Reviewer's report

Title: An Audit of Sample Sizes for Pilot Studies being Undertaken in the United Kingdom

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Reviewer: Charity G Moore

Reviewer's report:

This paper sought to assess the target sample sizes of pilot and feasibility studies currently running in within the United Kingdom. The significance of such a report is not stated and the recommendations from the analyses are not supported by their data.

Major concerns:

1. The conclusion of the article is completely not supported by the data provided. The data provided are purely a descriptive look at the range of sample sizes of ongoing pilot trials. There was no critical review of the sample sizes to see if they were appropriate for the aims of the proposed trial. Just because pilot trials are being conducted with sample sizes of 30 does not mean that is the best sample size for all pilot trials. How is one to know these trials have enough or too few participants for their goals?

2. Not clear what this analysis contributes to the literature. Would have been better to critically review the trials to see if the sample size aligned with their objectives.

3. Not clear why the authors looked for differences by funding status and by primary endpoint. No research questions or hypotheses were framed around these variables.

4. Seems important to know if the primary endpoints were efficacy endpoints or feasibility outcomes. The paper could be made richer with a description of the types of endpoints beyond normal or binary. Same applies to the description of the interventions, particularly the health technology interventions.

5. The authors acknowledge the skewness of the sample size data but still present the means. Only medians should be presented.

6. Conclusion: This paper does not assist researchers in sample size justifications for pilot investigations. Just because trials are practicing 30 patients per trial does not mean 30 patients per trial provides these pilot trials with the answers (preliminary data) they are seeking.

Minor concerns:

7. The title of the manuscript should state “Pilot Trials” instead of “Pilot Studies” which would imply any type of study design, including purely observational.

8. Multiple grammatical and syntax problems throughout the manuscript. Many
contractions used throughout article such as “won’t” – not ideal for a scientific manuscript. Possessives used incorrectly (funders’ website vs funders’ websites). The word ‘data’ is plural.

9. The abstract is written with abbreviations/acronyms such as UKCRN that will not be known to the reader. These should be removed and substituted with more general language (ex. Trial database)

10. The 7th paragraph of the background begins with a sentence that is not clear. In the same paragraph the citations listed do not have a citation.

11. More description needed on UKCRN. Who must register, how to access, who can access.

12. Only trials that were randomized were included. Data components included presence or absence of a control. If no control was in the study, what were the participants randomized to (two interventions)?

13. What information was sought from the internet search and clinicaltrials.gov

14. What is meant by ‘very limited information’ for 62 trials – did the PI and funders respond?

15. Results: ‘no data available’ in results means the sample size was not available?

16. Table 1: MSK not defined

17. 3.2 sample size: author states “all trials found…..provided a target sample size” but this conflicts with statement that some of the trials had missing data.

18. Most important part of this paper is that none of the trials explained justification for their target sampled.

19. Tables should be condensed to one table. Each table has so little information. Sample sizes (number of studies) are needed for each row of the tables.

20. No definition of public or industry.

21. Declarative statements are made about differences or lack of them without any statistical testing.

22. No justification for fully stratifying results by funder and endpoint.

23. Discussion: statement that target sample size for pilot study is often likely to be adapted during the study or main study is not cited? From where do the authors draw their conclusion. Same paragraph makes statement about what information participants should receive – this seems completely out of line with the rest of the discussion.

24. Why is it significant that funding source has been found to drive sample size in a previous study?

25. Strong statement made about “30% less patients required for a study with a continuous outcome” -- what is meant by required? How do you know these studies are the “right” size?

26. This statement is completely unsupported by the analyses “It is also apparent
that when preliminary studies are published, it appears to be only those which have found ‘significant’ results.”

**Level of interest:** An article of insufficient interest to warrant publication in a scientific/medical journal

**Quality of written English:** Not suitable for publication unless extensively edited

**Statistical review:** Yes, and I have assessed the statistics in my report.

**Declaration of competing interests:**

I declare I have no competing interests.