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EDITORIAL

Editor-in-Chief

Feasibility and pilot studies pave the way for definitive trials

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This edition of *Spinal Cord* has a nice example of a feasibility study [1]. The aim of the study was to determine the feasibility of a definitive trial of intermittent theta-burst stimulation (iTBS) for improving upper limb function following spinal cord injury. Importantly, the outcomes of the study are related to feasibility issues such as success of recruitment, ability to retain participants, completeness of data collection and safety of participants. The authors also use their study to test the effectiveness of their sham iTBS and to attain data to guide future sample size calculations. The study was not designed to determine treatment effectiveness. So even though the authors do provide point estimates (and 95% CI) of the between-group differences, they are rightfully very careful not to imply that their results can be used to infer that iTBS is an effective intervention. This needs to be ascertained in a larger properly powered study.

There are other examples of feasibility studies in *Spinal Cord*, although sometimes they are referred to as pilot studies. For example, a recent study looked at web-based physiotherapy to determine whether it would be feasible to provide physiotherapy in this way and whether patients would accept it and comply with their exercise programs [2]. Another study examined the safety, tolerability and feasibility of transplanting autologous bone marrow cells in people with recent spinal cord injury [3]. Both studies were conducted with the aim of doing preparation work before embarking on the definitive trial.

Some distinguish between feasibility and pilot studies [4], arguing that pilot studies should be conducted in exactly the same way as a definitive trial including randomisation and full adherence to methodology important for minimising bias but without analyses to determine between-group differences (or test hypotheses); instead, data are presented descriptively [5]. They argue that the purpose of the pilot study is to determine whether all the components of the definitive study can work together [5]. In contrast, a feasibility study may not have randomisation or even a control group, and the primary interest is to answer the question—'can this trial be done?' [4, 5] Others have perhaps added further confusion (or clarity depending on your perspective) by distinguishing between pilot work (any background work required before starting a trial), a pilot study (a study with objectives and methodology but not necessary randomisation) and a pilot trial (a study with all features of a definitive trial including randomisation) [6]. Most admit that the terminology in this area is awash with confusion [7].

Perhaps the best and most widely accepted definition is provided by the National Institute for Health Research Evaluation, Trials and Studies Coordinating Centre [8]. They do not try to distinguish between pilot or feasibility studies but instead state:

> *"Pilot studies ….also commonly known as "feasibility" or "vanguard" studies, … are designed to assess the safety of treatment or interventions; to assess recruitment potential; to assess the feasibility of international collaboration or coordination for multicentre trials; to increase clinical experience with the study medication or intervention for the phase III trials."*

Regardless of terminology, it remains apparent that there are many legitimate and useful types of studies that can be performed in preparation for a definitive trial. However, the outcomes need to be pre-specified and clearly articulated with a priori definitions of success [9]. Thabane et al. (page 5) [8] provides some nice examples of the same from the literature. For instance, a trial may be deemed a success if:

- 98.5% of participants receive the study drug within 12 h of randomization;
- One participant is recruited per centre per week (i.e., 200 participants from four centres over 50 weeks);

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- At least 70% of all eligible patients can be recruited;
- No more than 5% of all recruited participants cross over from one group to the other;
- Outcome data are attained in at least 95% of all included participants.

Thabane et al. (page 5) [8] also provide a clear way of thinking about the purposes of pilot or feasibility studies. They argue that to complete a definitive trial, researchers need to give consideration to the required processes, resources and management, as well as the obvious scientific issues. All of these can be looked at in pilot or feasibility studies.

Most are adamant that pilot and feasibility studies should not be used to estimate treatment effects, and between-group differences should not be presented. Although some argue that there may be scope to look at a possible range of outcomes if done with care (and according to a pre-specified statistical plan) [10]. It should come as no surprise that there are now CONSORT guidelines for the reporting of pilot and feasibility trials [11].

Spinal Cord welcomes pilot and feasibility studies but it encourages authors to clearly articulate the purpose of their studies and to ensure that their studies address important questions about the feasibility of conducting definitive trials.

Announcement

Spinal Cord is pleased to announce the winners of the 2017 Readers' Choice Awards.

1. Winner of the 2017 Readers' Choice for best review paper published in 2017 is: M Sharif-Alhoseini, M Khormali, M Rezaei, M Safdarian, A Hajighadery, MM Khalatbari, M Safdarian, S Meknatkhah, M Rezvan, M Chalangari, P Derakhshan, and V Rahimi-Movaghar for Animal models of spinal cord injury: a systematic review (doi: sc.2016.187;55:8).
2. Winner of the 2017 Readers' Choice for best original research paper published in 2017 is: D Zbogar, JJ Eng, WC Miller, AV Krassioukov, and MC Verrier for Movement repetitions in physical and occupational therapy during spinal cord injury rehabilitation (doi: sc.2016.129; 55:2).

These awards are determined by the number of downloads in the six months following publication. This excludes any papers authored by the Editor-in-Chief or Associate Editors.

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