PROJECT INFORMATION

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Statistical Methods to Measure Treatment Effect Using Observational Data Versus Randomized Trials

Principal investigator

Issa J. Dahabreh, MD, MS

Organization

Brown University

What was the research about?

A randomized trial is one of the best ways to learn if one treatment works better than another. Randomized trials assign patients to different treatments by chance. But they are not always affordable, and they take a long time to complete.

When randomized trials aren't possible, researchers can use observational studies to learn how treatments work. In observational studies, researchers look at what happens when patients and their doctors choose the treatments. Traits such as age or health may affect treatment choices. These traits may also affect patients' responses to treatment, making it hard to know if the treatment or the traits affected the patients' responses.

Some study designs and statistical methods may help address this problem and make results from observational studies more useful. These methods can give researchers more data about whether treatments work and how the same treatment can affect groups of patients differently.

The research team conducted three studies to test different methods of designing and analyzing observational studies. They wanted to know if observational studies that used these methods produced results similar to randomized trials.

What were the results?

In study 1, the research team found that the methods for designing and analyzing observational studies had

results similar to randomized trials for how well a treatment worked.

In study 2, the research team used statistical methods to divide patients into groups based on their risks of getting an illness. The methods helped show how patients who had high risk responded differently to the same treatment.

In study 3, some methods were better than others in finding out how the same treatment affects patients differently.

What did the research team do?

In the first study, the research team used methods to design observational studies to look like randomized trials. For example, they used data from health records to assess the effectiveness of two medicines for high blood pressure. Using different data from 11 randomized trials, the team then analyzed how effective the medicines were. The team compared the results from the observational study with the randomized trial results.

In the second study, the research team used randomized trial data to figure out patients' predicted risk of getting an illness. Then the team looked at how groups of patients with different levels of predicted risk responded to the same treatment.

In the third study, the research team used a computer program to create data. They used the data to compare the different methods of finding out how the

same treatment can affect patients differently. They also compared the methods using real data.

What were the limits of the study?

The methods used in this study may work only when data include patient traits such as age and other health problems.

Future research could test these methods using data from different data sources on different health problems and treatments.

How can people use the results?

Researchers can consider using these methods to design and analyze studies using observational data when randomized trials aren't possible.

To learn more about this project, visit www.pcori.org/Dahabreh257.