

Category: 7. Other Muscle Disease

Dantrolene as a possible prophylactic treatment for *RYR1*-related rhabdomyolysis

Renata Siciliani Scalco^{1,2}, Nicol C. Voermans³, Heinz Jungbluth^{4,5,6}, Ros Quinlivan^{1,7}

- 1) *MRC Centre for Neuromuscular Diseases and Department of Molecular Neuroscience, University College London (UCL) Institute of Neurology and National Hospital for Neurology and Neurosurgery, London, UK*
- 2) *CAPES Foundation, Ministry of Education of Brazil, Brasilia, DF, Brazil*
- 3) *Department of Neurology, Radboud University Medical Center, Nijmegen, The Netherlands*
- 4) *Department of Basic and Clinical Neuroscience, Institute of Psychiatry, Psychology and Neuroscience (IoPPN), King's College London (KCL), London, UK*
- 5) *Randall Division for Cell and Molecular Biophysics, Muscle Signalling Section, King's College London, London, UK*
- 6) *Department of Paediatric Neurology, Evelina Children's Hospital, Guy's & St Thomas NHS Foundation Trust, London, UK*
- 7) *Dubowitz Neuromuscular Centre, Great Ormond Street Hospital, London, UK*

r.scalco@ucl.ac.uk

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.