New Methods and Software for Designing Adaptive Clinical Trials of New Medical Treatments

What was the research about?
Research studies called clinical trials test treatments to see if they are safe and effective for patients. When designing clinical trials, researchers must plan to include enough patients with different traits for the study to have accurate results. Once the study starts, researchers must follow the plan. Sometimes, early results from a trial show that a group of patients with a certain trait may have more benefits or harms from the treatment than other groups. For example, the treatment may not work for patients with a history of heart disease. In the standard trial design, researchers can't change the plan to stop enrolling these patients once the trial starts.

In this study, the research team compared the standard trial design with more flexible approaches known as adaptive enrichment designs. These designs set up rules that allow researchers to change the study plan. For example, if early results show a treatment doesn't work for patients with heart disease, researchers can stop enrolling these patients in the trial. The team compared the trial designs using data from four completed trials.

What were the results?
Compared with the standard designs used in the example trials, the adaptive enrichment designs
- Had more accurate results
- Needed fewer patients in the overall trial
- Needed more patients to show that a treatment had a benefit or harm for a specific group

What did the research team do?
The research team developed different methods for designing clinical trials. To test these methods, the team used information from the four completed studies. Then the team created a computer program to help researchers choose a study design for future clinical trials. The program compares the standard and adaptive enrichment designs to predict
- How many patients would need to take part in a clinical trial to have accurate results
- How many patients with a specific trait would need to take part in the trial to show that the treatment has a benefit or harm for that group

What were the limits of the study?
The study tested the methods with only four example trials. The adaptive enrichment designs allowed researchers to look at the effect of treatment on only one specific group of patients in the clinical trial.

Future research could develop study designs that focus on the benefits or harms for more than one group of patients.

How can people use the results?
Researchers can use the computer program to choose a trial design and plan for the number of patients needed to take part in the trial.

To learn more about this project, visit www.pcori.org/Rosenblum144.