

MPS IVA Selected Publications

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Disease and Diagnosis	Harmatz P, Mengel KE, Giugliani R, et al. Longitudinal analysis of endurance and respiratory function from a natural history study of Morquio A syndrome. <i>Mol Genet Metab.</i> 2015;114(2):186-194. DOI: 10.1016/j.ymgme.2014.10.015
	Harmatz P, Mengel KE, Giugliani R, et al. The Morquio A Clinical Assessment Program: baseline results illustrating progressive, multisystemic clinical impairments in Morquio A subjects. <i>Mol Genet Metab.</i> 2013;109(1):54-61. DOI: 10.1016/j.ymgme.2013.01.021
	Wood TC, Harvey K, Beck M, et al. Diagnosing mucopolysaccharidosis IVA. <i>J Inherit Metab Dis.</i> 2013;36(2):293-307. DOI: 10.1007/s10545-013-9587-1
Elosulfase alfa	Burton BK, Stepien KM, Campeau PM, et al. Real-world treatment with elosulfase alfa in patients with MPS IVA is associated with improved endurance over time. <i>Genet Med Open.</i> 2025;3:103428. DOI: 10.1016/j.gimo.2025.103428
	Mitchell JJ, Burton BK, Bober MB, et al. Findings from the Morquio A Registry Study (MARS) after 6 years: long-term outcomes of MPS IVA patients treated with elosulfase alfa. <i>Mol Genet Metab.</i> 2022;137:164-172. DOI: 10.1016/j.ymgme.2022.08.007
	Cleary M, Davison J, Gould R, et al. Impact of long-term elosulfase alfa treatment on clinical and patient-reported outcomes in patients with mucopolysaccharidosis type IVA: results from a Managed Access Agreement in England. <i>Orphanet J Rare Dis.</i> 2021;16:38. DOI: 10.1186/s13023-021-01675-x
	Hendriksz CJ, Parini R, AlSayed MD, et al. Long-term endurance and safety of elosulfase alfa enzyme replacement therapy in patients with Morquio A syndrome. <i>Mol Genet Metab.</i> 2016;119(1-2):131-143. DOI: 10.1016/j.ymgme.2016.05.018
	Hendriksz CJ, Giugliani R, Harmatz P, et al. Multi-domain impact of elosulfase alfa in Morquio A syndrome in the pivotal phase III trial. <i>Mol Genet Metab.</i> 2015;114(2):178-185. DOI: 10.1016/j.ymgme.2014.08.012
	Hendriksz CJ, Burton B, Fleming TR, et al. Efficacy and safety of enzyme replacement therapy with BMN 110 (elosulfase alfa) for Morquio A syndrome (mucopolysaccharidosis IVA): A phase 3 randomised placebo-controlled study. <i>J Inherit Metab Dis.</i> 2014;37(6):979-990. DOI: 10.1007/s10545-014-9715-6
Management Guidelines	Akyol MU, Alden TD, Amartino H, et al. Recommendations for the management of MPS IVA: Systematic evidence- and consensus-based guidance. <i>Orphanet J Rare Dis.</i> 2019;14(1):137. DOI: 10.1186/s13023-019-1074-9

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