

## **TITLE PAGE**

**Title:** Comparative Effectiveness and Patient-Centered Outcomes Research: Enhancing Uptake and Use by Patients, Clinicians and Payers

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**ABSTRACT** (120 words max, currently 115)

Evidence from comparative effectiveness research (CER) and patient-centered outcomes research (PCOR) studies are increasingly available in the literature. However, there remain opportunities to better integrate that evidence into decision-making. An invitation-only conference held in January 2017, titled “Comparative Effectiveness and Patient-Centered Outcomes Research: Enhancing Uptake and Use by Patients, Clinicians and Payers,” sought to identify and discuss both gaps in the uptake and use of CER/PCOR, and approaches to enhance the uptake and use of CER/PCOR evidence by patients, clinicians, and payers. In this article, we summarize the conference proceedings, and highlight the themes and recommendations that resulted from the sessions. This paper also introduces other articles in this issue of *JCER* from that conference.

**Keywords:** Comparative effectiveness research, Patient-centered outcomes research, Knowledge translation, Dissemination, Implementation

## **BODY OF ARTICLE**

### **Background**

There is a growing consensus among stakeholders in the United States that we must shift the emphasis of the health care system from one based on volume to one based on value. To guide value-based decision making, patients, caregivers, clinicians, and policy-makers need access to, and the ability to use, evidence that compares the relative merits of available treatment options, and be adequate for their decision-making needs.

Comparative effectiveness research (CER), which has gained attention over the past decade, offers part of the solution. The purpose of CER is to facilitate decision-making and improve health outcomes by developing and disseminating evidence to patients, clinicians, and other decision-makers, about which interventions are most effective for a given application. CER emphasizes understanding the comparative benefits and harms of treatments as used in actual clinical practice. This evidence is increasingly seen as a building block for shared decision making that takes into account the trade-offs between the benefits and the potential harms of treatment options as viewed by the patient and their families as well as health care professionals.

Early efforts to support CER focused primarily on the development of research methods and on the training of researchers. For example, as early as 2006, the Agency for Healthcare Research and Quality (AHRQ) held conferences about methods in CER and published related resources.<sup>6-8</sup> With the advent of “patient-centered outcomes research” (PCOR) and the Patient-Centered Outcomes Research Institute (PCORI) in 2010, such efforts expanded.<sup>9</sup> Importantly, funding to train CER researchers was made available by

AHRQ, PCORI, the National Institutes of Health (NIH), the Pharmaceutical Research and Manufacturers Association (PhRMA) Foundation, and other organizations in the form of individual and institutional training awards, conference grants, center grants, and contracts. The momentum behind PCOR was a logical consequence of CER, introducing another facet to understanding value that recognized studies about group-based (average) treatment benefits are inadequate to guide clinical decision making at the individual-level. Not only do we seek to understand clinical heterogeneity so that treatment effectiveness can be better tailored to individual characteristics, but the patient's perspective – their preferences for different treatments – are a crucial part of the value proposition in health care.

Today, we have significant resources and expertise in the conduct of CER and PCOR. As a result, CER/PCOR evidence is rapidly accumulating in the literature. However, there remains a need to better integrate CER/PCOR evidence into decision-making.<sup>11-15</sup> A major limitation may be that potential users of this evidence need to be educated on the strengths and weaknesses of CER and its place within the hierarchy of evidence levels. A recent survey identified user application of CER and its role in decision-making among the top educational needs.<sup>11</sup> Other surveys reported that the majority of health care professionals were not adequately prepared to use CER/PCOR.<sup>13</sup> Others have noted that insufficient attention is paid to communication about CER/PCOR evidence to end-users.<sup>12,14</sup> Together, studies point to the need for better education of users of CER/PCOR, understanding of the barriers to using CER/PCOR evidence, design of effective strategies and tools to ensure uptake and use of CER/PCOR by clinicians, patients, payers/policy-makers. Lastly, there is a need to teach patients, payers, and policy makers how to use CER results to achieve value when selecting among health care products and services.

To begin to address these issues, the PhRMA Foundation, together with the Academy of Managed Care Pharmacy (AMCP), hosted an invitational conference in Washington, D.C., on January 26-27, 2017. Attended by 70 experts and opinion leaders representing clinicians, patients, funding agencies, and payers - the conference was titled “Comparative Effectiveness and Patient-Centered Outcomes Research: Enhancing Uptake and Use by Patients, Clinicians and Payers.” The 70 invited participants represented key “users” of CER/PCOR evidence: academic organizations, professional associations, healthcare provider groups, insurance companies and other payer organizations, patient advocacy groups, government agencies, research groups, pharmaceutical and biotech manufacturers, and others in the CER/PCOR field. Potential attendees were nominated by the organizing committee, or identified by web searches, or recommended by officers of foundations, professional societies/organizations and patient advocacy groups, based on interest in the aims of the conference and ability to represent one of stated stakeholder viewpoints. The overarching goal of the conference was to advance the use of CER/PCOR by patients, clinicians, and other decision-makers by identifying the needs of users and promoting best practices for the education of users of CER and PCOR. This issue of *JCER* includes several articles that stem from that conference. A full description of the conference presentations and participants is available at the PhRMA Foundation website at <http://www.phrmafoundation.org/events/>.<sup>16</sup>

### **Conference Outcomes and Recommendations**

The collective discussion and recommendations from the conference focused on the needs and gaps in the uptake and use of CER/PCOR evidence by patients, clinicians, and payers;

and suggested approaches to enhance the uptake and use of CER/PCOR evidence by patients, clinicians, and payers. The outcomes of the three groups – clinicians, patients, and payers – are summarized below. More detail is provided in the paper by Law and others also in this issue.

The clinician group felt that lack ability to efficiently access and use CER/PCOR evidence. They also identified other barriers common to implementation science (i.e., not just specific to CER/PCOR) that are important – like knowledge, attitudes, beliefs, and self-efficacy. The perceived low quality of CER/PCOR studies was another concern, as was the lack of evidence in many clinical areas/indications. Strategies discussed included incorporating CER/PCOR into clinical decision support systems and other tools that make it part of routine activities in care.

The patient group suggested that greater understanding is needed of what matters most to patients as it relates to their treatment, and to align CER/PCOR research with that. CER/PCOR research needs to be translated in a way that can be accessed by patients and disseminated to platforms that patients use. The language used to discuss CER/PCOR evidence needs to be understandable to patients. The group suggested that a publically available resource be created that summarizes CER/PCOR evidence in lay terms. Last, we should find ways to help patients reconcile fragmented information and adoption of CER/PCOR across the different providers with whom they interact.

The key barriers to uptake and use of CER/PCOR from the payer perspective are timeliness of the availability of results of CER/PCOR studies (e.g., not available when decisions are being made) and the robustness of the data (not directly transferable to the payer's population, or too many gaps in the research evidence available). Lack of resources on CER/PCOR, and lack of education on how to use CER/PCOR data are also barriers. Last, the

clinical nuances that are important in decision-making don't get incorporated into CER/PCOR evidence. Strategies proposed included better organization and coordination of CER/PCOR evidence (perhaps incorporating it into existing registries), education programs – especially for regulators, the availability of high quality summaries for CER/PCOR data that can be presented to decision-makers, and the need for a CER/PCOR trained person on formulary committees.

### **Future Perspective**

Results of comparative effectiveness studies, real-world evidence, and patient-centered outcomes research are being published in the medical literature at an accelerating rate. Over the next 5-10 years this type of evidence will grow exponentially, and has enormous potential value in influencing health care decisions and ultimately the outcomes of care. This conference sought to focus identify strategies to enhance the use and uptake of this type of evidence. . Several themes emerged from the stakeholder groups. Clinicians have limited time to stay abreast of the rapidly expanding medical literature. They need easy access to trusted sources that synthesize up to date evidence appropriate to their patient population, and can be incorporated into clinical decision support systems and other tools for routine care. CER/PCOR research should takes into account what patients with the condition(s) identify as most important, and be translated in a way that is accessible to patients and disseminated via platforms that patients use. Payers need CER/PCOR studies that are timely enough to inform decisions to be made, and address issues of generalizability. High quality summaries and education programs on CER/PCOR could assist formulary committees.



The questions of whether health care will benefit from the CER/PCOR movement will depend on how well the evidence that is generated is aligned with the needs of patients, clinicians, and policy-makers; whether it can be conveyed and incorporated into decision making in a way that is readily available and actionable with minimal effort; and whether that evidence will actually lead to improvements in health, quality of care, and better value. To make significant inroads, there needs to be a sustained effort towards knowledge translation and transfer among key stakeholders in health care that takes perhaps an even broader view of the issues than examined in this conference, and should be a key priority in the coming years.

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