Trading Perfectionism for Pragmatism

Most of us, at one time or another, find ourselves seeking the one right choice, the perfect option. I was reminded of this recently when both of my children experienced exhausting and ultimately futile attempts to find the perfect sofa. The discussions of the pros and cons of frames, seating support, arm heights, cushions, and filling were reminiscent of my early years of setting up new homes. My husband and I sympathized with their plights but also found their dogged quests for perfection humorous. Eventually, both children found sofas that were less than perfect, but lovely and functional. Similarly, as a teacher of research methods to future scientists, I find myself fighting the urge to teach the correct gold standard method so that I can provide students with a balanced view of what is gained and lost when choosing one methodology over another. In this editorial, I will explore some perspectives on what may be gained and lost when using pragmatic clinical trial methodologies to test gerontological interventions.

THE TIME IS RIGHT FOR THE PRAGMATIC CLINICAL TRIAL

The health care community and public policy makers continue to be frustrated with the time it takes to transfer research findings to practice, as well as the disparity between interventions researched and interventions used in real clinical practice situations. The four phases of the clinical trial are useful for drug studies in which a drug is first tested for safety in a small group and then tested in larger, more diverse groups of individuals to (a) determine whether it is effective and (b) further evaluate its safety. Nurse researchers conduct many Phase II efficacy studies in which small controlled studies are performed with an emphasis on using research designs and methods that maximize internal validity. As I wrote in a 2010 *Research in Gerontological Nursing* editorial, “Internal validity is not a trivial pursuit” (Kovach, 2010, p. 2). To maximize confidence that differences in outcomes between treatment and control groups are due to the intervention rather than another factor, researchers control as many threats to internal validity as possible. Thus, the randomized controlled trial (RCT) has become the gold standard research design for testing new interventions. However, we have all probably read studies that have excellent methodological form but lack substance or logic, or are divorced from the real word of clinical practice. These studies do little to move science forward.

To create evidence that is useful to public policy makers, research results must have more applicability for larger groups than is commonly seen in these small, well-controlled RCTs. Based on a desire for quicker uptake of effective treatments and to test treatments under more authentic conditions, methods for pragmatic or practical clinical trials (PCTs) have emerged. If nurse researchers try to balance experimental rigor with generalizability, will they be able to fulfill their mission to move science forward and enhance older adult health? Do the advantages of ecological validity and the power of replication found in PCTs outweigh our traditional commitment to use the most rigorous methodologies available to discover new knowledge?

Advances in technology and statistics provide greater opportunities to study larger, more representative samples. Computer technologies now provide access to large datasets of health care records. Sophisticated multivariate statistical modeling allows risk adjustments, control of sample selection biases, and examination of causal paths through the use of data collected in nonexperimental studies. Increased potential also exists for sampling and measurement error, as well as other threats to internal validity using these methods.
Accumulated data over time may be able to provide a reasonable amount of certainty that an intervention is credible using a PCT design. To know when a PCT-tested intervention is suitable for widespread clinical practice, the scientific community must establish benchmarks, such as the number of studies, heterogeneity of samples, possible alternative explanations, and effect sizes. Whether this accumulation of data takes more or less time to acquire than getting to Phase IV clinical trials is unknown.

FEATURES OF PRAGMATIC CLINICAL TRIALS

Many of the hallmark features of PCTs are already commonly used in gerontological nursing studies. For example, the classic RCT uses a placebo as a control group and occurs in highly controlled clinical or laboratory settings. In PCTs, usual care is often the control condition, or a delayed treatment design is used. The settings in which many nursing interventions are tested are authentic, real-world practice settings. The classic RCT is reductionistic in manipulating a single factor, while also tightly controlling as many other conditions as possible. Compliance with the manipulation of the variable is closely monitored, and variables are measured with depth and precision. Rather than manipulate one component of a treatment, nursing interventions often have multiple components and use specific collected assessment data to inform the choice of treatment. Information is collected on fidelity to the treatment, and studies vary in their emphasis on strict adherence to each component of the treatment protocol. Reports of nursing studies, such as PCTs, place more emphasis on describing (a) intervention components delivered and not delivered, (b) the time and costs to deliver the intervention, and (c) barriers and factors facilitating implementation of the intervention. In addition, the sample, setting, and interventionist characteristics are often described in detail.

Some of the commonly cited advantages of PCTs include the argument that these studies better reflect patients and practice, are less burdensome to conduct, and produce results that are more likely to generalize to practice. However, PCTs are less experimentally rigorous, and multiple factors can make it harder to detect treatment effects. Usual care may not serve as a stable comparison condition. Increased heterogeneity in samples can increase within-group variance, making it harder to detect intervention effects.

CONCLUSION

In summary, like the quest for the perfect sofa and so many other things in life, a perfect methodology does not exist, and one design does not meet all needs. The PCT will not and should not supplant the RCT. In the context of health care reform, the PCT may help address the translational gap and provide evidence about the usefulness and efficacy of an intervention that has been tested under more authentic clinical practice conditions. Clinical researchers must be at the forefront of developing methodologies to enhance rigor in PCTs and evaluation criteria and benchmarks. Nurse researchers must be savvy methodologists so that they can create and defend the advancement of methods for PCTs and translational research.

REFERENCE


Christine R. Kovach, PhD, RN, FAAN, FGSA
Editor

The author has disclosed no potential conflicts of interest, financial or otherwise.

doi:10.3928/19404921-20121201-01