Need for expertise based randomised controlled trials

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Surgical procedures are less likely to be rigorously evidence based than drug treatments because of difficulties with randomisation. Expertise based trials could be the way forward

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Although conventional randomised controlled trials are widely recognised as the most reliable method to evaluate pharmacological interventions,[1 2] scepticism about their role in non-pharmacological interventions (such as surgery) remains.[3–6] Conventional randomised controlled trials typically randomise participants to one of two intervenions (A or B) and individual clinicians give intervention A to some participants and B to others. An alternative trial design, the expertise based randomised controlled trial, randomises participants to clinicians with expertise in intervention A or clinicians with expertise in intervention B, and the clinicians perform only the procedure they are expert in. We present evidence to support our argument that increased use of the expertise based design will enhance the validity, applicability, feasibility, and ethical integrity of randomised controlled trials in surgery. We focus on established surgical interventions rather than new surgical procedures in which clinicians have not established expertise.

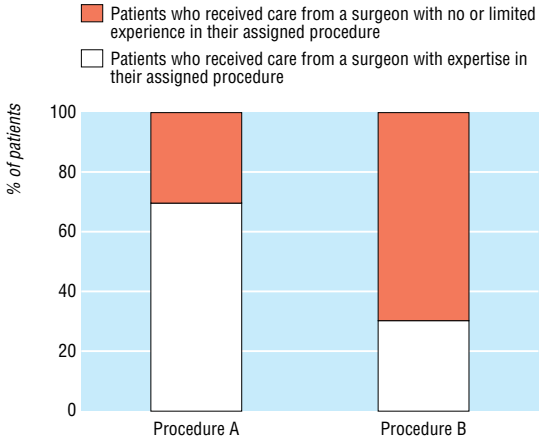
Use of expertise based trials

Investigators have used the expertise based design when conventional randomised controlled trials were impossible because different specialty groups provided the interventions under evaluation—for example, percutaneous transluminal coronary angioplasty versus coronary artery bypass graft surgery.[7–9] In 1980, Van der Linden suggested randomising participants to clinicians committed to performing different interventions in an area in which a conventional randomised controlled trial was possible.[10] Since that time, however, the expertise based design has been little used, even in areas where it has high potential (such as, surgery, physiotherapy, and chiropractic).

Problems with validity of conventional randomised controlled trials

Differential expertise between procedures

Because it takes training and experience to develop expertise in surgical interventions, individual surgeons tend to solely or primarily use a single surgical approach to treat a specific problem.[10 11] The restricted expertise that results can compromise the validity of conventional randomised controlled trials. For example, in a conventional randomised controlled trial, if surgeons with expertise in intervention A treat 70% of the patients in both groups A and B, and surgeons with expertise in intervention B treat 30% of those in both groups A and B, the trial results will be biased towards intervention A (fig 1). We will refer to this type of bias as differential expertise bias. The more disproportionate the number of cases being performed by surgeons with expertise in procedure A compared with surgeons with expertise in procedure B, the greater the impact of differential expertise bias on the trial results.



Differential expertise bias in a conventional randomised controlled trial in which 70% of surgeons are expert in procedure A and 30% in procedure B

We estimated the potential for differential expertise bias through a survey of 139 surgeons in a large (>1000 patients) conventional randomised controlled trial comparing two surgical procedures for treating a tibial shaft fracture (reaming versus no reaming before insertion of an intramedullary nail).[12] Seventy four surgeons completed the survey. Significantly more surgeons had no or limited experience with the

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Table 1 Experience of 74 surgeons with reamed and non-reamed procedure in the year before participating in the randomised controlled trial

No of cases	No (%) of surgeons	
	Reamed procedure	Non-reamed procedure
0	7 (9)	26 (35)
1-4	8 (11)	22 (30)
5-9	18 (24)	11 (15)
10-19	15 (20)	4 (5)
20-40	17 (23)	7 (9)
> 40	9 (12)	4 (5)

non-reamed procedure (which is more technically challenging) than the reamed procedure in the year before they joined the randomised controlled trial (table 1). The median number of cases surgeons performed in the year before randomised controlled trial participation was 12 reamed procedures and 2 non-reamed procedures (median difference 7 procedures, 95% confidence interval 5 to 11).

This example shows the potential for differential expertise bias. Three key considerations suggest that this problem is likely to be common in surgical trials. Firstly, trialists rarely, if ever, institute measures to ensure that the number of participating surgeons with expertise in each procedure is equal. Secondly, although some conventional randomised controlled trials try to reduce bias by requiring participating surgeons to perform a minimum number of both the experimental and control procedures before participating in the trial, this measure is unlikely to eliminate bias because outcomes often improve with extensive experience with a procedure. Thirdly, even if these two problems are overcome, one of the procedures (let us say procedure A) may be more technically challenging. If this is the case, after doing the required numbers of unfamiliar procedures, surgeons who have to acquire expertise in procedure A will remain more technically challenged than those who have to acquire skills in procedure B. In this situation, the trial will be biased towards B, the less technically challenging procedure.

Potential problems related to unblinded surgeons

Surgeons participating in conventional surgical randomised controlled trials usually have opinions about the relative effectiveness of the procedures under investigation. Surgeons solely or primarily using procedure A probably do so because they believe it gives better outcomes. As a result, they probably expect and hope that the randomised controlled trial testing the outcomes of procedure A versus procedure B will affirm their belief.

Thus, surgeons, who are necessarily unblinded to the procedure they perform, may subconsciously systematically bias trial findings in a conventional randomised controlled trial. This bias may manifest itself through several mechanisms, including being more meticulous when performing one procedure than the other or differentially prescribing effective cointerventions.[2] Although it is preferable for independent blinded individuals to collect data and assess outcomes, in some trials it is done by the surgeons. When outcome evaluation is open to judgment and surgeons are involved in the process, they may differentially record data, repeat measurements, or interpret outcomes depending on whether a patient received procedure A or procedure B.[13]

We asked surgeons participating in the randomised controlled trial of different strategies for nailing tibial fractures whether they thought a reamed procedure or non-reamed procedure was superior before participating in the randomised controlled trial and at the time of the survey (that is, when about 900 patients had been randomised).[12] Surgeons rated their confidence about the superiority of the procedure they selected on a seven point scale, with 1 representing no confidence, 4 representing moderate confidence, and 7 representing extreme confidence. Before participating in the randomised controlled trial, 87% (95% confidence interval 77% to 94%) of respondents believed that a reamed procedure was superior and 86% of respondents indicated their confidence about the superiority of a reamed procedure was in the moderate to extreme range. After 900 patients were randomised, responses remained similar.

The results of this survey reflect the possible magnitude of treatment preference among surgeons participating in a randomised controlled trial comparing surgical procedures. This may lead to bias for

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Table 2 Conditions for pragmatic and explanatory randomised controlled trials using conventional and expertise based methods

	Pragmatic trial	Explanatory trial
Conventional	All surgeons in routine clinical practice setting	Surgeons with advanced expertise in ideal clinical settings
Expertise based	All surgeons with expertise in procedure A or procedure B in routine clinical practice settings	Surgeons with advanced expertise in procedure A or B in ideal clinical settings

reasons outlined above. As is the case with balancing expertise, trialists are unlikely to be able to ensure the absence of a dominant treatment preference among participating surgeons.

Procedural crossovers

Our ability to determine if patients have a better outcome when they receive one of two procedures will be enhanced if patients actually receive the procedures to which they were randomised. If this is not the case because of procedural crossovers, the trial's ability to determine the true effect will be compromised.

We evaluated the number of crossovers in the reamed and non-reamed groups in the trial we surveyed. Of the 510 patients allocated to a reamed intervention, five received a non-reamed procedure, whereas of the 498 patients allocated to a non-reamed intervention, 40 received a reamed procedure ($P<0.0001$). These findings show the large potential for differential crossovers in a conventional randomised controlled trial. Procedural crossovers initiated by surgeons are more common when surgeons have limited experience with a procedure than when they have more extensive experience.[14–16] Except for the unlikely event that exactly the same number of participating surgeons have expertise in the experimental and control procedures (and both groups are allocated to perform an equal number of procedures A and B), there is a potential for differential crossover in the two arms.

Validity of surgical expertise based randomised controlled trials

In the surgical expertise based randomised controlled trial, patients are randomised to different surgeons with expertise in the relevant intervention. The first advantage of the expertise based randomised controlled trial is that surgeons will perform only the procedure in which they have expertise, avoiding the problem of differential expertise.

As in the conventional randomised controlled trial, surgeons in the expertise based randomised controlled trial will be unblinded. However, in the expertise based randomised controlled trial surgeons are likely to be subconsciously biased toward the procedure in which they have expertise. Consequently, the likelihood of differential procedural performance, cointerventions, data collection, and outcome assessment decreases. A third advantage of the expertise based randomised controlled trial is that procedural crossovers are less likely to occur because surgeons are doing the procedures with which they are most comfortable.[14–16]

Applicability of expertise based randomised controlled trials

If an expertise based randomised controlled trial shows that one procedure is superior to another, it does not follow that all surgeons with expertise in the less effective procedure and little or no experience in the more effective procedure can expect their patients to have better outcomes if they immediately start performing the superior procedure. Rather, if these surgeons acquire the same skill set and expertise as the surgeons who participated in the randomised controlled trial, they can expect their patients to have improved outcomes when they switch procedures.

The applicability of the results of a surgical randomised controlled trial further relates to whether a trial is an explanatory trial that uses only surgeons with advanced expertise in ideal clinical settings or if it is a pragmatic trial that uses surgeons with at least basic competence in routine clinical practice settings. Both conventional and expertise based randomised controlled trials can be explanatory or pragmatic trials (table 2).

Feasibility

Surgical expertise based randomised controlled trials may be more feasible than conventional randomised controlled trials. Surgeons may be more willing to participate in an expertise based randomised controlled trial because they have to perform only the procedure for which they have developed expertise. Furthermore, surgeons do not have to do a minimum number of operations with the unfamiliar intervention before participating in the trial. This is likely to appeal to both surgeons and investigators and could prevent delays in starting trials.

A surgical expertise based randomised controlled trial must ensure satisfactory competence among the surgeons doing each procedure. Strategies to achieve this goal will include selecting qualified surgeons who have attained a specified level of post training experience, who fulfil requirements established by professional guidelines, or who have documented their expertise is at the plateau of the learning curve.

Ethics

Although the medical community accepts conventional surgical randomised controlled trials as ethical, some surgeons may have ethical problems with enrolling patients in a trial when they know they may have to do a procedure with which they feel inexperienced.[10 11] This problem does not arise in expertise based randomised controlled trials because surgeons perform only the procedures in which they have established expertise.

The consent process for expertise based randomised controlled trials can inform patients that, regardless of the procedure to which they are allocated, a surgeon with specific expertise will do the assigned intervention. Although rarely acknowledged, this is not the case for most conventional surgical randomised controlled trials. Obtaining consent for the reamed versus non-reamed trial in which we conducted our survey might have been problematic had patients been

Summary points

Questions remain about the use of randomised controlled trials to evaluate non-pharmacological interventions such as surgery

An alternative is to use expertise based randomised controlled trials, in which participants are randomised to clinicians with expertise in intervention A or intervention B

Interventions are performed only by clinicians with expertise in the procedure, which reduces both bias and ethical concerns

Expertise based randomised controlled trials may have greater applicability and feasibility than conventional trials

informed that they might be randomised to a procedure in which their surgeon was both inexperienced and sceptical of its effectiveness.

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Work based learning

In the past medical students and doctors did much of their learning in lecture halls. They then sat an examination or some other type of formal assessment. Their learning had a specific end point, when they picked up a degree or certificate. Most doctors thought that this was "proper learning" and that all other learning was somehow not up to scratch.

The problem with this type of learning is that it misses out a lot of informal learning. It misses out the learning that takes place when you ask a colleague for advice, or answer a patient's question by looking up a website, or solve a problem by setting up a meeting with colleagues. The concept of work based learning tries to capture and to quantify this learning. Barr defined work based learning as learning that takes place at work or learning that takes place away from work with the objective of improving performance at work.[1] Work based learning fits in closely with how doctors now learn. It involves keeping up to date with new developments, learning to satisfy personal as well as professional goals, learning with and from colleagues from various disciplines, learning about non-clinical as well as clinical topics, and, most importantly, learning in order to directly improve care for patients.

We have based bmjlearning.com on the principles of work based learning. Certainly most of our users can and do use the website at their workplace, and we try to publish material that users say they need on a daily basis in their work. Many users have requested a learning module on how to deal with a patient whom they suspect is a victim of domestic abuse. Traditionally this topic has been the source of much rhetoric and little action, and, although primary care workers have received some training on it, many feel that the training was not tailored to meet their needs.

We have tried to overcome these shortcomings in our new module on how to care for victims of domestic abuse. The module gives specific advice on which patients you should ask about domestic abuse and how you should ask them. It points out how the whole care team can help with such patients' medical and social needs. If you want practical advice on this subject, try our new learning module on bmjlearning.com.

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