Building rigour in exploratory rodent studies of neuromuscular disease

M.D. Grounds, School of Human Sciences, The University of Western Australia, Nedlands, WA 6009, Australia.

Creative exploratory rodent studies of neuromuscular diseases can provide the basis for ultimate translation to human clinical trials. However, the final pre-clinical data need to be very strong and reproducible to justify an expensive clinical trial. Unfortunately, too many clinical trials have failed recently, due in large part to inadequate pre-clinical data. This is unacceptable and an increasing concern with pressure to conduct clinical trials from commercial companies, patient advocacy groups and also the business model that demands 'outcomes' from biomedical research. To address this problem, standard operating procedures for pre-clinical research have been developed over the last 20 years for many diseases, to increase rigour and enable easy critical comparison of global rodent data: importantly, use of these recommended procedures is now being enforced by more journals and funding agencies (Grounds *et al.*, 2008; Willmann *et al.*, 2018). These international developments aim to greatly improve the quality and reproducibility of pre-clinical data, especially for potential clinical translation.

Grounds MD, Radley HG, Lynch GS, Nagaraju K, De Luca A. (2008) Towards developing standard operating procedures for pre-clinical research using the mdx mouse model of Duchenne Muscular Dystrophy. *Neurobiology of Disease* **31**: 1-19.

Willmann R, Buccella F, De Luca A, Grounds MD, 227th ENMC workshop study group. (2018) 227th ENMC international workshop: Finalizing a plan to guarantee quality in translational research for neuromuscular diseases. *Neuromuscular Disorders* **28**: 185-192.