Continuous Patient Engagement in Comparative Effectiveness Research

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THE GOAL OF COMPARATIVE EFFECTIVENESS RESEARCH (CER) is to provide patients, their advocates and caregivers, health care professionals, federal officials, policy makers, and payers with evidence-based information to make informed health care decisions.[1,2] Previously, CER studies were designed by researchers and had relatively little input from patients. Patient engagement has rapidly gained acceptance as crucial to the successful translation of CER for all interested parties.[3] Experiences with patient engagement in research, including community-based participatory research,[4] suggest that success hinges on patients being interested and emotionally involved in the research question and understanding their role in the CER process.

Evaluation of information from the Agency for Healthcare Research and Quality and the Patient-Centered Outcomes Research Institute suggests that CER may be enhanced through continuous patient engagement. The framework for doing so, as proposed in this Viewpoint, reflects pragmatic experiences and observations in which patient engagement has helped to shape and translate CER for patients and health care professionals; however, the framework needs to be tested empirically (FIGURE). This proposed 10-step process for CER describes how patient engagement might guide CER toward patient-centered outcomes research and offers suggestions for the process and purpose of patient engagement across the 10 steps. Each step has different purposes, advantages and disadvantages, and implications for time and resources. The proposed framework is intended to span the entire "life cycle" of a CER project.

At the first step, patients could help identify understudied CER topics. By soliciting input from diverse patients, the process could generate a broad array of topics, from which a more narrow focus could be achieved by framing specific research questions. When designing a potential framework for a specific CER question, patients would provide a "reality check," indicating the extent to which the proposed framework reflects their personal experiences.

When CER involves primary data collection, patient input could help determine the best practices for data collection, provide input about the proposed content of the data collection tool, and participate in pilot testing survey items. Traditionally, patient feedback is infrequently used during the development of the analysis plan. However, this framework proposes that patients could assist in helping to define or categorize variables even if they do not have training in research methods. When reviewing and interpreting results, patients could reflect on whether results are plausible and believable, what other factors should be considered, and how results may vary across subgroups of patients.

In the translation phase, patients could identify which results are easy or difficult to understand. If the results do not affect patients or are counterintuitive, CER findings will not be translated into medical practice. Patients could also offer suggestions for how best to explain study findings to other patients. Patients could help determine the best dissemination strategies, provide dissemination channels, and craft specific messages targeted to patients who will benefit most.

Thoughtful consideration should be given to determining which approach would best elicit the patient's perspective at each particular step. For example, during the early stages of research, it may be most beneficial to engage patients through patient forums or telephone conversations to solicit potential topics of importance. As research progresses to protocol development, involving patients in stakeholder meetings could provide an opportunity for robust discussion regarding which outcomes to assess, populations to include, and treatment options to compare.[5] Input on the patient experience also could be obtained from indirect means of engagement. For example, using online patient forums in which groups of patients talk about their disease may be a source of valuable information provided the process adheres to ethical standards of protection of human research participants.[6,7]

When creating the conceptual framework, collecting data, and specifying the analysis plan, more in-depth information and patient input may be required. Methods of engagement such as in-person meetings, focus groups, and individual patient interviews could allow patients to give more detailed input. Patients could assist in framing the message, creating plain language summaries, targeting audi-

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Figure. Enhancement of Comparative Effectiveness Research (CER) Through Continuous Patient Engagement

Step in CER Process	Purpose of Patient Engagement
Topic solicitation	■ Identify topics that are important to patients, caregivers, and the community ■ Propose topics to be investigated
Prioritization	■ Solicit feedback on relevance and priority of topics ■ Discuss the urgency of addressing topics
Framing the question	■ Ascertain questions' relevance and usefulness ■ Assess "real-world" applicability
Selection of comparators and outcomes	■ Identify comparator treatments of interest ■ Identify outcomes of interest ■ Incorporate other aspects of treatment
Creation of conceptual framework	■ Provide a "reality check" ■ Verify logic of conceptual framework ■ Supplement with additional factors not documented in the literature
Analysis plan	■ Verify importance of factors and variables ■ Ascertain whether there is a good proxy for a specific concept ■ Inquire about potential confounding factors
Data collection	■ Determine best approaches for data collection (eg, trial, registry, medical charts) ■ Assist with selection of data sources
Reviewing and interpreting results	■ Assess believability of results ■ Suggest alternative explanations or approaches ■ Provide input for sensitivity analysis
Translation	■ Interpret results to be meaningful ■ Document which results are easy or difficult to understand ■ Indicate which results are counterintuitive
Dissemination	■ Facilitate engagement of other patients ■ Help other patients to understand findings

ences for dissemination efforts, and critiquing draft materials (eg, patient guides). However, these encounters should be supplemented by targeting patient advocacy organizations, media, social media, and patient navigators (peer counselors who help patients navigate through the complex health care system) to link patients with appropriate resources.[8] It would be important to communicate how patient input will be used so that patients remain engaged even when CER results do not answer all their questions.

Involving patients' perspectives would most likely make CER more meaningful and, therefore, potentially more useful. Nonetheless, including patients could pose substantial challenges. For example, in prioritizing research questions or selecting outcomes, different patients will want different questions answered and different outcomes to be measured. One solution could be to select patient representatives who are connected with a greater patient community (eg, through advocacy organizations) and able to discuss broad concerns of interest to diverse patients, not just for themselves or their special interests. It also is important to address real and potential conflicts of interest. Ultimately, principal investigators would be wise to develop partnerships with patients and their physicians, advocates, and caregivers, but should maintain authority in developing protocols to ensure scientific rigor. Patient engagement is a dynamic process and the relative amounts of time spent and costs incurred will depend on several factors, including the techniques, duration, and intensity of patient engagement. Despite the potential advantages and that there likely will be efficiencies over time as researchers learn how to engage patients in CER, patient engagement is likely to increase the cost and length of time to plan and conduct CER. As such, complete and meaningful patient engagement may be affected by the availability of funding and resources.

Best practices for patient engagement in CER will continue to evolve. A systematic approach for eliciting patient input could serve to promote a more patient-centered approach to CER despite many challenges. Incorporating input from a variety of patients would help provide insights for producing CER results that go beyond "average treatment effects" and produce results that are applicable to specific patient subgroups.[9] Engaging patients in CER will require additional effort compared with the traditional research process; however, if done well, engaging patients in this process could provide patient-centered outcomes research evidence that is useful and informative at the level of clinical practice and decision making.

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