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Understanding controlled trials

Why are randomised controlled trials important?

Bonnie Sibbald, Martin Roland

Randomised controlled trials are the most rigorous way of determining whether a cause-effect relation exists between treatment and outcome and for assessing the cost effectiveness of a treatment. They have several important features:

- Random allocation to intervention groups
- Patients and trialists should remain unaware of which treatment was given until the study is completed—although such double blind studies are not always feasible or appropriate
- All intervention groups are treated identically except for the experimental treatment
- Patients are normally analysed within the group to which they were allocated, irrespective of whether they experienced the intended intervention (intention to treat analysis)
- The analysis is focused on estimating the size of the difference in predefined outcomes between intervention groups.

Other study designs, including non-randomised controlled trials, can detect associations between an intervention and an outcome. But they cannot rule out the possibility that the association was caused by a third factor linked to both intervention and outcome. Random allocation ensures no systematic differences between intervention groups in factors, known and unknown, that may affect outcome. Double blinding ensures that the preconceived views of subjects and clinicians cannot systematically bias the assessment of outcomes. Intention to treat analysis maintains the advantages of random allocation, which may be lost if subjects are excluded from analysis through, for example, withdrawal or failure to comply. Meta-analysis of controlled trials shows that failure to conceal random allocation and the absence of double blinding yield exaggerated estimates of treatment effects.¹

Although randomised controlled trials are powerful tools, their use is limited by ethical and practical concerns. Exposing patients to an intervention believed to be inferior to current treatment is often thought unethical. For example, a non-random study suggested that multivitamin supplementation during pregnancy could prevent neural tube defects in children.² Although the study was seriously flawed, ethics committees were unwilling to deprive patients of this potentially useful treatment, making it difficult to carry out the trial which later showed that folic acid was the effective part of the multivitamin cocktail.³ On the

other hand, failure to perform trials may result in harmful treatments being used. For example, neonates were widely treated with high concentrations of oxygen until randomised trials identified oxygen as a risk factor for retinopathy of prematurity.⁴

In other circumstances a randomised controlled trial may be ethical but infeasible—for example, because of difficulties with randomisation or recruitment. Indeed, once an intervention becomes widespread, it can prove impossible to recruit clinicians who are willing to “experiment” with alternatives. A recent attempt to conduct a trial of counselling in general practice failed when practitioners declined to recruit patients to be allocated at random.⁵ Strong patient preferences may also limit recruitment and bias outcomes if not accommodated within the study design.⁶

A third limiting factor is that randomised controlled trials are generally more costly and time consuming than other studies. Careful consideration therefore needs to be given to their use and timing.

- Is the intervention well enough developed to permit evaluation? This can be especially difficult to decide when new interventions are heavily dependent on clinicians’ skills (surgical procedures⁷ or “talk” therapies).
- Is there preliminary evidence that the intervention is likely to be beneficial (from observational studies), including some appreciation of the size of the likely treatment effect? Such information is needed to estimate sample sizes and justify the expense of a trial. Given these constraints, it remains an ideal that all new healthcare interventions should be evaluated through randomised controlled trials. Given that poor design may lead to biased outcomes,¹ trialists should strive for methodological rigour and report their work in enough detail for others to assess its quality.⁸

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National Primary Care Research and Development Centre, University of Manchester, Manchester M13 9PL

Bonnie Sibbald, reader in health services research
Martin Roland, director of research and development

Correspondence to: Dr Sibbald.

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