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Understanding controlled trials

What is a patient preference trial?

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This is the third of an occasional series on the methods of randomised controlled trials

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A common problem in randomised controlled trials arises when patients (or their clinicians) have such strong treatment preferences that they refuse randomisation.¹ The absence of these patients from trials may restrict generalisation of the results, as participants may not be representative. A further potential source of bias exists when patients with strong treatment preferences are recruited and randomised. When it is not possible to blind patients to their treatment allocation they may suffer resentful demoralisation² if they do not receive their preferred treatment and may comply poorly. On the other hand, patients receiving their preferred treatment may comply better than average. There may therefore be a treatment effect which results from patient preferences and not from therapeutic efficacy. The effects of resentful demoralisation are so far a theoretical concern which have yet to be shown in practice, in part because they are difficult to evaluate.

Patients may be placed in one of three groups according to preference and willingness to be randomised: (a) patients who have no strong preferences and therefore consent to randomisation; (b) patients with a preference who still consent to randomisation; and (c) patients who refuse randomisation and opt for their treatment of choice.

To cope with patient preferences the use of a comprehensive cohort design³ or the patient preference trial has been suggested.⁴ Patients with treatment preferences are allowed their desired treatment; those who do not have strong views are randomised conventionally. Hence, in a trial of two interventions, A and B, we end up with four groups: randomised to A; prefer A; randomised to B; prefer B. The analysis of such a trial is uncertain. Any comparison using the non-randomised groups is unreliable because of the presence of unknown and uncontrolled confounders.⁵ At least one analysis should therefore be a comparison between the two randomised arms alone. Analyses which include the unrandomised groups should be treated as observational studies with known confounding factors adjusted for in the analysis. Olschewski and Scheurlen have suggested that an analysis using randomisation status as a covariate might be helpful.³ A further limitation of the patient preference approach is that it may increase the size and cost of trials.

An alternative to the partially randomised approach has been proposed whereby the strength and direction

of patient preferences are elicited before randomisation, with all consenting patients randomised.⁶ This approach combines the advantage of the partially randomised design—that is, gathering information on the effect of preference on outcome—but retains the rigour of a full randomised design.⁶ The design has been used in a randomised trial of physiotherapy treatment for back pain and, despite most patients expressing a preference, no patient refused randomisation.⁶ The practical advantages of establishing and including patient preferences in trials has not been fully established. However, using a patient preference design, Henshaw et al in a comparison of medical versus surgical abortion produced important additional information on the acceptability of the two treatments in different preference groups which would not have been available in the usual trial.⁷ In addition, a recent preference trial of early amniocentesis versus chorionic villus sampling for diagnosing fetal abnormalities showed that rate of pregnancy loss did not differ between the preference group and their randomised equivalent.⁸ This trial is important in that only 38% of patients accepted randomisation. Thus, including the unrandomised patients in the trial offered some reassurance that the results could be extrapolated to a wider group of patients.

Patient preference designs complement, but do not replace randomised trials. However, measuring patient preferences within a fully randomised design deserves further use as this conserves all the advantages of a fully randomised design with the additional benefit of allowing for the interaction between preference and outcome to be assessed.

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