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Editorial

The importance of pilot studies, how to write them and what they mean

For most clinicians, knowing about the effectiveness of their treatment is down to the experience that patients report after treatment and over a period of planned consultations. Osteopaths also rely on clinical examinations and their palpation to give them additional information about the progress or not that they are making with an individual. More recently in the mainstream medical literature, patient reported outcome measures (PROMS) are being promoted as an additional way of evaluating services [1] and significant work has been developed in the UK by the National Council for Osteopathic Research to enable osteopaths to use PROMS as part of routine practice (see http://www.ncor.org.uk/practitioners/patient-reported-outcomes/). Using PROMS is a useful way to evaluate clinical work and offers opportunities to enhance practice. However, there are a number of different more research orientated designs available to help us to more formally study the outcomes of treatment.

However, the research capacity within the osteopathic community is small. There are few individual osteopaths or osteopathic centres worldwide with the knowledge, capability and resources to secure funding and deliver high quality large scale studies. This is particularly the case for resource intensive studies that investigate the effectiveness or efficacy of osteopathic interventions. Whilst there is debate about the hierarchy of designs used to evaluate the outcomes of care [2], randomised controlled trials (RCT) are the dominant research design used to answer such questions. Designing and delivering a randomised clinical trial is not an easy task. Those who have completed and published trials deserve the osteopathic community's appreciation. Not only are there high levels of regulatory and governance issues to be overcome [3], but also, there are a range of design options to be considered with different strengths and weaknesses. RCTs were initially designed to assess pharmacological interventions and it has become apparent that complex interventions, including osteopathy, are not often suitable for being evaluated using traditional pharmacological trial designs [4] and additional guidance has been published to support the effective reporting of RCTs of complex interventions [5]. Therefore researchers investigating the effectiveness of osteopathy (as opposed to individual technique application) have tended to adopt pragmatic approaches to design their trials. Pragmatic trials address the question as to whether or not an intervention works when in normal practice and is usually compared with another available treatment rather than a sham or a placebo. Pragmatic trials do not reduce or strongly control all the variables associated with the intervention and lead to more applied or naturalistic designs. The benefit of this approach is that it more closely models real practice and tends to have more applicability in the real world. Whereas highly controlled explanatory trials evaluate efficacy and strictly control the intervention and whilst may have higher internal validity, have less applicability in the real world for treatments like osteopathy [6]. Interpreting the results of trials is also a challenge for researchers and clinicians. Potential biases may affect the design and indeed our interpretation of results in individual studies [7–10]. Trials of spinal manipulative therapy for low back pain have been reviewed for methodological quality. Recommendations included the use of mandatory reporting guidance and registration of trials as well as the avoidance of underpowered trials where there is an increased risk of type II errors. Small trials and single centred trials are associated with the reporting of larger treatment effects. Studies should include the use of effective sample size calculations [11].

The International Journal of Osteopathic Medicine (IJOM) has implemented the mandatory use of reporting guidelines and trial registration. This was announced in an editorial in IJOM as a reprint of a consensus statement from rehabilitation journal editors including IJOM [12]. However, many of the trials IJOM receives as submissions for publication are of a small scale and are often described as pilot studies. We thought that it would be helpful to provide some discussion of the issues associated with reporting pilot studies for authors and indeed identify some potential areas that readers may like to consider when interpreting the results of such studies.

Pilot study, feasibility study, small sample size study, pilot randomised controlled trial… these names are often used interchangeably. Whilst they may share some common aspects, they have specific definitions, aims and are associated with specific approaches to analysis. The overarching term for these studies is *feasibility studies* and they are conducted when there is uncertainty about future RCT feasibility. They help to design a further confirmatory study [13]. The Medical Research Council (MRC)'s recommendations for the development and evaluation of complex interventions include testing RCT designs with pilot studies to test procedures for their acceptability, to estimate recruitment and retention rates, and to determine sample sizes required in main trials [4]. One of the key aspects of feasibility studies is that they do not evaluate effectiveness; this is left to the main study [14]. It is nevertheless a very common temptation and pitfall for researchers to use small sample studies and run some inferential testing and reach conclusions about effectiveness. Instead, the analyses should be mainly descriptive and focus on confidence interval estimations and not on inferential testing [15–18].

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Feasibility studies are divided into three subgroups: randomised pilot studies, non-randomised pilot studies (including qualitative studies) and feasibility studies that are not pilot studies for evaluating specific aspects of a future RCT [19,20]. Historically, feasibility studies were mainly conducted to generate initial data to perform sample size calculation for a larger trial [16], but recently this has been discouraged as feasibility study sample sizes are small and therefore offer imprecise between-treatment group effect size estimates [13,17]. Feasibility studies' effect sizes can therefore produce inaccurate estimates of the true effect, resulting in an incorrect estimate of the sample size needed for the main trial [21]. If the true effect size was known with enough confidence before conducting the main trial, conducting the main trial would be clinically unethical. Sample size estimates for a main trial should instead be based on a clinically meaningful effect [17]. This can be challenging when there is no consensus on what constitutes a clinically meaningful change in the outcomes used. Lancaster et al. (2004) defined the objectives of conducting a feasibility study: to test the study protocol, the data collection, the randomisation procedure, the recruitment and consent procedures, the acceptability of the intervention and the feasibility of using selected outcome measures [16]. Not effectiveness. Feasibility studies are not powered to assess effectiveness.

Feasibility studies are extremely useful and necessary, as conducting an RCT with no prior feasibility study, has a high risk of compromising the results due to unplanned difficulties with for example, the RCT design, recruitment strategies or the acceptability of the intervention.

In summary for our readers, be careful about how you interpret small scale pilot RCTs with big scale claims about whether treatment works or does not work. These studies are getting published, and disseminated via social media with very little information about the study itself, but usually with strong claims about positive effects of osteopathy. Whilst we welcome studies into the effectiveness of osteopathy, we urge caution in interpreting claims until studies have been repeated or performed on a non-pilot basis. At IJOM, we are working hard at supporting authors to make the most of reporting their work effectively and are grateful to our dedicated reviewers for their support in this. Nevertheless, readers will need to make judgements for themselves as to how they interpret the claims made by authors and the extent to which studies have meaning within the context of a readers own practice.

For our authors, we recognise the enormous efforts of some of our researchers, faced with developing and writing up RCTs often with an absence of resources and recourse to expertise. However, we all have a duty to take care with how we interpret and report our research. It is better to lay strong foundations for high quality studies than to muddy the water about the claims we can make about effectiveness where there are strong risks of bias and insufficient evidence to support premature claims.

When designing a small pilot study, draw on available information as cited in this editorial to design reasonable studies with “deliverable” aims. When reporting feasibility studies look to published guidance to enhance the quality of your manuscripts [22].

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