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Long-term outcomes of elosulfase alfa enzyme replacement therapy in adults with MPS IVA: a sub-analysis of the Morquio A Registry Study (MARS)

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Abstract

Background Mucopolysaccharidosis (MPS) IVA is a rare disease with substantial, multisystemic morbidity. We assessed real-world safety and effectiveness of the enzyme replacement therapy (ERT) elosulfase alfa in patients with MPS IVA in the multinational, observational Morquio A Registry Study (MARS) who initiated ERT in adulthood (aged ≥ 18 years).

Methods Patients were enrolled between September 2014 and February 2022; urinary keratan sulfate (uKS), 6-minute walk test (6MWT) distance, forced expiratory volume in 1 s (FEV₁), forced vital capacity (FVC), EuroQoL-5D-5L (EQ-5D-5L) score, and safety were assessed during routine care.

Results As of February 13, 2022, 90 patients who initiated ERT had enrolled (median exposure: 5.6 years; median age at first ERT: 27.8 years). Reductions from baseline in uKS levels were sustained over mean follow-up of 5.4 years (mean percent change: -52.9%; $p < 0.0001$). In patients with available data, mean change in 6MWT distance was +15.8 m ($p = 0.3627$) over a mean follow-up of 5.8 years. FEV₁ and FVC remained stable over mean follow-up of 5.3 years (mean change: 0.0 L for both). The mean change from baseline in EQ-5D-5L index score was +0.1 after 1 year of treatment. Thirty-four patients (39.5%) had ≥ 1 adverse event (AE), 23 patients (26.7%) had ≥ 1 serious AE, and 10 (11.6%) had ≥ 1 drug-related AE (infusion-related reactions [$n = 3$; 3.5%], pyrexia [$n = 2$; 2.3%]). Eight deaths occurred; none were deemed treatment related.

Conclusions Real-world data collected from MARS suggest that patients with MPS IVA who initiated ERT in adulthood remained stable over 7 years of follow-up. No new safety signals were identified.

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