October 18, 2019

Steven D. Pearson, MD  
President  
Institute for Clinical and Economic Review  
Two Liberty Square, Ninth Floor  
Boston, MA 02109


Dear Dr. Pearson,

On behalf of the Cancer Support Community (CSC), an international nonprofit organization that provides support, education, and hope to people impacted by cancer, we appreciate the opportunity to respond to the request for public input for the Institute for Clinical and Economic Review’s (ICER) 2020 Value Assessment Framework. As the largest direct provider of social and emotional support services for people impacted by cancer, and the largest nonprofit employer of psychosocial oncology professionals in the United States, CSC has a unique understanding of the cancer patient experience. Each year, CSC serves more than one million people affected by cancer through its network of over 45 licensed affiliates, more than 170 satellite locations, and a dynamic online community of individuals receiving social support services. Overall, we deliver more than $50 million in free, personalized services each year to individuals and families affected by cancer nationwide and internationally.

Additionally, CSC is home to the Research and Training Institute (RTI)—the only entity of its kind focused solely on the experiences of cancer patients and their loved ones. The RTI has contributed to the evidence base regarding the cancer patient experience through its Cancer Experience Registry, various publications and peer-reviewed studies on distress screening, and the psychosocial impact of cancer, and cancer survivorship. This combination of direct services and research uniquely positions CSC to provide valuable patient and evidence-informed feedback on ICER’s value assessment frameworks.

We recognize the efforts that ICER has taken to better include patients and incorporate patient feedback. We have worked with ICER to ensure that the cancer patient voice is heard and understood and we are appreciative of the outreach offered by ICER staff. Yet, there is much more to be done. In the patient engagement guide, ICER states that their core mission is to “produce information that helps stimulate dialogue on how to achieve fair pricing, fair access, and future innovation.” As such, it is critical for ICER to understand the potential implications of their assessment for patient access. Value assessments influence the ability of patients to access...
the most appropriate therapies. As these therapies can improve quality of life, extend survival, or prove lifesaving, we believe that patients must truly be at the center of your work.

We respectfully submit the following comments and look forward to the opportunity to engage in future discussions for the purpose of securing a healthcare system that provides sustainable access to both high-quality and high-value care for all patients.

**Patient Engagement**

In section 1.1 of the ICER revised framework, ICER states that evidence assessment is “one component of ICER’s broader effort to provide mechanisms through which all stakeholders and the general public can engage in discussions on how best to use evidence as the foundation for a more effective and sustainable health care system.” We align with ICER on this point and support efforts to incorporate patients into discussions regarding a more effective and sustainable health care system.

On page 3, lines 360-365, ICER states:

> Even with its population-level focus, however, the ICER value framework seeks to encompass and reflect the experiences and values of patients. Representing the diversity of patient outcomes and values in a population-level framework is difficult because there will always be an inherent tension between average findings in clinical studies and the uniqueness of every patient. There will also always be diversity in the way that patients view the balance of risks and benefits of different treatment options.

We appreciate ICER’s acknowledgement of this dichotomy between value assessment and patient values, needs, and preferences. We volunteer to work with ICER to be world-leaders in finding ways to better incorporate the patient voice into value assessments.

We also appreciate ICER’s update to the framework to debrief with patient groups after a report is complete. We look forward to better understanding the formal debriefing process and volunteer as a resource to help ICER finalize that process.

It is also important for ICER to recognize the challenges facing patients and patient advocates as they seek to engage in ICER’s processes. Access to the evidence is a critical component of engagement. However, stakeholders who are not affiliated with an academic institution or who do not have the means to access academic databases or purchase expensive journal subscriptions are many times unable to review the data necessary to participate in the value assessment process. While we recognize that the limitations posed by the publishing system are not due to actions by ICER, we are seeking solutions to this barrier.

**Real World Evidence**

On page 4, section 3.1, ICER “reaffirms use of existing real-world evidence.” We appreciate this commitment and encourage ICER to work with patient advocates to clearly outline the types of real-world evidence that will be accepted for use during value assessments. We agree with ICER’s statement on page 5, lines 409-411 that “…randomized controlled clinical trials have
their own limitations and are often inadequate to address all questions relevant to assessments of comparative clinical effectiveness.” We also agree with ICER in that patient-reported outcome studies can provide evidence not always captured in clinical trials. As noted above, CSC has a Cancer Experience Registry of over 14,000 participants and we would like to work with ICER to leverage the data from the Registry and determine how such sources can be meaningfully integrated into ICER’s value assessments. In addition to Registry findings, our team of researchers can hold focus groups and conduct mixed methods research during each phase of assessment to ensure that ICER is incorporating the concepts most salient to patients living with the specific disease. We believe that patient level data must be incorporated. If this level of data is not readily available, it IS ICER’s responsibility to secure it in order to be fully informed.

**Sustainable Access to High-Value Care for All Patients**  
The stated goal of ICER’s value assessment framework is to help the United States evolve toward a health care system that provides “sustainable access to high-value care for all patients.” ICER calculates incremental cost effectiveness from the health system perspective. Specifically, ICER applies two distinct elements - namely *Long-Term Value for Money* and *Short-Term Affordability* - to derive high-value care for all patients. Cost-effectiveness from the health system perspective is one endpoint, but cannot be the primary driver to determine high-value care for *all* patients.

As noted by CSC in previous comments, it is critical to clearly delineate the difference between the concept of “value” as it pertains to medical treatments and devices compared to an assessment based primarily on the financial implications of those treatments and devices. The concept of value, if truly intended to provide sustainable access to high-value care for *all* patients, must be broader than cost-containment and budget impact. Patients seek care for different diseases, diagnosed at different stages of progression, with different states of underlying physical and mental health, and with different life goals and perspectives. Given the unique physical, mental, and psychological make-up of each individual patient, there is no one-size-fits-all value framework to determine high-value care for all patients. Patients make different determinations regarding their care based on any number of variables unique to them. Therefore, we would be pleased to partner with ICER to ensure the inclusion of more real-world evidence such as that gained from our 14,000 Cancer Experience Registry participants.

**Cost per Quality-Adjusted-Life-Year and Equal Value of Life Years Gained**  
Notwithstanding ICER’s blanket statement that the cost per quality-adjusted-life-year (QALY) will continue to be the primary measure of incremental cost-effectiveness, CSC urges ICER to recognize the limitations of the QALY and work towards inclusion of a more patient-centered measure. In 2018, CSC published a study that found that three-quarters of cancer patients did not believe that the QALY is a good way to measure value in healthcare and were concerned that decision makers were utilizing the QALY in ways that could negatively impact their access to care (Franklin et al., 2018).

Allen et al. (2017) note that the QALY may not capture the full range of components necessary for individual decision-making. The QALY only captures some of the benefits created by a health care intervention and does not always capture the full health or well-being of patients (International Society for Pharmacoeconomics and Outcomes Research, 2017). It also doesn’t
incorporate preferences regarding the weight given to health gain and has been criticized for being discriminatory against certain patient groups such as people with disabilities (International Society for Pharmacoeconomics and Outcomes Research, 2017). The QALY operates from the premise that a more desirable health state is deemed more valuable (Weinstein, Torrance, & McGuire, 2009). However, we agree with Weinstein, Torrance, and McGuire (2009) when they stated that a critical question is “value to whom?” There remain ethical, conceptual, and operational concerns regarding its use (Prieto & Sacristán, 2003).

Throughout its value assessment framework, ICER references the importance of high-quality evidence. Yet, the QALY is derived from assumptions made by individuals often lacking any experiential basis upon which to measure either the burden or the quality of life of someone confronting a particular condition, advanced age, or a disability. Furthermore, these assumptions are often inherently discriminatory and have negative consequences on the access to care for those who are ill, elderly, or living with a disability.

We also do not believe that the Equal Value of Life Years Gained (eVLYG) is an appropriate tool and includes many of the same challenges as the QALY. The eVLYG does not account for improvement of quality of life and other important components of value. Although these types of tools have long been utilized, we support the movement to utilize more transparent, patient-centered tools such as multi criteria decision analysis.

**Patient Experience Data**

As mentioned above, ICER’s value assessment framework is from the health system perspective, with the two economic elements used to support this perspective being long-term value for money and short-term affordability. Despite a ‘sustainable access to high-value care for all patients’ being the ultimate identified goal, there is no mention of patients in any of the domains contributing to this goal. The domain titled “other benefits or disadvantages” appears to offer a mechanism for collecting stakeholder information. CSC recognizes and appreciates ICER’s efforts in its 2020 value assessment framework to seek stakeholder input for the next year in seven delineated potential “other benefits or disadvantages” and five delineated “contextual considerations.” However, we remain concerned that these components are included in reports after the assessment has been made. While it has been communicated to us that they play a critical role in decision making, this is not clear in the assessment reports.

CSC urges ICER to follow the lead of the U.S. Food and Drug Administration (FDA) and increase the opportunities for patients to submit valuable data and to require ICER to incorporate patient data in its efforts to better define value. The FDA has made significant inroads in requesting and incorporating patient experience data in the drug development process. Under the 21st Century Cures Act, the FDA has embarked on an aggressive plan to systematically collect and use key information about patient experiences beginning with the early phase of drug development and translation into a validated measurement set. The FDA recognizes that patients are in a unique position to contribute to an understanding of benefit and risk in the development of prescription medications, including methodological approaches to develop and identify what is most important to patients with respect to burden of disease, burden of treatment, and the benefit and risk in the management of disease. To ensure the patient experience is secured in the process, the FDA is required to issue draft and final versions of guidance documents over a five
year period. Title III of the 21st Century Cures Act is described as giving the FDA an opportunity and directive to advance the science and efficacy of medical innovation to address critical unmet needs of patients.

The Belmont Report also emphasizes the importance of including patient experience data in the research process. In its discussion of basic ethical principles for research involving human subjects, the Belmont Report identifies beneficence as an obligation for both individual investigators and society at large to: (1) do no harm and (2) maximize possible benefits and minimize possible harms. With regard to particular projects, the Belmont Report states “investigators and members of their institutions are obliged to give forethought to the maximization of benefits and the reduction of risk that might occur from the research investigation.” Similarly, in the case of scientific research in general, the Belmont Report provides “members of the larger society are obliged to recognize the longer term benefits and risks that may result from the improvement of knowledge and from the development of novel medical, psychotherapeutic, and social procedures.” It is essential that ICER both increase opportunities for patients to submit valuable data and require patient data be incorporated in its efforts to better define value, whereas beneficence is an obligation, not merely “other benefits or disadvantages” and/or “contextual considerations.”

**Additional Dimensions of Value**

ICER states on page 13, lines 608-610 that methods for the quantification of value dimensions highlighted by Lakdawalla et al. (2018) are “viewed by many health economists as too exploratory for routine incorporation into assessments.” The “value of hope” is given as one example. As noted in CSC’s letter to ICER regarding the proposed Value Assessment Methods for “Single or Short-Term Transformative Therapies,” ICER states that they “believe there are significant risks or double counting within the QALY or within existing “other benefits” or “contextual considerations” that ICER already includes as part of its value framework.” ICER also notes that such additional elements of value are all “unidirectional” and would all “add” to treatments, and none have negative scores that would help balance out added value within an opportunity cost framework for determining the cost-effectiveness threshold.” Finally, it is noted that methods for measuring additional elements of value are “not mature” and “further research is needed before it can be determined how to measure them.” As a result, ICER proposes that “no quantitative integration of additional elements of value” will be included in the value assessments framework for the assessment of SSTs. However, patient input will be sought regarding the “value of choice among treatments with a different balance and timing of risks and benefits.” We do not believe that the stated “value of choice” appropriately captures the concept of the “value of hope.” We disagree that the concept of the value of having the choice among treatments with different balance and timing of risks and benefits captures the same concepts as the value of hope. We are currently validating a new tool called the “Valued Outcomes in the Cancer Experience” or the VOICE measure. This project began as a study of what patients hope for and has evolved into a measure of their values and how much control they believe they have over what they consider most valuable. We believe that this measure could be useful to ICER and propose a meeting to discuss potential collaboration on this topic.

In conclusion, CSC’s recommendations are as follows:
Value Assessment

- Limit inclusion of budget impact in the final value assessment, reporting it as just one endpoint.
- Recognize ongoing value including late and long-term benefits and effects.
- Incorporate real world evidence whenever possible and partner with patients, patient advocates, and other experts to ensure the inclusion of such evidence from registries, qualitative analyses, patient studies, etc.
- Include the full range of health care costs and cost offsets in the final assessment.
- Measure and account for alternative systems costs or offsets—such as treatment every 3 weeks vs. once per week which allows for fewer disruptions to work, home, and family life and reduced costs as they relate to out-of-pocket expenses and transportation.
- Conduct value assessments only when adequate data (which is inclusive of patient experience data) are available.
- Eliminate the use of the QALY and evLYG and rely on alternative measures such as multi-criteria decision analysis.
- Ensure that “other benefits or disadvantages” and “contextual considerations” play a key role in assessments with a specific focus on patient experience data. Communicate how voting panels incorporated these concepts into their decision making.
- Organize assessment by subpopulations, to be defined with significant patient input.
- Provide not only health system perspective but also societal perspective, both of which should be informed by patient input.

Transparency

- Ensure transparency at each point of the methodological process including not only the specifics of the method but also the rationale, assumptions, and literature to support those decisions.
- Ensure transparency with all resources used in the development of evidence reports.

Revisions

- Revise assessments within two months of new evidence becoming available (including new options for treatment both in terms of treatment types, medications available, and administration options) and previous information becomes outdated and/or reviews of past assessments on a regular basis to ensure timeliness.
- Provide transparent and specific guidance for assessment updates to reflect the evolution of scientific evidence and introduction of new treatments and devices.

Patient Input

- Partner with patient advocates at each stage of the assessment process, particularly at the beginning stages so that they may help inform assumptions and key concepts.
- Allow for a more flexible process by which patients can access all of the relevant information and apply weights that are most appropriate for their circumstances and preferences.
- Include patients and multidisciplinary experts (throughout the entire value assessment process and voting) who have experience and knowledge of that specific disease state.
- Incorporate a specific number of diverse patient representatives who represent a broad range of voices and experiences. They should be involved at each step of the value assessment process including (but not limited to) the evidence report develop and voting.
• Allow for weights to be assigned based on user preferences and assign higher levels of value to components that are most important to patients.
• Partner with patient advocates to create and disseminate information necessary to understand and participate in the value assessment process.
• Describe when input was utilized and when it was discarded, and the reasons for each.
• Work to ensure that outcomes reflect patient experiences and preferences.
• Utilize existing patient registries and survey databases to explore and incorporate patient experience data. Engage in additional data collection if data doesn’t exist.
• Include costs that are representative of the price most relevant to the patient.
• Change ICER comment period to 90 days to allow for sufficient time for patient and patient advocate feedback.

Implementation
• Understand the potential and applied use of value assessments by a variety of stakeholders regardless of intended use and audience.

Dissemination
• Work with patient advocacy groups and patients to disseminate results in a manner that is clear and understandable for all stakeholders.
• Provide clear instructions for implementation and warnings against unintended use.

In closing, thank you for the opportunity to submit these comments. We welcome the opportunity to engage in further discussions with you to ensure the patient experience is valued and all patients have access to high-quality health care. If you have questions regarding our comments, or if we can serve as a resource, please reach out to me at Efranklin@cancersupportcommunity.org.

Sincerely,

Elizabeth F. Franklin, MSW
Executive Director, Cancer Policy Institute
Cancer Support Community Headquarters

References


how-can-they-meet-the-challenge/


