New policy created for this disease state and class of drugs. The Enzyme Replacement Therapy policy will be retired |
| 1. | Effective Date: | 0HGLFDOSROLFNHVVWDEOLVKG |

*The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or <http://dailymed.nlm.nih.gov/dailymed/index.cfm>.