New Statistical Methods to Compare the Effectiveness of Adaptive Treatment Plans

What was the project about?
Treatments for patients with long-term health problems such as diabetes or arthritis often change over time. Such plans are called adaptive treatment plans as doctors adapt treatment based on the patient's health problem and response to earlier treatments. Adaptive treatment plans are common, but the methods to assess how well a plan works may not always provide accurate results. To know which plans are best for patients, researchers need better methods to compare these adaptive plans.

In this study, the research team developed and tested a new statistical method and looked at whether it could more accurately compare adaptive treatment plans.

What did the research team do?
The research team first developed a new statistical method called GPMatch. Using a computer program, the team created test data. The team used the test data to look at how well GPMatch worked compared with current statistical methods.

Then the research team used GPMatch with real data from patients' health records. The team compared two types of adaptive treatment plans for children with polyarticular-course juvenile idiopathic arthritis, or pcJIA. The two types of plans were

- **Early combination plan.** Patients start two types of medicines at the same time after diagnosis.

- **Step-up plan.** Patients start with one type of medicine and then start the second type later.

Using GPMatch, the research team checked which plan improved children's health after 6 and 12 months.

Patients, parents of patients with pcJIA, doctors, and patient advocates helped develop and test GPMatch.

What were the results?
With the test data, GPMatch was more accurate than current statistical methods in measuring the effects of adaptive treatment plans.

Using GPMatch, the research team found that the early combination plan was better at improving health for patients with pcJIA than the step-up plan. Both plans improved quality of life.

The research team developed a computer program to help other researchers use GPMatch.

What were the limits of the project?
For studies with data on more than 5,000 patients, GPMatch takes more than one day to run on the computer. The research team tested the new methods using data from patients with pcJIA from one health center. Results may differ with data from other centers or for other health problems.

Future research could work on ways to use the methods with more complex data. Studies could also test these methods using data from other clinics or for other health problems.
How can people use the results?
Researchers can use the new statistical methods to compare treatment plans that change over time.

To learn more about this project, visit www.pcori.org/Huang316.