

The Data Mine: Value Assessment Organizations Should Learn from the Experts on Patient Engagement

Dr. Steven Pearson, founder and president of the Institute for Clinical and Economic Review (ICER), recently wrote that no clear framework exists to engage patients in medical research. In *A proposed framework for patient engagement throughout the broader research enterprise*, published in the Journal of Comparative Effectiveness Research, Dr. Pearson acknowledges that patient engagement is an important component of medical research, but he fails to recognize years of accomplishments in the field. This misleading premise ignores rigorous patient-centered outcomes research and evolving development of evidence-based frameworks for patient engagement.

Stakeholders have long recognized the need for evidence-based information to make informed health care decisions and understood that existing comparative effectiveness research was too often designed by researchers with relatively little input from patients. Dr. Daniel Mullins, a known expert in the field of patient engagement, [explained in 2012](#) that experience has taught us that success “hinges on patients being interested and emotionally involved in the research question and understanding their role in the [comparative effectiveness research] process.” In fact, there is a tremendous amount of [literature on patient engagement](#) to support institutions that are genuinely committed to doing it well.

Unfortunately, Dr. Pearson did not cite some of the most well-known and respected literature on patient engagement. One review of stakeholder engagement practices yielded the development of a 7-Item Stakeholder Engagement Reporting [Questionnaire](#) in 2014 that is widely used and cited by experts in the field. In another 2014 review of existing literature, other researchers recognized the need to get beyond “[tokenistic](#)” patient engagement. To do so, a group of experts worked with stakeholders to identify a [new taxonomy](#) for engagement in patient-centered outcomes research. As the field evolves, we are gaining real-world examples of how patient engagement [improves research](#).

ICER recently announced the creation of a new Vice President of Patient Engagement position, and this field of research will be useful in developing a patient engagement framework fit for use in value assessment. Over the years, patients have [shared](#) with ICER the value of their lived experience in assessing the value of new treatments and the value of engaging patients in a manner similar to patient centered outcomes research. Yet, Dr. Pearson’s article reflects an ivory tower perspective that patients need to be trained professionals in the areas of their engagement in order to be suited for engagement in value assessment. The literature on patient engagement does not support this conclusion. What it does show is that “[for patient engagement to be successful, adequate institutional support, interprofessional training and collaborative interdisciplinary partnerships need to be in place](#).” Therefore, it will be important

for Dr. Pearson and ICER to not only proactively engage patients, but also to strengthen their own skills to be better partners.

As value assessment organizations seek to develop a patient engagement framework that is fit for their purposes, institutions such as the [Patient-Centered Outcomes Research Institute \(PCORI\)](#), the [Patient-Driven Values in Healthcare Evaluation \(PAVE\) Center](#), the [Innovation and Value Initiative](#) and the Food and Drug Administration provide valuable models.

Since 2010, PCORI has been explicitly required by its authorizing statute to articulate the limitations of its research, and its funded projects must assure that patients are at the table in both the design and dissemination of research so that its strengths and limitations are effectively understood and communicated. [PCORI](#) has learned from experience the importance of continuous and genuine partnerships, strategic selection of stakeholders, and accommodation of stakeholders' practical needs. In developing its own framework, PCORI consulted with its Patient Engagement Advisory Panel to publish a [patient engagement rubric](#) in 2014 which was later updated in 2016 and again in January 2020. It describes specifically how to engage patients throughout the research process, from planning the study through dissemination of the results. It provides a foundation for cultural change throughout the research infrastructure to center on patients.

It is important to also recognize the work of the PAVE Center, which is a Center of Excellence in Patient-Driven Value Assessment dedicated to promoting value-based decision-making through a diverse multi-stakeholder collaboration and engaging patients from an extensive network of partners to build technical expertise in patient-centered outcomes research, education, and dissemination. PAVE has also partnered with [National Health Council \(NHC\)](#), an organization with expertise in developing educational tools for patients and patient advocacy organizations, to train patients and ensure these stakeholders have the tools they need to engage productively in the research process.

Additionally, at the forefront of developing a model for patient-centered value assessment, the [Innovation and Value Initiative](#) has a mission to advance the science and improve the practice of value assessment. That includes a commitment to patient partnership and dedicated resources for it.

With regard to regulatory decisions, the Food and Drug Administration (FDA), most notably through Patient-Focused Drug Development, has included the patient perspective in FDA Advisory Committee meetings since 1991. Patient engagement in research at the FDA has [grown and evolved](#) over the past thirty years, using a [prescriptive framework](#) for the patient role in research.

It is concerning that Dr. Pearson implies that patients should meet certain criteria to engage in health coverage decisions that are so heavily influenced by value assessments conducted by the organization he leads. Patients have [long fought](#) for a seat at the table in payer-level decision-making and bring to the table their personal experience fighting for coverage. Patients are

uniquely qualified to identify the unintended consequences of insurer policies when implemented in the real world. This has been a consistent mantra of the patient-centeredness movement as patients have fought for a role not only in insurance decisions, but also in the development, implementation and evaluation of [alternative payment models](#).

Dr. Pearson also touches on the importance of patient engagement in developing clinical practice guidelines. Indeed, patient engagement is a key component of clinical practice guideline development with important implications. [Existing studies](#) show the importance of engaging patient stakeholders in guideline development. Therefore, clinician specialty societies and their patient partners should be referenced as the experts on strategies for engaging patients in guideline development.

As value assessment organizations seek to develop their own patient engagement frameworks, we urge them to engage with advocacy organizations representing patients and people with disabilities that are well-versed on the lifetime implications of a disease or condition, including the clinical diversity of patients and their experiences navigating insurance and coverage policies. Patients' relationships with healthcare stakeholders – ranging from the life sciences industry, researchers, health systems, and payers – gives them a broad perspective. It is vital for any value assessment organization to understand how patient preferences are identified, how outcomes that matter to patients are incorporated into research, and how that research then translates (or not) into real world health decisions.

We are pleased that since publication of this article, ICER has chosen to create a position within its organization dedicated to patient engagement. Doing so provides ICER an opportunity to learn from existing engagement experiences, such as within PCORI, the PAVE Center, and the FDA, and to dig deep into the literature on best practices for patient engagement.