Intention-to-treat analysis: Protecting the integrity of randomization

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Randomization is a crucial part of most clinical trials. The purpose of randomization in a trial comparing 2 groups is to ensure that the groups differ only with respect to the interventions being compared. Randomization determines not only which treatment subjects receive (eg, drug vs placebo), but also how the results are analyzed at the end of the trial.

Intention to treat prevents biased outcomes

The *intention-to-treat* principle states that all subjects must be analyzed with respect to the group to which they were randomized. Consider a recent study by Spector et al[1] in which patients with dementia were randomized to receive an intervention known as cognitive stimulation therapy (CST) or control (equivalent to placebo in a drug trial). The investigators used an intention-to-treat analysis. The intervention was a complex 14-session training program. Many patients did not complete all the sessions. Whether they had completed all the sessions or not, patients initially randomized to the intervention were still considered to have received the intervention when the results were analyzed.

At first, intention to treat doesn't seem logical. If we are testing an intervention, doesn't it make sense to evaluate its effect among patients who complied with it fully, and then compare them with patients who were not assigned to the intervention or failed to comply? The problem is, patients who fail to comply with an intervention for whatever reason (not attending all training sessions in the example above) may differ in an important way from those who do.

In the Spector study, for example, it is possible that patients who attended very few of the sessions were more likely to have some subtle cognitive deficits that limited their participation. If these patients were excluded from the analysis or placed in the control group, the 2 groups would differ not only with respect to the intervention, but also with respect to these subtle factors. The value of randomization, therefore, would be compromised. The treatment arm may even appear to be more effective than it really is.

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1. Spector A, Thorgrimsen L, Woods B, et al. Efficacy of an evidence-based cognitive stimulation therapy programme for people with dementia: randomised controlled trial. *Br J Psychiatry* 2003; 183:248–254.