Engaging Patients and Clinicians to Determine Design Features of N-of-1 Trials

Principal investigator

Nathalie Moise, MD, MS

Organization

Columbia University Medical Center

What was the research about?

Medical studies usually focus on learning how a treatment works for most people. These studies have strict rules about who can take part. Patients with multiple health problems, such as high blood pressure and diabetes together, often can't take part because in such medical studies having multiple health problems can cause patients to respond differently to treatments than people with fewer health problems.

N-of-1 studies are a different way to design research. People with multiple health problems can take part in N-of-1 studies. An N-of-1 study has only one patient. Doctors try different treatments with that person and look for the treatment that works best.

In this study, the research team wanted to know

- Whether patients and doctors thought N-of-1 studies could be useful
- Which health problems patients thought N-of-1 studies could be helpful for
- Which features, such as the length of the study or how much time it took to track treatment results, affected whether patients wanted to be in an N-of-1 study

What were the results?

Patients and doctors thought N-of-1 studies could be useful to identify the best treatments for patients. Patients were concerned about taking part in studies that were costly or that took a lot of time. Patients worried that the studies could interrupt their

treatments and their visits with other doctors. Doctors had concerns about the trial process and rules and about how the studies could affect their management of patients' conditions. Surveys found that patients thought N-of-1 studies would be good for studying treatment of health problems such as high blood pressure, diabetes, and depression. Patients wanted to take part in studies that took less than five minutes a day to track treatment results.

Who was in the study?

The research team held group discussions with 54 patients with multiple health problems who speak English or Spanish and with 24 doctors who treat patients with multiple health problems. All participants were from New York.

Then the research team surveyed 1,002 patients from across the United States with at least two of six health problems. In the first survey, 89 percent of patients were white, 8 percent were African American, and 1 percent were Asian; 4 percent were Hispanic. The average age was 56, and 57 percent were women. In the second survey, 79 percent of patients were white, 13 percent were African American, and 5 percent were Asian; 15 percent were Hispanic. The average age was 57, and 56 percent were women.

What did the research team do?

The research team used findings from the group discussions to design the first survey. The survey asked patients which health problems the N-of-1 studies would be most useful for. Then, using the results of this survey, the team designed a second

survey that asked patients which features they liked in different types of N-of-1 study designs.

Patients with multiple health problems, doctors, researchers, and web designers helped design the study.

What were the limits of the study?

Patients and doctors in the group discussions came from two health systems in New York. The patients had specific health problems. The results may be

different in other locations or with patients who have other health problems.

Future research can look at the views of other doctors and patients on N-of-1 studies. Studies could test the types of N-of-1 studies that patients like.

How can people use the results?

Researchers can consider the results when designing N-of-1 studies.

To learn more about this project, visit www.pcori.org/Moise185.