Dantrolene as a possible prophylactic treatment for RYR1-related rhabdomyolysis

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Background and aims: Mutations in RYR1 lead to various neuromuscular phenotypes including malignant hyperthermia susceptibility and Rhabdomyolysis (RM). We report the use of oral dantrolene as a prophylactic treatment for RYR1-related RM.

Methods: Case report.

Results: Three patients were prescribed 25mg of dantrolene to take orally up to a maximum of four times a day at the onset of symptoms in an attempt to stop progression of the RM episodes. All patients reported benefits with no drug-related side effect.

Conclusion: In the short term use of low dose dantrolene-associated benefits appear to outweigh risks in the management of patients with recurrent rhabdomyolysis due to mutations in RYR1, in particular ad hoc use during symptoms might possibly prevent or abort an attack of RM. Undertaking a randomised, double-blinded, placebo controlled clinical trial to assess risks and benefits of dantrolene in this group of patients could help to evaluate the role this drug in preventing RM due to RYR1 mutations in the future.