The Trials and Tribulations of Interpreting Clinical Trials

By Gabrielle Saunders

As I am sure you know, evidence-based practice (EBP) is the new mantra for healthcare practice, including audiology. For EBP, research evidence, clinical expertise, and patient values and preferences should be integrated into clinical decision making, with the assumption that this will result in selection of the optimal intervention—and thus will yield the best clinical outcomes (see Wong and Hickson, 2012, for information on EBP in audiology). As a clinician you have clinical expertise, and the ability to assess the values of each of your patients, but what research evidence should you use to assess the evidence for a particular clinical practice? This is where clinical trials come in. In general, data from a randomized controlled trial (RCT) is considered to be the gold standard level of research evidence for EBP. A few issues ago in this column, Anne Olson described how to plan and implement an RCT. In this issue I am going to elaborate on her article by discussing efficacy versus effectiveness trials, the questions they address, and the aspects of clinical practice that each can inform.

Unfortunately, the terms efficacy and effectiveness are often used interchangeably. When used in the context of a clinical trial this is not appropriate because these terms refer to quite different constructs. An efficacy trial is designed to assess the outcome of an intervention when conditions for the provision of the intervention are ideal, the test conditions are highly controlled, and the participants in the trial are carefully selected such that together the conditions maximize the likelihood of showing an effect of the intervention. An effectiveness trial, on the other hand, is designed to assess the outcome of an intervention when it is provided under circumstances that more closely reflect the conditions in which the intervention would be provided in real-world clinical practice. In other words, an efficacy trial answers the question “Can the intervention work under ideal circumstances?” while an effectiveness trial answers the question “Does the intervention work in real-world practice?”

To put these two types of trial into the context of audiology, let’s compare how an efficacy trial and an effectiveness trial might be designed. Let’s imagine that a researcher is interested in knowing whether a computerized auditory training (AT) program improves understanding of speech in noise. The researcher decided that playing computer games will be the control condition against which the AT program is compared. The researcher decides to use the change in QuickSIN score as her outcome measure. In terms of training, the developer of the AT program says that to obtain the best outcome, patients should use the AT program for one hour each day for one month while wearing their hearing aids, and specifies that training should be conducted at a comfortable listening level in a quiet environment with no auditory or visual distractions. In both the efficacy trial and effectiveness trial, half of the participants would be randomly assigned to use the AT program and half would be assigned to play computer games. All would conduct the QuickSIN before beginning any training/playing of computer games and then again immediately after doing so. Ideally, QuickSIN testing would be carried out by an audiologist who was blinded to the participant’s intervention group. The difference between...
the trials would come in where the training/playing of computer games took place. In the efficacy trial, training would take place in a laboratory, while in the effectiveness trial, training would take place in the participant’s home. As a result, as shown in the sidebar, substantial differences arise.

In other words, a fundamental difference between these two imaginary studies is the degree to which the training/playing of computer games is controlled and thus the extent to which external factors will influence the outcome of the trial. In practice both of these types of trial are important because they provide different information. The efficacy trial would tell the researcher whether the AT program, when used in ideal conditions, improves speech in noise understanding to a greater extent than playing computer games, while the effectiveness trial would provide the researcher with information about whether the AT program improves speech in noise understanding to a greater extent than playing computer games when it is used in the manner in which patients will typically use it. It can be argued that if an efficacy trial is not complemented by an effectiveness trial, clinicians may be disappointed at the clinical outcomes of patients who do not use the intervention as recommended. On the other hand, an effectiveness trial alone may lead to the conclusion that a particular intervention is not helpful, when in fact the intervention could be helpful if it were used as recommended.

From a clinical perspective, efficacy trials sometimes overestimate the effect an intervention will have when it is used in clinical practice, because the data were collected under ideal circumstances. Effectiveness trials, on the other hand, tend to yield data that better reflect how well an intervention will work in clinical practice because they better mimic the circumstances in which the intervention would actually be used by the patients for whom it was developed (Singal et al., 2014). By combining information from both, one can gain insight about ways in which an efficacious intervention could be made more effective, or by knowing up front that an intervention is efficacious, it could motivate a patient to use an intervention in a more ideal manner so as to gain benefit.

Hopefully this article will prompt you to think about these differences when you read papers describing clinical trials and evaluate the findings to make decisions for your evidence-based audiological practice. If you want to look more deeply into this topic, check out Singal et al. (2014) or Sacristán (2013) for easy-to-digest, informative papers.

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References

