PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE
RESEARCH SUMMARY

PROJECT INFORMATION

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Using Statistical Methods to Predict Treatment Response Based on Patients' Likelihood of Having Benefits or Side Effects from the Treatment

Principal investigator
David Kent, MD, MS

Organization
Tufts Medical Center

What was the research about?

Sometimes, people with a certain trait respond differently to the same medicine. For example, women might, on average, benefit more from a medicine than men or have worse harmful side effects. If researchers group everyone together when they analyze study data, they may miss learning about which people are more likely to benefit or have harmful side effects from a medicine.

In this study, the research team grouped people based on their likelihood of having benefits or side effects from a certain medicine. The team used data from research studies of two medicines:

- **Pioglitazone** treats diabetes in patients who have had a stroke but can increase the risk of breaking a bone.
- **Anthracycline** treats women with breast cancer but can harm the heart.

What were the results?

Grouping data on people based on their likelihood of having benefits or side effects from each medicine showed differences in how the medicines affected them. The group of patients with a low risk of breaking a bone was more likely to benefit from pioglitazone. Patients in the group less likely to benefit from anthracycline had more heart problems.

What did the research team do?

The research team used statistical methods to analyze the data from two studies. The studies looked at benefits and side effects of medicines for different groups of people. The groups included patients with a

- Low or high chance of benefitting from the medicines
- Low or high chance of having side effects from the medicines

In each group, the team then looked at the actual benefits and side effects patients had from the medicines.

What were the limits of the study?

The research team used data from past studies. The studies didn't include some health data, such as history of fracture and smoking, that could affect how well the medicines work or their side effects. Having this information might have changed how the team grouped the patients. Some groups of people may have been too small for the statistical methods to work well.

Future research could further explore ways of grouping patients to study differences in the effects of medicines.

How can people use the results?

Researchers can use the statistical methods to better understand how likely patients are to have benefits or harms from medicines.

To learn more about this project, visit www.pcori.org/Kent240.