

EDITORIALS

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Can patient centered outcomes research improve healthcare?

We believe it can; now we should put it to the test

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The Patient-Centered Outcomes Research Trust Fund (PCORTF) was established in 2009 to support research that is funded, designed, carried out, and put into practice within a culture of patient centeredness.¹ A legislatively mandated review in 2017 of the “adequacy and use of funding” will be used to determine whether PCORTF funding levels should be continued or adjusted after its authorization runs out in autumn 2019. As 2017 approaches, researchers ought to begin taking stock of our work in patient centered outcomes research: how well are we are doing and what might we do better?

In the linked paper (doi:10.1136/bmj.h3786), Xian and colleagues present a solid example of how to refocus research on questions, outcomes, and approaches that could help patients and clinicians to make better healthcare decisions. The team studied prescribing of warfarin for patients with ischemic stroke who were discharged from hospital between 2009 and 2011 with persistent or paroxysmal atrial fibrillation or flutter.² This study was funded by the Patient-Centered Outcomes Research Institute (PCORI) in 2013.

This team’s preliminary work with patients resulted in prioritization of an outcome that actually matters to people with ischemic stroke: time “alive at home, without recurrent stroke, and without being hospitalized for complications.”² The team also measured major adverse clinical events (MACE), as well as other clinical indicators as secondary outcomes. Their continued work with patients as co-investigators assured involvement throughout the research process, allowing patients to participate in research design and provide input into the statistical analysis plan. The study focused on patients who were older and at higher risk than those in previous efficacy studies and was conducted in settings where patients go for care.

Treatment settings included 1487 hospitals with expertise in stroke, or nearly a quarter of the 6300 hospitals that treat adults in the United States.³

Since its establishment in 2010, PCORI has funded hundreds of studies through its regular call for proposals.⁴ This work reflects a large nationwide effort in what was essentially a start-up venture only a few years ago. Measurement of performance in this effort should track along three streams.

Firstly, we need an ongoing and public inventory to explain how trust fund dollars earmarked for patient centered outcomes research have been spent. PCORI already provides a great deal of inventory information on its website. Each quarter, the organization publishes an online dashboard of funded projects, results, publications, and other milestones.⁴ The most recent dashboard shows that many more studies are on their way to reporting in the next few years. The dashboard also accounts for a range of other activities, including milestones reached in PCORnet, a program designed to establish new infrastructure for ongoing patient centered research. Inventory assessments of other trust fund programs are also needed, such as the inclusion of patient centered outcomes research in the portfolios of the National Institutes of Health and the Agency for Healthcare Research and Quality.

Secondly, procedural assessments are needed to examine whether investigators are getting the principles of patient centeredness right. This will involve comparing patient centered work against quality standards. PCORI publishes a range of standards on its website, including a rubric that can guide newcomers through the tasks of involving patients and other stakeholders in a study.⁵ A recently published framework lists four principles of patient centeredness that could help to ensure that we are getting it right: patient centered outcomes research should be relevant, pragmatic, feasible, and participatory.⁶

Finally, we need to evaluate whether patient centered outcomes research is making a difference to healthcare quality and outcomes. We have seen a growing commitment to patient centeredness in part because we believe it can help us to create new evidence that is relevant to patients and other decision makers, research methods that are more transparent to decision makers, and findings that are usable in a wider range of settings.⁷ This belief needs to be put to the test with carefully designed evaluation protocols.

The team led by Xian has performed exceedingly well in all of these streams. The project was reported on schedule and according to a pre-specified analytic plan. The study gets the basics of patient centeredness right: investigators took on a question and measured outcomes that matter, designed a

pragmatic study to answer the question, and used a robust and participatory approach in conducting the research. Whether this study results in improved uptake of warfarin among older and higher risk patients is yet to be seen. However, we can already see how it might change patient-clinician dialogue about treatment options. Patients leaving the hospital may be better able to remember that taking warfarin could help them to stay healthy and at home longer. Many, on the other hand, will not understand or remember what MACE stands for.

Recent investments in patient centered research have been large and rapidly scaled up. On one level, the United States has embarked on this course because we believe it is the right thing to do; it may help us to meet the important goal of supporting broad participation in the use of public dollars. However, we also do it in the belief that it will change healthcare by supporting patients and clinicians to make better decisions. It is time now to take stock of how much patient centered research we are doing, how well we are doing it, and whether it improves healthcare.

Competing interests: I have read and understood the BMJ policy on declaration of interests and declare the following interests: I have given a paid talk about patient centered research to a conference organized by American Leaders.

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