Improving the reporting of pragmatic trials: an extension of the CONSORT statement

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Pragmatic trials are designed to inform decisions about practice, but poor reporting can reduce their usefulness. The CONSORT and Practihc groups describe modifications to the CONSORT guidelines to help readers assess the applicability of the results

Randomised controlled trials are used to assess the benefits and harms of interventions in health care. If conducted properly, they minimise the risk of bias (threats to internal validity), particularly selection bias.[1 2] There is, however, considerable evidence that trials are not always well reported,[3 4] and this can be associated with bias, such as selective reporting of outcomes.[5]

The usefulness of a trial report also depends on the clarity with which it details the relevance of its interventions, participants, outcomes, and design to the clinical, health service, or policy question it examines. Furthermore, a trial may be valid and useful in the healthcare setting in which it was conducted but have limited applicability (also known as generalisability or external validity) beyond this because of differences between the trial setting and other settings to which its results are to be extrapolated.

Schwartz and Lellouch[6] coined the terms "pragmatic" to describe trials designed to help choose between options for care, and "explanatory" to describe trials designed to test causal research hypotheses—for example, that an intervention causes a particular biological change. Table 1 shows some key differences between explanatory and pragmatic trials. Table 2 compares a trial that was highly explanatory in attitude[7] with one that was highly pragmatic.[8] There is a continuum rather than a dichotomy between explanatory and pragmatic trials. In fact, Schwartz and Lellouch characterised pragmatism as an attitude to trial design rather than a characteristic of the trial itself. The pragmatic attitude favours design choices that maximise applicability of the trial's results to usual care settings, rely on unarguably important outcomes such as mortality and severe morbidity, and are tested in a wide range of participants.[9-11] As Schwartz and Lellouch wrote: "Most trials done hitherto have adopted the explanatory approach without question; the pragmatic approach would often have been more justifiable."[6]

Calls have been made for more pragmatic trials in general,[6 12 13] and in relation to specific clinical problems.[14-16] Articles have been published discussing the characteristics and value of pragmatic trials[17-21] proposing improvements in the design and conduct of these trials.[22-24] Patients, advocacy groups, clinicians, systematic reviewers, funders, and policymakers want to use the results of randomised controlled trials. As such, a clear description of the design and execution of the trial, the intervention and comparator, and the setting in which health care is provided may simplify their decision on the likely benefits, harms, and costs to be expected when implementing the intervention in their own situation. There is, however, no accepted standard to guide reporting on the aspects of design and conduct of trials that affect their usefulness for decision making, particularly considerations that would affect the applicability of the results.

Table 1 | Key differences between trials with explanatory and pragmatic attitudes, adapted from a table presented at the 2008 Society for Clinical Trials meeting by Marion Campbell, University of Aberdeen

	Explanatory attitude	Pragmatic attitude
Question	Efficacy—can the intervention work?	Effectiveness—does the intervention work when used in normal practice?
Setting	Well resourced, "ideal" setting	Normal practice
Participants	Highly selected. Poorly adherent participants and those with conditions which might dilute the effect are often excluded	Little or no selection beyond the clinical indication of interest
Intervention	Strictly enforced and adherence is monitored closely	Applied flexibly as it would be in normal practice
Outcomes	Often short term surrogates or process measures	Directly relevant to participants, funders, communities, and healthcare practitioners
Relevance to practice	Indirect—little effort made to match design of trial to decision making needs of those in usual setting in which intervention will be implemented	Direct—trial is designed to meet needs of those making decisions about treatment options in setting in which intervention will be implemented

Table 2 | Comparison of trial that was highly explanatory in attitude with trial that was highly pragmatic

	Highly explanatory attitude (NASCET7)	Highly pragmatic attitude (Thomas et al8)
Question	Among patients with symptomatic 70-99% stenosis of carotid artery can carotid endarterectomy plus best medical therapy reduce outcomes of major stroke or death over next two years compared with best medical therapy alone?	Does a short course of acupuncture delivered by a qualified acupuncturist reduce pain in patients with persistent non-specific low-back pain?
Setting	Volunteer academic and specialist hospitals with multidisciplinary neurological-neurosurgical teams and high procedure volumes with low mortality in US and Canada	General practice and private acupuncture clinics in UK
Participants	Symptomatic patients stratified for carotid stenosis severity, with primary interest in severe carotid stenosis (high risk) group, who were thought to be most likely to respond to endarterectomy. Exclusions included mental incompetence and another illness likely to cause death within 5 years. Patients also were temporarily ineligible if they had any of seven transient medical conditions (eg, uncontrolled hypertension or diabetes)	Anyone aged 18-65 with non-specific low back pain of 4-52 weeks' duration who were judged to be suitable by their general practitioner. There were some exclusion criteria, eg those with spinal disease
Intervention	Endarterectomy had to be carried out (rather than stenting or some other operation), but the surgeon was given leeway in how it was performed. Surgeons had to be approved by an expert panel, and were restricted to those who had performed at least 50 carotid endarterectomies in the past 24 months with a postoperative complication rate (stroke or death within 30 days) of less than 6%. Centre compliance with the study protocol was monitored, with the chief investigator visiting in the case of deficiencies	Acupuncturists determined the content and number of treatments according to patients' needs
Outcomes	The primary outcome was time to ipsilateral stroke, the outcome most likely to be affected by carotid endarterectomy. Secondary outcomes: all strokes, major strokes, and mortality	Primary outcome was bodily pain as measured by SF-36. Secondary outcomes included use of pain killers and patient satisfaction
Relevance to practice	Indirect—patients and clinicians are highly selected and it isn't clear how widely applicable the results are	Direct—general practitioners and patients can immediately use the trial results in their decision making

We propose here guidance for reporting pragmatic trials, as a specific extension of the CONSORT statement.[25] Our aim is to identify information which, if included in reports of pragmatic trials, will help users determine whether the results are applicable to their own situation and whether the intervention might be feasible and acceptable. Reporting this information is crucial for any trial that is intended to inform decisions about practice.

The CONSORT recommendations are intentionally generic, and necessarily do not consider in detail all types of trials. Extensions of the CONSORT statement have been developed for non-inferiority and equivalence,[26] cluster randomised designs,[27] reporting of abstracts,[28] data on harms,[29] trials of herbal interventions,[30] and of non-pharmacological interventions,[31 32] but not yet for the reporting of pragmatic trials, although some issues pertaining to pragmatic trials were discussed in the CONSORT explanation and elaboration paper.[4]

Methods

In January 2005 and in March 2008, we held two-day meetings in Toronto, Canada, to discuss ways to increase the contribution of randomised controlled trials to healthcare decision making, focusing on pragmatic trials. Participants included people with experience in clinical care, commissioning research, healthcare financing, developing clinical practice guidelines, and trial methodology and reporting. Twenty four people participated in 2005 and 42 in 2008, including members of the CONSORT and Pragmatic Trials in Healthcare (Practihc) groups.[33]

After the 2005 meeting a draft revised checklist for the extension was circulated to a writing group, including some of those invited to the meeting but unable to attend. After several revisions the writing group produced a draft summary paper. At the 2008 meeting the draft was discussed and modified. It was circulated to the CONSORT group for feedback, modified, and submitted for publication.

Recommendations for reporting pragmatic trials

Meeting participants agreed that no items needed to be added to the CONSORT checklist and that the flow diagram did not need modification. However, participants felt that eight items (2-4, 6, 7, 11, 13, and 21) needed additional text specific to the reporting of pragmatic trials (table 3). Although participants discussed additional text for item 1 of the checklist (title/abstract), principally adding the word pragmatic to the title or abstract, we decided against making this recommendation because it may reinforce the misconception that there is a dichotomy between pragmatic and explanatory trials rather than a continuum. We elected not to extend item 5 (objectives), although we would encourage trialists to report the purpose of the trial in relation to the decisions that it is intended to inform and in which settings; we have included this recommendation in connection with the extension of item 2 (background).

Discussion

As demand rises for more pragmatic trials to inform real world choices,[13] so too does the need to ensure that the results are clearly reported. Readers need to be able to evaluate the validity of the results, the extent to which they are applicable to their settings, and the feasibility of the tested interventions. The existing CONSORT statement applies fully and directly to pragmatic trials. Here we have proposed extensions for eight items in the statement to make more explicit the important attributes of pragmatic trials and thus to ease the task of users in assessing feasibility, relevance, and likely effects of the intervention in their own setting.

We reached consensus that the trial results are likely to be more widely applicable if the participants, communities, practitioners, or institutions were not narrowly selected; if the intervention was implemented without intense efforts to standardise it; if the comparator group received care or other interventions already widely used; and if the outcomes studied were of importance to the relevant decision makers. The

Table 3 | Checklist of items for reporting pragmatic trials

Section	Item	Standard CONSORT description	Extension for pragmatic trials
Title and abstract	1	How participants were allocated to interventions (eg, "random allocation," "randomised," or "randomly assigned")	
Introduction			
Background	2	Scientific background and explanation of rationale	Describe the health or health service problem that the intervention is intended to address and other interventions that may commonly be aimed at this problem
Methods			
Participants	3	Eligibility criteria for participants; settings and locations where the data were collected	Eligibility criteria should be explicitly framed to show the degree to which they include typical participants and/or, where applicable, typical providers (eg, nurses), institutions (eg, hospitals), communities (or localities eg, towns) and settings of care (eg, different healthcare financing systems)
Interventions	4	Precise details of the interventions intended for each group and how and when they were actually administered	Describe extra resources added to (or resources removed from) usual settings in order to implement intervention. Indicate if efforts were made to standardise the intervention or if the intervention and its delivery were allowed to vary between participants, practitioners, or study sites
			Describe the comparator in similar detail to the intervention
Objectives	5	Specific objectives and hypotheses	
Outcomes	6	Clearly defined primary and secondary outcome measures and, when applicable, any methods used to enhance the quality of measurements (eg, multiple observations, training of assessors)	Explain why the chosen outcomes and, when relevant, the length of follow-up are considered important to those who will use the results of the trial
Sample size	7	How sample size was determined; explanation of any interim analyses and stopping rules when applicable	If calculated using the smallest difference considered important by the target decision maker audience (the minimally important difference) then report where this difference was obtained
Randomisation—sequence generation	8	Method used to generate the random allocation sequence, including details of any restriction (eg, blocking, stratification)	
Randomisation—allocation concealment	9	Method used to implement the random allocation sequence (eg, numbered containers or central telephone), clarifying whether the sequence was concealed until interventions were assigned	
Randomisation—implementation	10	Who generated the allocation sequence, who enrolled participants, and who assigned participants to their groups	
Blinding (masking)	11	Whether participants, those administering the interventions, and those assessing the outcomes were blinded to group assignment	If blinding was not done, or was not possible, explain why
Statistical methods	12	Statistical methods used to compare groups for primary outcomes; methods for additional analyses, such as subgroup analyses and adjusted analyses	
Results			
Participant flow	13	Flow of participants through each stage (a diagram is strongly recommended)—specifically, for each group, report the numbers of participants randomly assigned, receiving intended treatment, completing the study protocol, and analysed for the primary outcome; describe deviations from planned study protocol, together with reasons	The number of participants or units approached to take part in the trial, the number which were eligible, and reasons for non-participation should be reported
Recruitment	14	Dates defining the periods of recruitment and follow-up	
Baseline data	15	Baseline demographic and clinical characteristics of each group	
Numbers analysed	16	Number of participants (denominator) in each group included in each analysis and whether analysis was by "intention-to-treat"; state the results in absolute numbers when feasible (eg, 10/20, not 50%)	
Outcomes and estimation	17	For each primary and secondary outcome, a summary of results for each group and the estimated effect size and its precision (eg, 95% CI)	
Ancillary analyses	18	Address multiplicity by reporting any other analyses performed, including subgroup analyses and adjusted analyses, indicating which are prespecified and which are exploratory	
Adverse events	19	All important adverse events or side effects in each intervention group	
Discussion			
Interpretation	20	Interpretation of the results, taking into account study hypotheses, sources of potential bias or imprecision, and the dangers associated with multiplicity of analyses and outcomes	
Generalisability	21	Generalisability (external validity) of the trial findings	Describe key aspects of the setting which determined the trial results. Discuss possible differences in other settings where clinical traditions, health service organisation, staffing, or resources may vary from those of the trial
Overall evidence	22	General interpretation of the results in the context of current evidence	

intervention needs to be precisely described if readers are to be able to assess its feasibility.

The multiplicity and independence of the elements constituting the design of pragmatic trials guarantee that pragmatism is not an all or none attribute; rather, it might be best conceived as a continuum along several dimensions. For example, a randomised trial could have broad inclusion criteria for participants but rely primarily on a short term, physiological outcome rather than one that is more meaningful to the participants. Alternatively, a trial might include a wide range of participants, meaningfully assess the effect, but evaluate an intervention that is enforced or tightly monitored and thus not widely feasible. Other permutations probably exist. It is not the case that more pragmatic is always better; a trial's design should be such that the results will meet the needs of the intended users. A trial intended to inform a research decision about the biological effect of a new drug is likely to be more explanatory in design. At a later date, a trial of that same drug aimed at helping patients, practitioners, or policymakers to decide whether it should be prescribed is likely to be more pragmatic in design. To help display this multidimensionality, we have developed of a tool, primarily intended to be used in designing a trial, for characterising where it will stand along the pragmatic-explanatory continuum in relation to each design decision.[34]

We hope that these reporting guidelines will help editors, reviewers, trialists, and policy makers in reporting, reviewing, and using pragmatic trials. Journals that have endorsed the CONSORT statement could also support CONSORT for pragmatic trials, by including reference to this extension paper in the journal's instructions to authors. We also invite editorial groups to consider endorsing the CONSORT extension for pragmatic trials and encourage authors to adhere to it. Up to date versions of all CONSORT guidelines can be found on the CONSORT website (www.consort-statement.org).

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