thebmj

Letters

Incorporating patient preferences into clinical trials

BMJ 1998; 317 doi: https://doi.org/10.1136/bmj.317.7150.78 (Published 04 July 1998) Cite this as: BMJ 1998;317:78

Information about patients' preference must be obtained first

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EDITOR—Torgerson and Sibbald discuss the difficulties of assessing the relative merits of treatments when patients have strong preferences for one of the alternatives.[1] In these circumstances, however, patients should not be expected to participate in randomised comparisons, and neither should the professionals caring for them.

It is important to consider the bases of these preferences, particularly as there is a widespread and unsupported belief that new treatments are likely to be superior to existing alternatives.[2] For example, it seems that people with diabetes who were being recruited to a randomised comparison of insulin pumps with conventional management were left with the impression that pumps represented an important advance (C Bradley, personal communication). Not surprisingly, therefore, those allocated to pumps were pleased, while those allocated to conventional management were disappointed. Randomisation thus created comparison groups that were incomparable in these psychological characteristics, and this may have had implications for compliance and evaluation of treatment outcome.[3]

Bradley's response[3] was to propose the partially randomised patient preference design to which Torgerson and Sibbald refer. Unfortunately, this does not help because it cannot distinguish between an effect of preferences and an effect of confounding of preferences with prognosis. Since it is impossible to randomise between sincerely held preferences, measuring their effects reliably requires a more complicated design, which was suggested originally by Rucker[4] and recently

discussed by McPherson et al.[5] In this design, people are randomised between either a randomised comparison or a preference comparison.

Genuine therapeutic effects may, however, be associated with preferences, over and above those related to adherence to treatment. Several blind trials show an advantage associated with adherence to placebo.[5] Obtaining hard evidence on possible preference effects is problematic as it is difficult to distinguish reliably between simple therapeutic effects and preference effects mediated through psychological pathways in experiments.

There are thus two areas that need attention. Firstly, there needs to be wider acknowledgement that preferences for treatments should be based on beliefs that are founded on reliable information. This should help to increase the proportion of well informed people who have no strong preferences and would thus be eligible to participate in comparisons between randomised treatments. Secondly, studies are required to enable a rigorous distinction to be made between simple therapeutic effects and preference effects. This means that well accepted biological hypotheses will be needed for adequate recruitment, and a plausible biological model to distinguish the two kinds of effect. As Torgerson and Sibbald suggest, however, the first step is routinely to elicit information about the preferences of well informed patients.

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Merits of alternative strategies for incorporating patient preferences into clinical trials must be considered carefully

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EDITOR—In their overview of patient preference trials, Torgerson and Sibbald suggest that, given the potential drawbacks of such designs, research might usefully take the alternative approach of measuring patient preferences within a traditional randomised controlled trials design.[1] This would conserve “the advantages of a fully randomised design with the additional benefit of allowing for the interaction between preference and outcome to be assessed.” We used Torgerson and Sibbald's approach to compare two schedules of routine antenatal visits.[2] Our unpublished findings on patient preferences show how such analysis can extend and clarify trial findings.

We stratified our two groups—new style care (6-7 antenatal visits) and traditional care (13 antenatal visits)—by the initial preferences of the women who took part. Within each stratum we compared those allocated to traditional and new style care for one key acceptability outcome (dissatisfaction with the frequency of antenatal visits) and one key outcome relating to psychosocial effectiveness (negative attitude to the fetus). The findings are shown in the table.

Initial preference	Allocated to traditional care	Allocated to new style care	Odds ratio (95% CI)*
Dissatisfaction with frequency of visits (%)			
None	7.4 (29/391)	31.0 (135/435)	5.62 (3.61 to 8.94)
Traditional care	3.5 (11/317)	71.1 (118/166)	68.39 (33.31 to 149.14)
New style care	48.9 (110/225)	12.2 (36/294)	0.15 (0.01 to 0.23)
Negative attitude to fetus (mean (SD))			
None	6.0 (4.25)	6.8 (4.26)	0.003
Traditional care	6.3 (4.05)	7.5 (3.83)	0.005
New style care	5.7 (4.12)	5.8 (3.77)	0.452

- * P value for negative attitude to fetus.

Comparison of two groups of women for one key acceptability outcome (dissatisfaction with frequency of antenatal visits) and one key outcome relating to psychosocial effectiveness (negative attitude to fetus)

Our main overall finding of greater dissatisfaction in the new style group applies only to those who had either an initial preference for traditional care or no initial preference. When women had an initial preference for new style care the effect was in the opposite direction. Similarly, the finding that women in the new style group had a more negative attitude to their fetuses only applies to those who initially preferred traditional care or had no initial preference. This analysis provides information that is relevant for new policies: women with an active preference for fewer visits should not be denied this option because of concern about possible detrimental psychosocial effects.

A partially randomised patient preference design would have yielded similar findings, but at the cost of a substantial increase in sample size. We assumed that all those with an initial preference would have opted for their preferred type of care and that eligible women who declined participation because they did not want to have fewer antenatal visits would have taken part if it had been a preference trial. We calculate that the required overall sample size would have increased from 2830 to 7989. This highlights the need to consider carefully the respective merits of alternative strategies for incorporating patient preferences into clinical trials.

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Authors' reply

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EDITOR—McPherson and Chalmers are correct that more research is required into the interaction between preferences and outcome. Indeed, the first randomised trial comparing an ordinary randomised trial and a patient preference trial has just been published.**1** This trial showed no difference in recruitment and retention rates between the two randomised segments of the trials.

Clearly it is important that patients are given reliable information. It is, however, likely that some patients have preferences and are still prepared to be randomised. What should be done with such patients? Including them in a “partially randomised preference trial” is unsatisfactory, as McPherson and Chalmers state. Randomising patients between a fully randomised design and a preference trial is problematic, as suggested, not least because those patients allocated to a fully randomised design must be denied their preferences if full assessment of preferences is to be taken into account. This is probably ethical if the preferred treatment is available only to randomised patients.

Randomising all consenting patients and eliciting their treatment preferences may help.2 Clement et al show that this is both feasible and a better alternative to the partially randomised design. They are, however, incorrect to assume that a partially randomised patient preference trial would have yielded similar findings. The preference arms in a preference trial could have been subject to confounding and, furthermore, would not have yielded information on the dissatisfaction rates and attitude scores of women allocated to their unpreferred treatment.

The importance of this is illustrated in the findings of their previous paper. The odds ratio of dissatisfaction was 2.50 (95% confidence interval 2.00 to 3.11) for the new group compared with the traditional group.3 When all women with an initial preference are removed (hence achieving a balance of preference between the two groups) the odds ratio increases to 5.62 (3.61 to 8.94), thus indicating a significant effect of preference on this trial outcome.

McPherson et al point out the sample size required to test for any interaction between preference and outcome would need to be relatively large.4 Many trials may be too small to examine the interaction between preference and outcome. One solution may be to try to standardise how preferences are elicited and then use meta-analytical techniques by combining a number of trials to assess differences in outcome by preference.

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