PROJECT INFORMATION

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Developing Guidelines for the Analysis of Randomized Controlled Trials in Real-World Settings

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Organization

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What was the project about?

In randomized controlled trials, or RCTs, researchers assign patients by chance to different treatments to compare the benefits and harms. In RCTs, researchers have a high level of control over how patients receive treatment. RCTs often take place in research clinics with staff who monitor how patients follow treatment plans.

Pragmatic RCTs, or pRCTs, take place where patients typically receive treatment, such as a regular clinic. pRCTs can help capture the real-world effects of treatment but determining whether a treatment works can be hard in pRCTs. Also, no clear guidelines exist about how to collect and analyze data from pRCTs. Some kinds of analysis are better for helping researchers focus on what's important to patients.

In this study, the research team created guidelines for collecting and analyzing data in pRCTs so that results reflect what matters to patients and researchers.

What did the research team do?

First, the research team asked patients and researchers what results from pRCTs matter to them. The team had three group discussions with adult patients. The team also did five interviews and an online survey with pRCT researchers. Then the team reviewed results from published pRCTs. They looked at the gaps between results that matter to patients and results generally reported in pRCTs.

From this information, the research team developed four recommendations about how to design pRCTs. One focused on reporting per-protocol effects. Per-protocol effects are what would happen in a trial if all patients closely followed the treatment plan. Based on this recommendation, the research team looked closely at per-protocol effects. The team used data from six previous RCTs to show how three statistical methods can estimate per-protocol effects. Then the team applied the new methods to data created using a computer program. The test data mimicked data in pRCTs.

The research team used what they learned to create guidelines for pRCTs. Patients, doctors, and researchers provided feedback about the guidelines.

What were the results?

The research team created 14 guidelines for collecting and analyzing data from pRCTs, including

- How to choose results that matter to patients and researchers
- How to collect data to predict if someone will drop out of a pRCT
- How to predict if a patient will follow the treatment plan

What were the limits of the project?

The study included patients from the Boston area and researchers in the United States. Results may differ for

patients and researchers in other places. The research team looked at only certain types of pRCTs, such as those with yes-or-no outcomes like death. The guidelines may not apply to other types of pRCTs.

Future research could test and refine the guidelines for other types of pRCTs.

How can people use the results?

Researchers can use the results when planning pRCTs.

To learn more about this project, visit www.pcori.org/Hernan270.