Open Research Awards Application (**Stefanetti et al. 2022)**

Measuring the Effects of Exercise in Neuromuscular Disorders: Synthesizing A Systematic Review and Meta-analyses into an Outcome Measures Database and Computational Research Platform

Introduction:
This was the broadest, most robust analysis, to date, of frequently utilised outcome measures in exercise intervention studies in Neuromuscular Disorders (NMDs). We developed an innovative, interactive, outcome measures database as an open access (OA) online resource. It provides the world's first evidence-based tool for clinicians and researchers supporting exercise prescription in NMD.

Description of the research context in which the open practices were employed:
The benefit and safety of exercise training in NMD has long been a contentious topic. We performed a systematic review and meta-analysis to evaluate the effectiveness and safety of interventional exercise and set out to establish minimal clinically important differences, to transform trial design, delivery, and potential for success. Significant advances have been made in the development of candidate drugs for NMD; however, a lack of validated, standardised outcome measures has hindered progress. A tailored outcome measures database (with inclusion of underpinning raw datasets) did not readily exist. Based on our own research experience, and following consultation with commercial pharmaceutical companies, it was clear there was an important unmet need.

Open practices used and why:
Our OA research outputs included:
- [Wellcome Open Research](#) publication ([Stefanetti et al. 2020](#)), including Open Peer Review for scientific rigor and critique.
- Interactive [database](#) of systematic review data; allowing users to filter via selected domains. The data (exportable via download file) contains additional information (e.g. article weblinks).
- Interactive graph of [meta-analyses](#) data. Raw data underpinning each meta-analysis (and individual study details) are available for download.
- Data deposit in a dedicated FAIR-compliant data repository ([codeocean)](#). Including metadata file ('README') describing the data and outputs, and links to the database. Reproduce capsule allows for coding to be executed and exported.
- Pre-registered protocol via publicly available database (PROSPERO ID: [CRD42018102183](#))

Why:
In our quest to introduce an evidence-based approach to exercise prescription, we sought to maximise data sharing and dissemination from the outset. Moreover, publishing outputs via OA embraces our Wellcome Centre for Mitochondrial Research (WCMR) research culture ethos, which endorses FAIR principles (Appendix A) and aligns to Wellcome [OA policy](#). We hope the outcomes of our OA online resource will revolutionise the field, and be utilised to increase efficiency, reproducibility of results, and ultimately guide future research and clinical practice.

Barriers/challenges encountered:
- Resources/time commitments, across varying areas (to learn new software, adapt code/datasets). _Handled:_ Senior supervisor (Gorman) was supportive and appreciative of time required. WCMR strategy is to embed an open data research ethos. Project was collaborative and involved several disciplines (librarian, statistician, allied health professionals, neurologists). Lead (Stefanetti) attended Newcastle University's Systematic Review Group workshops to upskill.

- Lack of raw data available in publications to facilitate meta-analyses. _Handled:_ We contacted corresponding authors to obtain missing data (provided individually via tailored data forms).
- Perception of greater benefits/accolades from publishing in 'well-regarded' refereed journals vs. OA. _Handled:_ Wide dissemination (including conferences, social media platforms). Additional criteria available in academic promotion criteria (citation number).

Benefits realised and for whom, because of using the open practices:
- Positive impact for the NMD community via accessible tool to 1) inform clinical trial design and delivery; 2) evidence- based clinical exercise prescription and assessment.
- Benefit to WCMR via increased and broadened impact. Documented workflow will streamline future work. Adhering to OA standards and increasing collaborations, sets a positive example and aligns to our WCMR research ethos.
-

Lessons learnt from the experience:
This was a valuable and rewarding project. In addition to the useful technical skills developed (e.g. use of codeocean), key lessons are:
- When setting out for OA, keep accurate data records for transparency.
- Describe the data content and processing requirements thoroughly.
- It _IS_ possible to make all datasets available.
- It takes a collaborative approach.
- It is valuable to have a team to check the data for accuracy.
- Collaborations in similar initiatives should be encouraged and promoted.
- Cross- sector collaboration is extremely valuable and enhances outputs.

Conclusion:
Utilising the most appropriate outcome measures is essential to generate meaningful and usable results to facilitate future research and clinical practice. Health policy and guidelines, including increased specialised support to implement exercise prescription is required to translate these clinically significant evidence-based findings into clinical practice.

OA peer review comment (leading expert in the field): 'Overall this is a huge advancement to the field and will no doubt influence care'.