PROJECT INFORMATION

March 2021

Developing New Methods for Comparing Treatments in Case-Control Studies

Principal investigator

Goodarz Danaei, MD, MS, DSc

Organization

Harvard T.H. Chan School of Public Health

What was the project about?

A randomized controlled trial, or RCT, is the best way to compare how well different treatments work to improve patients' health. In RCTs, researchers assign patients to treatment groups by chance. But RCTs aren't always an option due to high costs or ethical concerns. In these cases, researchers use other types of study designs such as

- **Cohort studies**, which look at patients' data over time to see how a treatment affects the risk of a certain health event, such as a heart attack
- **Case-control studies**, which compare data from patients who did and didn't have a certain health event

These designs often use data from health records to compare treatment results. In these studies, researchers use statistical methods to make results more like results from RCTs. Current methods work well for cohort studies but not for case-control studies.

In this study, the research team created and tested new methods and a guide to analyze case-control studies so that results would be more like results from an RCT.

What did the research team do?

The research team talked with researchers who do case-control studies. The team used what they learned to create the new methods and a guide. The guide explained how to use the new methods for case-control studies. The team then asked the researchers for feedback to improve the guide.

To test the guide and new methods, the research team used health records from patients receiving care at health systems in Washington State. The team did a case-control and cohort study to look at whether statins, a medicine to lower cholesterol, helped prevent heart attacks. Next, the team compared the results from previous RCTs of statins to results from

- The new methods for case-control studies
- Current methods for case-control studies
- Current methods for cohort studies

What were the results?

The guide had information on how to mimic an RCT with case-control data and how to set up data for analysis. The research team also created a computer program to analyze case-control data.

Compared with current methods for case-control and cohort studies, the new methods for case-control studies got more accurate results.

What were the limits of the project?

The new methods only work when researchers collect data at multiple time points. The research team tested the new methods using data for one treatment for heart attacks.

Future research could test if the new methods work for other health problems or treatments.

How can people use the results?

Researchers can use the guide and computer program when designing case-control studies.

To learn more about this project, visit www.pcori.org/Danaei363.