

How Comparative Effectiveness Research Became Patient-Centered Outcomes Research

by Eugene Rich, MD, FACP
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Note: For this column, Dr. Rich responds to questions posed by colleagues at Creighton on his recent publication in the *Journal of Comparative Effectiveness Research* entitled "[Past as prologue: how comparative effectiveness research became patient-centered outcomes research.](#)"

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Question 1: It is pretty clear that the transition from Comparative Effectiveness Research (CER) to Patient-Centered Outcomes Research (PCOR) was motivated by political considerations and prior concerns that CER might result in lack of availability of some diagnostic and therapeutic measures based on cost effective analyses. But why is the Patient Centered Outcomes Research Institute (PCORI) established as a private non-governmental organization (albeit with its governing body appointed by the GAO) instead of establishing it as an Institute within NIH?

Dr. Rich's Answer: The debate over the "placement" of the CER (now PCOR) function relative to federal research agencies was lengthy and multifaceted. A full exposition would delve into some of the intricacies of constitutional law as well as the arcana of competing stakeholder concerns. However establishment as an Institute at the NIH was never a widely debated option, given the NIH's longstanding role in basic science discoveries and in "proof of concept" research. During the early years of the CER debate, then-Director Zerhouni emphasized the NIH's role in translational rather than in effectiveness research saying "we don't do Coke versus Pepsi."

AHRQ actually has had substantial infrastructure supporting CER since 2003 and was thus considered a key option for housing it as recounted in my 2009 JGIM article "The Policy Debate over Public Investment in Comparative Effectiveness Research." Indeed the version of CER in the House Health Care Reform legislation placed the CER research center at AHRQ. During the debate over health care reform, there were numerous concerns regarding government "bureaucrats" taking away valuable treatment options because they were too costly. These voices were not persuaded that NIH and AHRQ are research agencies whose staff and grantees are scholars focused on producing good science. In this climate even the highly objective and independent US Preventive Services Task Force (USPSTF) administrated by AHRQ was accused of "rationing." The USPSTF released a cautionary statement in 2009 regarding the balance of goods and harms associated with mammography screening for women under 50. Some advocates claimed that the rationing-obsessed government bureaucrats were no longer content "killing Granny", now they were going after Mommy too! Whether the House and Senate could have found an acceptable compromise between the placing of CER at AHRQ (the House version) and the Senate's creation of an independent outside government research entity (PCORI) can't be known. As recounted in my "Past to Prologue" piece, the Senate version prevailed because of the Democrats' loss of the Massachusetts Senate seat.

Question 2: Given that it is generally acknowledged many existing clinical guidelines are inadequately supported by evidence, what will PCORI do to assure better evidence-based guidelines?

Dr. Rich's Answer: Interestingly, as part of the various compromises to allay stakeholder anxiety, PCORI is legislatively forbidden from describing its research findings as recommendations or guidelines. Nonetheless, the hope is that by producing research evidence that informs real world clinical decisions relevant to the point of care, PCORI will greatly improve the evidence base that professional associations and other bodies can use to develop practice guidelines.

Question 3: Has PCORI established research priorities and, if so, what are they? What research is it currently funding?

Dr. Rich's Answer: The establishment of research priorities is of course a "hot button" issue for various stakeholders. Despite the independent nature of PCORI, it would seem the governing board has been slow to engage this difficult challenge. Nonetheless, PCORI now has an active process for identifying specific PCOR priorities and has recently identified 5 specific topics, with more to follow shortly. PCORI encourages broad participation in its topic priority process. More information can be found at <http://www.pcori.org/research-we-support/priorities-agenda/getting-specific/>

PCORI has also relied on the broader research community to identify priority research questions and has begun to fund projects in a variety of topic areas. These projects have been selected through a new peer review process that assesses both the scientific merit of a research proposal and the likely usefulness of results to real world decision makers. More about the current PCORI grant awards can also be found at the PCORI website. <http://www.pcori.org/funding-opportunities/pfa-awards/>

Question 4: The article suggests that the move from CER to PCOR intended a prominent move toward patient empowerment. Is it correct that CER would not necessarily have fostered patient capacities? Are there good reasons to think that implementing PCOR will better empower patients?

Dr. Rich's Answer: Clearly the shift in terminology represented a desire by some policy makers to further emphasize the focus of this research on providing patients the information they and their clinicians need for point of care decisions. The advocates for evidence based medicine and CER also intended the research would be developed and used in this way. Indeed the House version of CER legislation, while not adopting the "patient centered" title, emphasized the need for the research conducted to be guided by the patient and clinician perspective. I have another commentary coming out in JCER that explores these issues further, considering both the benefits and the challenges of building the patient perspective into effectiveness research. This might be a good topic for another edition of "Inside the Beltway."



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