Morquio syndrome treatment

The treatment for Morquio syndrome consists of prenatal identification and of enzyme replacement therapy. On 12 February 2014, the US Food and Drug Administration approved the drug elosulfase alfa (Vimizim) for treating Type A. Currently, there is no treatment for Type B.

Twenty six international healthcare professionals across multiple disciplines, with expertise in managing MPS IVA, and three patient advocates formed the Steering Committee (SC) and contributed to the development of this guidance. Representatives from six Patient Advocacy Groups (PAGs) were interviewed to gain insights on patient perspectives. A modified-Delphi methodology was used to demonstrate consensus among a wider group of healthcare professionals with experience managing patients with MPS IVA and the manuscript was evaluated against the validated Appraisal of Guidelines for Research and Evaluation (AGREE II) instrument by three independent reviewers.

RESULTS: A total of 87 guidance statements were developed covering five domains: (1) general management principles; (2) recommended routine monitoring and assessments; (3) disease-modifying interventions (enzyme replacement therapy [ERT] and haematopoietic stem cell transplantation [HSCT]); (4) interventions to support respiratory and sleep disorders; (5) anaesthetics and surgical interventions (including spinal, limb, ophthalmic, cardio-thoracic and ear-nose-throat [ENT] surgeries). Consensus was reached on all statements after two rounds of voting. The overall guideline AGREE II assessment score obtained for the development of the guidance was 5.3/7 (where 1 represents the lowest quality and 7 represents the highest quality of guidance).

CONCLUSION: This manuscript provides evidence- and consensus-based recommendations for the management of patients with MPS IVA and is for use by healthcare professionals that manage the holistic care of patients with the intention to improve clinical- and patient-reported outcomes and enhance patient quality of life. It is recognised that the guidance provided represents a point in time and further research is required to address current knowledge and evidence gaps [1].

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Akyol MU, Alden TD, Amartino H, Ashworth J, Belani K, Berger KI, Borgo A, Braunlin E, Eto Y, Gold JI, Jester A, Jones SA, Karsli C, Mackenzie W, Marinho DR, McFadyen A, McGill J, Mitchell JJ, Muenzer J, Okuyama T, Orchard PJ, Stevens B, Thomas S, Walker R, Wynn R, Giugliani R, Harmatz P, Hendriksz C, Scarpa M; MPS Consensus Programme Steering Committee; MPS Consensus Programme Co-Chairs. Recommendations for the management of MPS IVA: systematic evidence- and consensus-based guidance. Orphanet J Rare Dis. 2019 Jun 13;14(1):137. doi: 10.1186/s13023-019-1074-9. PubMed PMID: 31196221; PubMed Central PMCID: PMC6567385.