Reviewer's report

Title: Sample size requirements to estimate key design parameters from external pilot randomised controlled trials: a simulation study.

Version: 1 Date: 18 February 2014

Reviewer: Chris Sutton

Reviewer's report:

Major Compulsory Revisions

None.

Minor Essential Revisions

1. Abstract: I feel that the sentence “Hence if the primary outcome is binary a total of at least 120 subjects (60 in each group) may be required in the pilot trial.” should be reviewed and revised accordingly. This aspect is not really discussed explicitly in the paper’s conclusion and suggests that a randomised pilot trial with equal numbers may be being recommended without corresponding discussion. This is all, of course, dependent on the feasibility objectives. This could, potentially, be addressed by suitable revision of the preceding sentence by starting this “If the event rate in an intervention group needs ...”.

2. Abstract: Remove ‘a total’ from the second sentence of the Conclusions.

3. Background: Revise the adverb describing ‘underpowered or overpowered’; the use of the term ‘significantly’ could be misinterpreted.

4. Background: The material on the lower part of p.4 and upper part of p.5 needs reorganisation. The quoted material from the NIHR guidance should be separated out (e.g. table or ‘box’) rather than split the paragraph, which currently makes it difficult for the reader to follow. So “Other authors … preclinical trial work [7],” should be followed immediately by “For the purposes of this paper … for the full RCT design.”. Also, the term ICC should be written in full in the NIHR guidance.

5. Background: The sentence that starts ‘Secondly’ (penultimate sentence in this section) should be revised for form a grammatically correct sentence, preferably be rephrasing as a question in a manner consistent with the immediately preceding sentence.

6. Methods: p7. The section on binary outcomes should start with a sentence briefly explaining that the investigation of binary outcomes is based on a single group and state the rationale for this clearly. That this follows the description of a two-group continuous case makes this necessary to change the reader’s mind set.

7. Methods: In the section on ‘Binary Outcomes’ (p.10), it states that there are nine true success probabilities, but the descriptor that follows (0.1(0.1)0.5)
suggests only five. This should be corrected.

8. Methods: The left-hand side of the equation at the foot of p.9 should not be indexed by i. Also, the description of this equation should be revised to read “Second, this process was repeated for Ns (number of simulations to estimate the true success probability to within 0.1% of its se) and the average observed success probability for each of the nine true success probabilities …”.

9. Methods: The superfluous (repeated) text (Finally, the relative percentage gain in precision around the true binomial proportion per increase in 5 study participants for a fixed true success probability is defined as) on p.10 should be removed.

10. Figures: Figures 7 and 8 each need a legend to identify the individual plots within the figures.

11. References: Reference 6. has its year of publication missing and References 11 and 12. have capitalised article titles.

Discretionary Revisions
None

Level of interest: An article of outstanding merit and interest in its field

Quality of written English: Acceptable

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

Declaration of competing interests:

I declare that I have no competing interests.