A new CONSORT extension should improve the reporting of randomized pilot and feasibility trials

Peter Craig

MRC/CSO Social and Public Health Sciences Unit
University of Glasgow
Glasgow
G12 0XS
Scotland
UK

Peter.craig@glasgow.ac.uk
+44 141 353 7559

Peter Craig receives core-funding from the UK Medical Research Council (funding code: MC_UU_12017/15) and the Scottish Government Chief Scientist Office (funding code: SPHSU15). He was a member of the Feasibility and Pilot Studies Development Group that contributed to the development of the CONSORT extension. Preparation of the commentary did not receive any specific grant from funding agencies in the public, commercial, or not-for-profit sectors.

Key words

Pilot; feasibility; randomized; trials; CONSORT; reporting

What is new?

- An extension to the CONSORT statement to guide the reporting of randomized pilot and feasibility trials has been published.
- Use of the statement should improve transparency and completeness of reporting.
- Standards of reporting are unlikely to improve unless adoption of guidelines is actively encouraged.
- Funders, journal editors and researchers should collaborate to develop and test methods for encouraging the use of reporting guidelines.

Introduction

Poor reporting is one of the main reasons why much research effort is wasted.[1] Another is inadequate feasibility testing and piloting before an efficacy or effectiveness study is conducted.[2] A new extension to the CONSORT statement seeks to improve the way randomized pilot and feasibility trials are reported.[3][4] Adoption of the extension by journals that publish such trials should encourage its use by researchers, directly improving standards of reporting, and indirectly improving the way pilot and feasibility trials are designed and conducted.

Why was the extension needed?

Thorough feasibility testing and piloting should reduce the number of full scale efficacy or effectiveness trials that fail outright or are delayed due to unforeseen problems with recruitment or retention of participants, delivery of the intervention, and other aspects of study implementation. It may also help to reduce the number of efficacy or effectiveness trials that produce inconclusive findings due to unrealistic expectations about size or variability of effects.

Until recently there was little explicit guidance or support for pilot or feasibility trials. The UK Medical Research Council (MRC) guidance for the development and evaluation of complex interventions emphasised the need for careful development and piloting, and clearly demarcated this work from later phases in the research process, but did not clearly distinguish between pilot and feasibility studies.[5] It may therefore have inadvertently contributed to the terminological confusion that has dogged this area. The CONSORT extension sidesteps these problems by noting that the same reporting requirements apply to any small scale trial that seeks to determine whether and how an efficacy or effectiveness trial should be conducted, and proposes the term ‘pilot trial’ to cover all such studies.

In the last few years recognition of the importance of pilot trials has grown. A number of reviews of existing practice have been published,[2][6][7] and some funders have developed guidance for applicants or explicitly set aside funding for such work.[8] Development of the extension has inspired the establishment of a journal, *Pilot and Feasibility Studies*, which publishes pilot trials and seeks to provide ‘a forum for discussion of methodological issues that will lead to increased scientific rigour in this area.’[9]

What does the extension add?

The extension adopts 14 of the 37 CONSORT items unchanged, identifies two as inapplicable to pilot trials, adds three new items, and modifies the remainder. It also incorporates a revised CONSORT statement for abstracts and revised flow diagram for describing how participants progress through the trial. Many of the changes to the CONSORT items aim to identify pilot trials clearly, and distinguish them from efficacy or effectiveness studies. For example, item 1a of the extension reads 'Identification as a *pilot or feasibility* randomized trial in the title' (additions in italics). These changes are more than nominal: pilot trials have distinct aims, which are reflected in the way the data should be analysed and interpreted.

The distinction between the aims of a pilot and an efficacy or effectiveness trial is reflected in changed items 17(a) and 18, and excluded items 12b and 17b (Table 1). In presenting the results of a pilot trial, the aim of including measures of uncertainty (item 17a) is to highlight the imprecision of estimates from a small sample, rather than to support interpretation of effect sizes. The distinction between absolute and relative effects (item 17b) reflects their differing clinical or policy meanings, so is not relevant to the interpretation of results from a pilot trial. Estimates from a pilot trial should only be used as inputs to the design of a future effectiveness study, and even then should only be used alongside other information, such as previous trial results and judgements about the minimum clinically meaningful effect.[10] Likewise, the modification to item 18 reflects the exploratory nature of a feasibility trial, which makes the distinction between exploratory analyses and prespecified or subgroup analyses less important. Item 12b is deemed inapplicable for similar reasons.

Table 1 Revised CONSORT items relating to the analysis of pilot trials

Outcomes and estimation:	CONSORT	Extension for pilot trials
12b	Methods for additional analyses, such as subgroup analyses and adjusted analyses	Not applicable
17a	For each primary and secondary outcome, results for each group, and the estimated effect size and its precision (such as 95% confidence interval)	For each prespecified objective, results including expressions of uncertainty (such as 95% confidence interval) for any estimates. If relevant, these results should be by randomized group
17b	For binary outcomes, presentation of both absolute and relative effect sizes is recommended	Not applicable
Ancillary analyses:		

18	Results of any other analyses performed, including subgroup analyses and adjusted analyses, distinguishing prespecified from exploratory	Results of any other analyses performed that could be used to inform the future definitive trial

The three new items (4c, 19a, and 26) in the extension are in principle applicable both to efficacy or effectiveness trials and to pilot trials. Item 4c, 'how participants were identified and consented,' is particularly salient to pilot trials given their role in testing recruitment procedures. Likewise, item 19a, 'If relevant, other important unintended consequences,' could apply to any trial, but is crucial to the role of pilot trials in anticipating what may happen in a future larger scale trial. Item 26, 'Ethical approval/research review committee approval confirmed with reference number,' seems equally important to both pilot and efficacy or effectiveness trials.

What effect will the extension have?

CONSORT has been endorsed by over 600 medical journals, and the main CONSORT papers have been cited more than 8000 times. Despite this, its influence is surprisingly weak. The CONSORT website acknowledges that 'Even among endorsing journals the reporting of key methodological items was still dismal.'[11] Only a small minority of CONSORT Items are significantly better reported in endorsing than in non-endorsing journals, and few are reported significantly better after endorsement by a journal than they were before endorsement. These kinds of comparisons are obviously subject to confounding and endogeneity, so the actual impact may be still smaller.[12] Part of the reason for the lack of improvement may be that endorsement often does not amount to much. According to the CONSORT website, endorsement 'typically occurs in the form of a supportive statement.' Information on additional procedures used by endorsing journals to enforce compliance is scarce. Ironically, for a guideline designed to support the reporting of randomized trials, there have been very few randomized studies of the impact of procedures for improving compliance with CONSORT, or with other reporting guideline.[13]

Publication of the CONSORT extension for pilot trials is an important step forward. For the extension to have a significant impact, action must also be taken to encourage compliance. The most persuasive evidence of the impact of using the guideline on reporting quality will come from trials of methods for encouraging use of guidance. Journals that publish CONSORT statements and benefit from the citations that such papers attract should be willing to support the conduct of such trials, and to publish the results. Research funders, much of whose investment in trials is squandered by poor reporting,[1] should be willing to help meet the costs. Trial methodologists should be willing to contribute their expertise to the design and running of the trials.

What else is needed to improve the conduct of pilot trials?

As well as directly influencing quality of reporting of pilot trials, the extension may also help to improve standards of design and conduct. It provides a checklist of design elements that researchers should consider in developing proposals for pilot trials. Reports of pilot trials based on the extension will provide exemplars that other researchers can use as a guide. However there are a number of uncertainties about the design of such early stage studies that still need to be resolved. They

include: how to strike the right balance between intervention development and evaluation design; how to decide whether randomisation is needed; how progression criteria should be defined and applied; and what role (if any) pilot trial findings should play in determining the sample size for a future efficacy or effectiveness trial. The UK MRC has recently funded two studies, INDEX, which focuses on the developmental stage, and GUEST, which addresses the pilot and feasibility stage. The studies will seek to identify the consensus among researchers, funders and journal editors about good practice, and to develop and disseminate methodological guidance

Conclusion

If pilot trials came of age with the launch of *Pilot and Feasibility Studies*, the publication of the CONSORT extension is another important step towards maturity. Its impact will depend on whether journals are willing to go beyond passive endorsement towards active promotion of its use by authors and reviewers.

References

[1] Glasziou P, Altman DG, Bossuyt P, Boutron I, Clarke M, Julious S, et al. Reducing waste from incomplete or unusable reports of biomedical research. Lancet 2014;383:267–76

[2] Thabane L, Ma J, Chu R, Cheng J, Rios LP, Robson R, et al. A tutorial on pilot studies: the what, why and how. BMC Med Res Methodol 2010;10:1

[3] Eldridge SM, Chan CL, Campbell MJ, Bond CM, Hopewell S, Thabane L, et al. on behalf of the PAFS consensus group. CONSORT 2010 statement: extension to randomized pilot and feasibility trials. Br Med J 2016;355:i5239 doi: 10.1136/bmj.i5239

[4] Eldridge SM, Chan CL, Campbell MJ, Bond CM, Hopewell S, Thabane L, et al. on behalf of the PAFS consensus group. CONSORT 2010 statement: extension to randomized pilot and feasibility trials. Pilot Feasibility Stud 2016;2:64. doi: 10.1186/s40814-016-0105-8

[5] Craig, P, Dieppe, P, Macintyre S, Michie S, Nazareth I, Petticrew, M. Developing and evaluating complex interventions: the new Medical Research Council guidance. Br Med J 2008;337;a1655

[6] Arain M, Campbell MJ, Cooper CL, Lancaster GA. What is a pilot or feasibility study? A review of current practice and editorial policy. BMC Med Res Methodol 2010;10:67

[7] Lancaster G, Dodd S, Williamson PR. Design and analysis of pilot studies: recommendations for good practice. J Eval Clin Pract 2002;102:307–312

[8] Eldridge SM, Lancaster GA, Campbell MJ, Thabane L, Hopewell S, Coleman CL, et al. Defining Feasibility and Pilot Studies in Preparation for Randomized Controlled Trials: Development of a Conceptual Framework. PLoS ONE 2016;11(3): e0150205. doi:10.1371/journal.pone.0150205

[9] Lancaster GA. Pilot and feasibility studies come of age! Pilot Feasibility Stud 2015;1:1

[10] Westlund E, Stuart A. The nonuse, misuse, and proper use of pilot studies in experimental evaluation research. Am J Eval 2016. doi: 10.1177/1098214016651489

[11] http://www.consort-statement.org/about-consort/impact-of-consort, accessed 08/11/2016

[12] Turner L, Shamseer L, Altman DG, Schulz KF, Moher D. Does use of the CONSORT Statement impact the completeness of reporting of randomized controlled trials published in medical journals? A Cochrane review. Systematic Rev 2012;1:60

[13] Stevens A, Shamseer L, Weinstein E, Yazdi F, Turner L, Thielman J, et al. Relation of completeness of reporting of health research to journals' endorsement of reporting guidelines: systematic review. Br Med J 2014;348:g3804 doi: 10.1136/bmj.g3804