March 27, 2017

Steven D. Pearson, MD, MSc, FRCP
President, Institute for Clinical and Economic Review
One State Street, Suite 1050
Boston, MA 02109 USA

RE: Institute for Clinical and Economic Review: Background and Scope Document on Poly ADP-ribose polymerase (PARP) Inhibitors for Ovarian Cancer: Effectiveness, Value, and Value-Based Price Benchmarks

Dear Dr. Pearson,

On behalf of the Cancer Support Community (CSC), an international nonprofit organization that provides support, education, and hope to cancer patients, survivors, and their loved ones, we appreciate the opportunity to respond to the request for comments regarding the Institute for Clinical and Economic Review’s Background and Scope Document on Poly ADP-ribose polymerase (PARP) Inhibitors for Ovarian Cancer: Effectiveness, Value, and Value-Based Price Benchmarks. 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 44 licensed affiliates, more than 120 satellite locations, and a dynamic online community of individuals receiving social support services. Overall, we deliver more than $40 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—the only entity of its kind focused solely on the experiences of cancer patients and their loved ones. The Research and Training Institute 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 value frameworks such as ICER’s Value Framework.

CSC is pleased to offer the following comments on this background and scope document:

**Unrealistic Timeframe to Respond**
The timeframe to read, consider, and respond to ICER documents continues to pose a challenge to many organizations and individuals who wish to respond. Starting with the open input period and timeframe to comment on background and scope documents, three weeks is simply not
sufficient to devote the appropriate amount of time and resources to review these documents. Further, ICER is currently calling for comments on the 2017-2018 Value Framework, Patient Engagement Guide, and Manufacturer Engagement Guide. As we draft comments in response to several of those documents, the timeframe to comment on this background and scope document becomes even more onerous.

As noted in previous comment letters to ICER, all comment letters should be guaranteed to be made public and should remain on the ICER website. Currently, ICER removes comments on the background and scope document once they are considered and the final draft is released. It is also unclear why PDFs are not an acceptable format in which to submit comment letters.

CSC recommends the following:
1. Provide ample time (at minimum 60 days) to respond to any document included in the value assessment process.
2. Documents available for comment should not overlap. However, if this is unavoidable an additional time should be allowed for comments.
3. Allow stakeholders to submit comments in PDF form.
4. Post all stakeholder comments to all documents on ICER’s website in perpetuity.
5. Allow for comment documents of any length.
6. Incorporate comments from stakeholders into documents or provide rationale why feedback was not incorporated into final documents.

**Process for Patient Representation**
ICER states that this background and scope document was developed with input from key stakeholders, however, a transparent process must be in place to involve patients in every step of the value assessment process.

The inclusion of patients is supported by numerous entities including: 1) The National Health Council (2016) which highlights the domains of a “meaningful patient-centered value model” including patient partnership, transparency to patients, inclusiveness of patients, diversity of patients/populations, outcomes patients care about, and patient-centered data sources; 2) PhRMA which states that value assessment frameworks should ensure a strong role for physicians and patients who bring essential expertise and perspective and should play a central role; and 3) The National Pharmaceutical Council which states that interested stakeholders should be involved in the assessment process to represent all perspectives. In their *Health Affairs* article, DuBose and Westrich (2017) agree that as patients are the recipients of health care services, frameworks should incorporate components of value that are important to them. Not including that perspective can “lead to assessments that over- or under- estimate a therapy’s value” (DuBose and Westrich, 2017).

We ask that patients be included throughout the decision making process and for inclusion in panels and activities as they are the most knowledgeable about the full patient journey. We look forward to working with ICER to ensure that this integration of patient feedback is consistently implemented. We also appreciate ICER’s creation of a guide to engagement for patients and plan to comment separately on that document.
CSC recommends the following:

1. Include a sufficient number of diverse patient representatives (throughout the entire value assessment process) who have experience and knowledge of that specific disease state. For example, patients who have had ovarian cancer should be commenting on ovarian cancer treatments specifically.

2. Provide patient representatives with information in a transparent, timely, and understandable manner. CSC would be pleased to work with ICER to pilot such information.

3. Obtain patient feedback PRIOR to the release of the scoping document.

**Concept of Value**

In this background and scope document, ICER identifies both cost-effectiveness as well as the “potential budgetary impact of each regimen over a 5-year time horizon…” It is critical to clearly delineate the differences between the concept of “value” as it pertains to medical treatments and devices, and assessment based primarily on the financial implications of those treatments and devices. ICER identifies the “primary anchor” of the value framework, which is “long-term value for money.” This is bolstered by the complementary perspective of “short-term affordability.” Although cost-effectiveness is a reasonable endpoint in the value discussion, the use of budget impact is inappropriate.

DuBois and Westrich (2017) note that value assessments should be “based on the net benefits and net costs at an individual patient level.” They state further that “budget impact is a dollar amount pertaining to a particular payer or other economic perspective…but it does not indicate whether that expenditure is a good or poor value.” As evidenced by Cohen, Anderson, and Neumann (2017), the budget constraint requirement “can break the proportional relationship” between value and cost effectiveness. The authors show that the “budget constraint imposes a more stringent (lower) price on PCSK9 inhibitors than cost-effectiveness considerations.” Further, they suggest that ICER should continue reporting budget impact but that it should not be reflected in the value-based price (Cohen, Anderson, & Neumann, 2017). Neumann and Cohen (2017) point to cost effectiveness as a more appropriate value indicator because it provides a common scale for comparing products, although they also note that cost effectiveness itself is an incomplete tool and other salient factors are necessary to consider. The National Pharmaceutical Council (n.d) states that “budget impact is not a measure of value” and CSC agrees that “it can inform the use of what they are paying, but now about what they are paying for—value.” Combining budget impact assessments with value assessments is misleading.

While the short- and long-term financial impacts of drugs and devices are clearly important to consider, there are other aspects of value that are critical to include in any comprehensive “value assessment.” Meaningful patient and stakeholder representation is vital to all institutions determining value, including ICER. Any value framework cannot be a one-size-fits-all approach and the concept of value must be broader than budget impact and cost containment. Patients
make different determinations regarding what they value most throughout their illness and service journeys. This is evident in incremental gains for patients who are coping with particularly deadly diseases.

In 2016, the National Health Council released the Patient-Centered Value Model Rubric and CSC was pleased to serve on the working group to develop that document. The National Health Council (2016) states that “value is individualized, disease-dependent and evolving”, and highlight the importance of “outcomes patients care about” which are those that patients have “identified as important and consistent with their goals, aspirations, and experiences.” The National Pharmaceutical Council (n.d.) states that the “measurement of value should include a broad array of benefits that are important to patients and society.” Further, we agree with the National Pharmaceutical Council (n.d.) which suggests that weights should be included in any value framework in order to accommodate varying user preferences. This methodology is also utilized in the DrugAbacus tool developed at Memorial Sloan Kettering. However, Neumann and Cohen (2017) state that even though there are value frameworks that are designed to incorporate user preferences, the final assessment may be inconsistent with those preferences. It is critical that user preferences are not only recorded and weighted, but also reflected in the final assessment.

CSC recommends the following:
1. Limit inclusion of budget impact in the final value assessment, but rather report it as one endpoint.
2. Recognize value beyond 5-year timeline including late and long-term benefits and effects.
3. Include and apply weights to user preferences. Ensure that user preferences are appropriately reflected in final assessment.
4. Include value endpoints that are important to patients.

**Population Perspective**
Although the intent of ICER is to take a “population” level perspective as opposed to trying to create shared decision making tools to be used by individuals and their clinicians, this intention belies the real-world implications of ICER determinations. Our concern is that ICER assessments will be used at all levels within the care system from the micro/individual to the macro/policy and payer levels. As DuBois and Westrich (2017) note, many of the value frameworks including ICER’s are operating “de facto, to influence health care decision making.” It is important for ICER to understand the impacts of their assessments and carefully consider the repercussions, even if unintended, on the lives of patients and their families in need of treatment and services. This is particularly salient in an era of precision and patient-centered medicine. DuBois and Westrich (2017) also state that “it is essential…that stakeholders also demand the necessary improvement to these frameworks, and that their developers respond promptly and accordingly.”

CSC recommends the following:
1. Recognize the potential and applied use of ICER value assessments by a variety of stakeholders, regardless of intended use and audience.
2. Define patient perspective as opposed to societal perspective.
3. Outline when and how ICER will incorporate the relative impact of different care options on work productivity as a scenario analysis.

Evidence and Outcomes

CSC fully recognizes the importance of evidence in setting policy and when making decisions with patients. Evidence should not be limited to randomized controlled trials (RCTs) as it is not always possible to perform nor can an RCT encompass all of the available and relevant evidence from various sources an RCT.

Patient-definitions of value must be included in any assessment. Conway and Clancy (2009) state that “clinicians and patients need to know not only that a treatment works on average but also which interventions work best for specific types of patients.” The National Health Council (2016) outlines “patient-centered data sources” as integral to a patient-centered value model. They note that the value model should incorporate a variety of credible data sources that allow for timely information and account for the diversity of patient populations. This information should come from real-world settings and be reported by patients directly. Outcomes should be important to patients and capture their experiences. We applaud ICER’s statement that “recognition that what matters to patients is not limited to measured “clinical” outcomes. Patient registries and survey databases could provide opportunities to better understand patient experiences from a wide-range of individuals.

While we appreciate ICER’s use of health-related quality of life, we ask that additional patient-defined outcomes be included in the assessment. These should be aligned with the list of “other benefits and disadvantages” and “other contextual considerations” that were included in the 2017-2018 ICER Value Framework update. CSC will be submitting comments on the value framework update which includes suggestions to this particularly component but of note for this background and scope document.

CSC recommends the following:

1. Ensure transparency at each point of the methodological process including not only the specifics of the method but also the rationale behind the choice and literature to support those decisions.
2. Include a balance of data derived from controlled clinical trials (including observational trials) and real world evidence including data and information from patient and patient advocacy groups.
3. Create principles to ensure that the use of data meets a high level of scientific credibility.
4. Provide a transparent a priori statement of key assumptions.
5. Include weights to accommodate varying user preferences.
6. Incorporate a timeframe that is sufficient to reflect the full range of immediate and late- and long-term treatment benefits and effects.
7. Ensure that outcomes reflect patient experiences and preferences.
8. Utilize existing patient registries and survey databases to explore and incorporate patient experience data.

9. Incorporate review and approval from multidisciplinary, disease-specific experts.

10. In addition to the ICER-defined “other benefits and disadvantages” and “other contextual considerations” the concepts of “financial toxicity” and “costs associated with late and long-term side effects” should be included in outcomes.

**Conclusion**

We appreciate the opportunity to provide feedback on ICER’s Background and Scope Document on Poly ADP-ribose polymerase (PARP) Inhibitors for Ovarian Cancer: Effectiveness, Value, and Value-Based Price Benchmarks. We look forward to future opportunities to contribute to ICER’s work. As always, we encourage ICER to provide meaningful opportunities to engage patients in each step of the value assessment process. CSC would be pleased to work with ICER to identify and encourage patient participation. Please feel free to contact me at 202.650.5382 or linda@cancersupportcommunity.org if you have questions or if we can serve as a resource to your work.

Sincerely,

Linda House, MSM, BSN, RN
President
Cancer Support Community Global Headquarters

**References**


