Developing Statistical Methods to Improve Stepped-Wedge Cluster Randomized Trials

Principal investigator

Patrick Heagerty, PhD

Organization

University of Washington

What was the project about?

Cluster randomized trials, or CRTs, are research studies that compare treatments among different groups of patients, or clusters. An example of a cluster is a group of people who receive care at a single clinic.

One type of CRT is a stepped-wedge CRT. These CRTs compare patients' health before and after a new treatment. In stepped-wedge CRTs, all groups start with the standard treatment. Then, each group switches to the new treatment at a specific time during the study. By the end of the study, all groups are receiving the new treatment.

In stepped-wedge CRTs, group characteristics, such as how clinics follow up with patients, can affect how well a treatment works. It's hard to figure out if changes in a patient's health are due to the treatment or group characteristics.

In this study, the research team wanted to improve how to plan and analyze stepped-wedge CRTs for studying the effect of treatments.

What did the research team do?

The study had two parts. In the first part, the research team looked at ways to measure how well treatments work in stepped-wedge CRTs. The team also looked at how to analyze data from stepped-wedge CRTs in ways that account for group characteristics.

In the second part, the research team looked at which statistical methods got accurate results when using data from stepped-wedge CRTs. The team first used a computer program to create test data that looked like

data from a stepped-wedge CRT. The team created the test data using nine scenarios; each scenario had a different set of conditions. For example, the number of patient groups varied across each scenario. Using the test data, the team compared six statistical methods for analyzing data from stepped-wedge CRTs.

The research team also created a statistical program to help plan and analyze stepped-wedge CRTs.

What were the results?

The research team found that all six statistical methods worked for some scenarios; none worked for all nine scenarios.

The new statistical program helps calculate the number of patients that should be in a study.

What were the limits of the project?

The study focused on stepped-wedge CRTs that group patients at a single level, such as by clinic.

Future research could extend the methods to stepped-wedge CRTs that group patients at multiple levels, such as patients receiving care at clinics within a region.

How can people use the results?

Researchers may be able to use these results to improve the design and analysis of stepped-wedge CRTs.

To learn more about this project, visit www.pcori.org/Heagerty375.