

# Patient Forward:

## Comparing ICER and PCORI's Use of Patient Feedback

### Introduction and Purpose

With the growing emphasis on value-based care, healthcare stakeholders in the United States (US) are increasingly looking to organizations that produce comparative effectiveness evaluations to make better-informed healthcare decisions in a resource-constrained environment. However, many of these assessments do not sufficiently incorporate patient perspectives, thereby limiting their use in decision making. In particular, the Institute for Clinical and Economic Review (ICER) has been criticized for leaving critically important patient feedback out of its evaluations. ICER's recent updates to its value assessment framework address many of these concerns, including the addition of a Patient Engagement Program, but these changes have been incremental at best. Conversely, the Patient-Centered Outcomes Research Institute (PCORI) specifically focuses on topics that are most important to patients and encourages patient involvement throughout the entire research process.

This issue brief highlights differences and similarities between PCORI and ICER approaches to incorporating patients in research and the impact on the results by looking at assessments conducted by each organization in 2 therapeutic areas: rheumatoid arthritis (RA) and sickle cell disease (SCD).

### Key Findings



**PCORI provides specific examples of how patient perspectives are incorporated throughout the research process and emphasizes the importance of including patient-centered outcomes in research.**



**ICER asserts that patient-centered outcomes are important to include in its research yet provides few specific examples for how and when patient perspectives are incorporated into its analyses, although this appears to be improving.**

### Background on PCORI and ICER

While PCORI and ICER profess to improve patient care, they have notably different approaches to incorporating patient perspectives in their research. PCORI is a federally funded, nonprofit, nongovernmental organization with a federal mandate to conduct and fund comparative clinical effectiveness research. As a mission-based organization, PCORI seeks to involve patients and other stakeholders from the start, which leads to more useful results that will be more likely to be used in clinical practice. During the initial application phase for research awards, PCORI encourages research grant applicants to describe how they will espouse 6 patient engagement principles: reciprocal relationships, co-learning, partnerships, transparency, honesty, and trust.<sup>1</sup> Patients and other stakeholders are invited to contribute meaningful feedback to help identify, measure, and evaluate patient-centered outcomes. PCORI's goal is to leverage patient-centered research that will provide reliable and useful information to help people make informed healthcare decisions and improve patient care and outcomes.

ICER is an independent nonprofit funded through private sources, including payers, pharmaceutical manufacturers, and nonprofit foundations. ICER's mission is to provide an independent source of evidence and analysis of effectiveness and value to improve the quality of care that patients receive.<sup>2</sup> By objectively evaluating the value of different healthcare interventions, ICER seeks to provide recommendations on the treatments that best represent "high-value care" so patients will benefit from these treatments at a price they can afford. As part of its 2020–2023 value assessment framework, ICER uses patient input (eg, through direct feedback or online surveys) to inform its research objectives, and patients can participate in this process in several ways: provide input on new topics, comment on draft scoping documents, comment on draft evidence reports, and attend public meetings to share feedback.



### “Drug Therapy for Early Rheumatoid Arthritis: A Systematic Review Update”

PCORI’s RA report included a systematic review of “early” (ie, within 1 year of diagnosis) RA in adults to evaluate the benefits and harms of several RA treatments.<sup>3</sup> For this project, PCORI partnered with the Agency for Healthcare Research and Quality (AHRQ) to engage in evidence synthesis and distribute the effectiveness research to patients and providers. The study utilized PCORI’s patient-centered standards, which focus on the engagement of individuals affected by RA, their caregivers, clinicians, and other relevant stakeholders.<sup>4,5</sup>

During the planning phase, patients and other stakeholders participated in a virtual workshop facilitated by PCORI and helped to develop the research protocol, examine the evidence base, and finalize the key research questions. In conjunction, PCORI identified the benefits and harms of the drug therapies in multiple patient subgroups affected by RA. Key research questions focused on patient-reported symptoms, functional capacity, quality of life, and patient subgroups. Patient-centered outcomes data were incorporated to compare RA drugs among different patient subgroups whenever these data were available. As part of its conclusions, **PCORI noted that more “patient-centered research is needed with appropriate use of patient-reported outcomes and other patient-generated health data so that results are truly reflective of patient preferences and desires.”** PCORI partnered with AHRQ to disseminate the final report to patients and ensured the report was made available at no cost through PubMed Central.



### “Targeted Immune Modulators for Rheumatoid Arthritis: Effectiveness & Value”

ICER’s assessment focused on the comparative clinical effectiveness and value of targeted immune modulators used to treat RA. As part of the scoping process, ICER consulted with clinical experts, patients, manufacturers, and other stakeholders to understand what outcomes are important to patients. Specifically, during the open input period, ICER noted it received feedback from several stakeholders but did not describe its patient engagement approach or any of the specific comments received by patients. Additionally, **the scope of the assessment was “developed with extensive and critically important input from several patient advocacy organizations,” but the findings do not appear to have influenced any of ICER’s analyses.** ICER stated that patients and advocacy organizations suggested specific patient-reported outcomes be incorporated as part of its Population, Intervention, Comparison, Outcomes, Timing, Setting (PICOTS) framework; however, it is unclear if any comments were related to ICER’s approach when assessing comparative value or if ICER considered incorporating these comments.

While ICER acknowledged it gained insights from discussions with patients and patient groups during the public comment periods (eg, delayed diagnosis; difficulty finding tolerable treatment; substantial financial burden; few quantitative, patient-centric measures of treatment success), these issues were not further addressed in the report. ICER conducted 2 patient surveys in collaboration with a patient advocacy organization, but these perspectives were only briefly summarized and did not appear to have influenced the results (eg, network meta-analysis or long-term cost-effectiveness). During the public meeting, patients noted several problems with ICER’s comparative effectiveness evaluation, including the lack of inclusion of patient-reported data, which would more accurately reflect what patients experience in the clinical setting and make the report more patient-friendly. Finally, although ICER emphasized that identifying appropriate patient subgroups can better inform treatment response and selection of appropriate medications, it did not highlight any specific findings (or lack thereof) on subgroups as part of its final report.



### “Evaluating a Program to Improve Patient Experiences After Discharge From the Hospital—The PArTNER Study”

The objective of the PCORI study was to compare a navigator intervention to usual care to evaluate the effectiveness and desirability of a program that supports a patient up to 60 days post discharge as part of a randomized clinical trial.<sup>6</sup> The study focused on the ability of the interventions to reduce avoidable readmissions and address concerns of patients who feel abandoned during the transition from the hospital to their home. Before the study began, patients were interviewed on their experiences around the time of hospitalization, hospital discharge, being at home following discharge, and follow-up care. PCORI used the results of these interviews to refine the navigator program used in the study to tailor the intervention to patients’ recovery needs. PCORI then evaluated the navigator’s ability to improve overall patient experience post discharge and examined its effectiveness in different patient subgroups, highlighting the need to focus on core social issues facing the sickle cell patient population.

***PCORI conducted its SCD study in partnership with patients and caregivers***, which helped shaped the study and prioritize the design of the intervention program to focus on the patient experience. Specifically, patients shaped the PCORI study eligibility criteria and recruitment/retention plan, as well as affirmed the need for an intervention arm that was developed by patients. Because the navigator intervention did not affect the primary outcomes—which were based on patient preferences—in the overall population, PCORI did not advocate for implementing the navigator intervention as a transitional care service to improve anxiety or informational support during hospital-to-home transitions. An easy-to-read summary describing the results and their uses was posted on the PCORI website 90 days after its release of the final research report.<sup>7</sup> PCORI also collaborated with patient groups to disseminate the findings from this research.

### “Crizanlizumab, Voxelotor, and L-Glutamine for Sickle Cell Disease: Effectiveness and Value”

In one of ICER’s more recent evidence reports, the organization assessed the comparative clinical effectiveness and value of treatments for SCD, including crizanlizumab, voxelotor, and L-glutamine.<sup>8</sup> During ICER’s open input periods, SCD patients submitted comments that were used to draft the introductory narrative in the final evidence report, ***but it was unclear if and how the feedback was used to develop the overall scope of the assessment.*** Similar to PCORI, ICER collaborated with 2 patient advocacy organizations to conduct an online survey of patients to fill evidence gaps regarding quality of life and productivity, which were used as inputs in its cost-effective analyses.

Although ICER indicated that patient feedback influenced the development of the PICOTS criteria, some of the suggested patient-centric outcomes were not analyzed further, even when these data were available in the literature. Some of the patient perspectives from the survey were included in the economic model, but most of the survey results were described qualitatively in the “Potential Other Benefits and Contextual Considerations” section of the report.

## Prioritizing Patient Engagement

**In terms of acknowledging and using patient input, PCORI both shows and tells, while ICER mostly tells.** Both ICER and PCORI engaged with patients and patient advocacy groups throughout the research process, but PCORI more transparently included patient perspectives that directly influenced how the research was conducted with a distinct impact on the results. PCORI also provided specific examples of how patient perspectives were incorporated. In contrast, ICER asserted that patient-centered outcomes and patient heterogeneity were important considerations but provided few details on how and when patient feedback was incorporated into its analyses.

Additionally, while both organizations used patient surveys as part of their research, PCORI directly incorporated this input to help formulate the research protocol, design research questions focused on identifying patient-centered outcomes, as well as emphasized the importance of identifying patient subgroups, while ICER primarily captured these perspectives qualitatively. ICER's approach to using patient feedback as part of its evaluations appears to be improving, as demonstrated by its use of some patient survey data as inputs to its cost-effectiveness analyses. Nevertheless, ICER did not analyze or further address some of the suggested patient-centric outcomes even when those data were available.

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