New Statistical Methods to Assess How Patients with Different Traits Respond to the Same Treatment

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What was the project about?

Patients may respond differently to the same treatment due to individual traits such as age or gender. Knowing how different traits can affect a patient's response to treatment can help doctors and patients make better treatment decisions. For example, this information can help doctors know what types of cancer medicines work better for certain patients. This project focuses on improving the methods that researchers use to compare how treatments work for different patients.

In this project, the research team developed and tested a statistical method called random forests, or RF. RF is a way to analyze data using a technique called machine learning. In machine learning, computers use data to learn how to perform different tasks with little or no human input. Many types of RF methods exist. The team compared multiple RF methods to learn how well the methods would work to find out how patients with different traits respond to the same treatment.

What did the research team do?

The research team tested the different RF methods using data created with a computer program. They compared the RF methods with each other, with non-RF methods, and with methods not based on machine

learning to see which were more accurate and precise. The team also tested the methods using real data.

A group of doctors, other researchers, and staff from a public health department helped design the study.

What were the results?

The research team found that the RF methods worked better than methods not based on machine learning to show how different patients respond to treatments. Some RF methods were more accurate and precise than others. In some cases, non-RF methods worked as well as the RF methods.

What were the limits of the project?

The research team only looked at one type of machine learning method. Future research could look at machine learning methods not used in the study and test their use in treatment decisions in real-world settings.

How can people use the results?

Researchers can use RF methods in clinical research to learn how patients with different traits may respond to treatments.

To learn more about this project, visit www.pcori.org/Feaster267.