March 27, 2017

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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 (ICER) 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 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.

We are pleased to offer these comments and we appreciate the timeframe offered to do so as well as the online orientation presentation provided to keep stakeholders informed about the process and proposed changes. As opposed to previous comment periods, the 60-day timeline is more reasonable and the guidance more transparent. We agree with the overall purpose of the value framework to help the United States evolve toward a health care system that provides sustainable access to high-value care for all patients. As such, we offer the following feedback regarding the 2017-2018 ICER Value Assessment Framework.

**Concept of Value**

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.

ICER’s use of cost per quality-adjusted-life-year (QALY) is also debatable. ICER states that QALY is the “standard used by academics, manufacturers, patient groups, and governments around the world.” Further, ICER adjustments to QALY were based on “empiric evidence, academic literature, and discussion with stakeholders.” Yet, none of these references were expounded upon with the specific sources or literature that were considered as these decisions were made. ICER simply states that QALY is the “established benchmark” without illustrating the full range of instruments considered and reasons for decisions. For example, ICER references “the true opportunity cost at the margin of health spending.” It is incumbent upon ICER to define exactly what is meant by this and how it impacts the framework. Likewise, more information is needed for stakeholders to understand the “affordability and access alert” and how it is measured.

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, as noted below, 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. Clarify ICER definition of value as currently outlined in the framework. If budget impact is used as part of the definition, consider renaming the framework to reflect this.
2. Limit inclusion of budget impact in the final value assessment, but rather report it as one endpoint.
3. Recognize value beyond 5-year timeline including late and long-term benefits and effects.
4. Include and apply weights to user preferences. Ensure that user preferences are appropriately reflected in final assessment.

Population Perspective and Health System 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.”

ICER also calculates incremental cost-effectiveness using a health systems perspective versus a societal perspective. However, it is unclear what components are included in this perspective and when and how ICER will incorporate the relative impact of different care options on work productivity as a scenario analysis.

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 health system 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.

**Patient and Stakeholder Representation**

As communicated in CSC’s response to the previous version of ICER’s Value Assessment Framework, we are pleased with the intent of the organization to meaningfully include patients and stakeholders in the assessment of value and effectiveness of different drugs, devices, tests, and delivery system innovations. 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 entire value assessment process. Patients should be sought out and meaningfully included 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 patients (throughout the entire value assessment process) who have experience and knowledge of that specific disease state. For example, patients who have had breast cancer should be commenting on breast cancer treatments specifically.
2. Incorporate a sufficient 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 development and when votes are taken.
3. Provide patient representatives with information in a transparent, timely, and understandable manner. CSC would be pleased to work with ICER to pilot such information.
4. Incorporate diverse patient representatives on the Governance Board with expertise and knowledge that represent the full spectrum of wellness, disease stage, specific disease state, and geography. This board should be expanded to include individuals who can represent or who have access to resources that would allow ICER to benefit from a more comprehensive level of information on the patient experience.

**Integration of Evidence from Multiple Sources**

As noted above, evidence informing ICER’s value assessments cannot be limited solely to financial impact. The same holds true for evidence from randomized controlled trials (RCTs).
RCTs are widely deemed the gold standard of research, allowing for limited bias and increased usefulness in judging clinical effectiveness. While this is true, it is also not always possible to perform an RCT nor can an RCT encompass all of the available and relevant evidence from various sources. We commend ICER for promulgating a policy on inclusion of grey literature, but this alternative source of information must rise to a minimum of peer-reviewed and published literature.

ICER states in this update to the framework that they are creating “an explicit place and role for consideration of elements of value that are important to individual patients but that fall outside traditional clinical measures.” 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.

CSC recommends the following:
1. Incorporate a wide array of evidence into value assessments. This includes patient-centered data sources.
2. Work to ensure that outcomes reflect patient experiences and preferences.
3. Utilize existing patient registries and survey databases to explore and incorporate patient experience data.
4. Incorporate review and approval from multidisciplinary, disease-specific experts.

Clinical Expertise
ICER works to ensure that patients on panels have the appropriate level of expertise to fully understand complex clinical scenarios. Likewise, CSC encourages ICER to require all health care professionals serving on voting panels to have relevant, thorough, and up-to-date expertise in caring for patients with the disease condition under review. CSC would like ICER to mandate that physicians serving on the voting panels have board certification in the relevant specialty.

CSC recommends the following:
1. Allow patient participants to be involved in all steps of ICER framework process including voting.
2. Incorporate multidisciplinary health professionals on all panels.
3. Require health care professionals serving on panels to have relevant, thorough, and up-to-date expertise in caring for patients with the disease condition under review.

Cancer Support Community 5
4. Mandate that physicians serving on the voting panels have board certification in the relevant specialty.

Methodology
ICER has indicated a commitment to making the process of value assessment transparent and inclusive. DuBois and Westrich (2017) state that ICER’s “methodology and evidence sources are well described”, yet “the executable models and associated computer code of its economic models are not publicly available.” ICER has improved transparency yet there are still lingering questions regarding specific components of the process. For example, ICER states that “the potential budget impact threshold for new drugs will continue to be calculated as double the average net budget impact for new drugs that would contribute to overall health care cost growth beyond the anticipated growth in national GDP plus an additional 1%.” Although ICER describes this process, it is still unclear how ICER developed this calculation. All methodological assumptions and approaches should be informed by current science and explicitly defined for all stakeholders to understand and evaluate.

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. Ensure transparency with all resources used in the development of evidence reports.
3. 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.
4. Create principles to ensure that the use of data meets a high level of scientific credibility.
5. Require peer-review by a panel of multidisciplinary, disease-specific experts for all documents informing the value assessment process.
6. Incorporate comments from stakeholders into documents or provide rationale why feedback was not incorporated into final documents.
7. Provide a transparent a priori statement of key assumptions.
8. Include weights to accommodate varying user preferences.
9. Incorporate a timeframe that is sufficient to reflect the full range of immediate and late- and long-term treatment benefits and effects.
10. Conduct vote only after full comment period has commenced.

Relevance and Timeliness of Recommendations
DuBose and Westrich (2017) state that value is dynamic, evidence continues to accrue and indication evolve requiring value assessments to account for the moving target of evidence and innovation. As scientific evidence evolves and new treatments and devices are introduced, it is incumbent that any value assessments be updated to reflect the most up-to-date evidence.

CSC recommends the following:
1. Provide, at minimum, 60 days for stakeholders to comment on any documents released by ICER.
2. Ensure that stakeholder groups have the opportunity to inform and review preliminary report findings before the first draft is made available for public comment.
3. Specify how patient groups will be given the opportunity to present the results of their own evidence generation and ensure that this occurs before the first draft document is released for comment.

4. Specify if manufacturer comment and discussion will occur before the vote and not only following the presentation of the summary of the evidence review.

5. Determine a deadline for decision that does not impact the ability of a patient to access a treatment option determined to be effective for a particular disease.

6. Allow for full transparency of the data used for decision making.

7. Revise assessments as new evidence becomes 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.

8. Adhere to a defined standard of peer review in regards to the list of patient groups, clinical experts, and policy experts who have been consulted as part of the report development process. For example, stakeholders who have been consulted must be established experts in the specific field under analysis. They should be given ample time to review documents and provide substantive feedback that is considered by ICER as they finalize documents.

9. Provide transparent and specific guidance for assessment updates to reflect the evolution of scientific evidence and introduction of new treatments and devices.

Patient Validated Endpoints and Definitions of Value
As noted in CSC’s previous comments on the ICER Value Framework, while we understand the use of the quality-adjusted life year (QALY) as an endpoint, this does not serve as an endpoint that is meaningful to patients. The QALY should not be used as a sole or primary measure of value. Multiple studies, including CSC’s Registry data, show that for patients with cancer and other chronic, debilitating illnesses, there is a delicate balance between quality and quantity of life. Patients have reported a desire for a shorter overall survival in exchange for higher quality of life. The QALY framework assigns the exact same score to an individual who lives six months in perfect health to an individual who lives a full year in a debilitated state. Patients may assign a very different level of value to each of these scenarios, which is why we support different weights applied to different outcomes and factors as noted above. Attention to individual preferences and needs is key. Other value models (American Society of Clinical Oncology, the National Comprehensive Cancer Network, and DrugAbacus) have taken similar approaches to assigning higher levels of value to endpoints such as overall survival without a full appreciation and representation to the value that patients assign to shorter, incremental gains. CSC once again encourages ICER to utilize a framework that more closely represents the endpoints that are meaningful to patients. CSC would be pleased to work with ICER to develop these endpoints.

In summary, CSC recommends the following:
1. Include and apply weights to user preferences. Ensure that user preferences are appropriately reflected in final assessment. (As noted above)
2. Assign higher levels of value to outcomes that are meaningful to patients.

**Focus on Medication Acquisition Costs**

As noted in our previous comments, the impact on the individual in terms of personal health care spending is increasing. CSC believes the focus solely on sales or acquisition costs to estimate treatment costs minimizes the reality and attention that should be placed on finding solutions that address the multitude of factors impacting elevated spending. As suggested by the National Pharmaceutical Council (n.d.) all health care costs and cost offsets should be included and costs should be representative of the net price most relevant to the user, for example co-pays are more meaningful to patients than what their insurance plan pays. Finally, as noted previously the timeframe for costs should be long enough to assess the full benefits of treatment.

In summary, CSC recommends the following:
1. Include the full range of health care costs and cost offsets in final assessment.
2. Include costs that are representative of the net price most relevant to the patient.
3. Incorporate a timeframe that is long enough to assess the full benefits and effects of treatment.

**Other Benefits and Disadvantages and Other Contextual Considerations**

In our September 2016 comments, we asked ICER to consider including low-grade, chronic side effects and we are pleased with the effort to include ten elements that are inclusive of these long-term issues. Although, the ten elements considered in other benefits and disadvantages and other contextual considerations are a welcome addition to the value framework, much more clarification is necessary in order for this component to be thoroughly understood by all stakeholders and ultimately to operate effectively. First, each element is broad in nature and not well-defined. For example, how will concepts such as “unmeasured patient health benefits” and “impact on public health” be analyzed?

One specific area that is touched upon but not included in a way that reflects its magnitude is financial toxicity. In addition to patient cost sharing for medications and services, it is well documented that patients experience additional expenses related to their cancer treatment. Some expenses are more difficult to measure (parking, housing, etc.), but the framework could allow the capture of true out-of-pocket patient costs. In particular, ICER could apply some level of consideration to frequency of treatment as a part of the evaluation. Given the high cost of travel and time off work, a regimen that would be administered once per month may be less financially toxic to a patient and the system than one administered once per week, as one example. Additionally, this framework does not give consideration to the costs associated with interventions required as a comprehensive part of treatment. For example, supportive care agents needed to manage nausea, steroids required as a part of a treatment regimen, etc. Another area that is not currently included in the framework in a meaningful way is the cost of late and long-term side effects to the patient such as heart disease, hypertension, endocrine system problems, bone issues, cognitive impairment, and secondary and recurrent cancers. Further, the psychosocial repercussions of many late and long-term side effects can be devastating to patients, particularly if they were not prepared for them.
Additionally, the process of gauging a “relative score” for each element, considering evidence, and gathering further input from stakeholders before an independent appraisal committee will assign an overall ranking of one to five of the relative contribution to overall long-term value for money of all other benefits and disadvantages and other contextual considerations seems exceedingly complex and imprecise. Although the movement from qualitative evidence that is taken into consideration as a whole to a more structured approach to identifying this component is commendable, it is not yet well formulated. The concept of “evidence and other information relevant to these value elements” is unclear both in terms of what this evidence will look like, how it will be collected, and from whom. Who will author the case studies and how will they be vetted prior to being presented to the committee? It is also unclear if greater weight will be given to evidence and information provided by clinical experts versus that provided by patients. Finally, taking all of this evidence and information on ten elements and reducing it to a single quantitative score of relative contribution is reducing complex, nuanced, and varied concepts into a simple score that belies the overall importance.

In summary, CSC recommends the following:

1. Clarify the process and outline how it fits into the overall framework.
2. Define each of the ten elements and include patient feedback in each definition. Indicate how each will be analyzed and/or measured.
3. Add “patient financial toxicity” to the list of elements.
4. Measure and account for alternative systems costs or off-sets (e.g. treatment every 3 weeks vs. once per week allows for fewer disruptions to work, home, and family life and reduced costs as they relate to out-of-pocket expenses and transportation.
5. Incorporate “late and long-term side effects and benefits” to the list of elements.

Specific Update Proposals

Ultra-Orphan Drugs

It is unclear from this framework update how ICER is defining “ultra-orphan drugs.” We recognize that ICER plans to draft and distribute additional guidance on this component of the framework and we look forward to the release of that document.

Devices, Tests, and Delivery System Innovations

While we appreciate the specific mention of devices, tests, and delivery system interventions, it is unclear from the proposed framework update how specific methods for incorporating and judging evidence will be analyzed and implemented for these categories. In addition to specific methodology and evidence, it will be important to incorporate an additional list of other benefits and disadvantages and other contextual considerations as devices, tests, and delivery system innovations range widely across purpose and intended use. As such, we recommend that ICER develop a separate guidance document outlining the specific framework guidelines as they apply to each category of devices, tests, and delivery system innovations.
Dissemination
Once assessments are complete, ICER should work with patients and the patient advocacy community, among others, to present the results in a manner that is understandable and accessible. There should be clear instructions for use, particularly regarding the population perspective as noted above. Patients and providers should be warned against the use of value frameworks for individual decision making. Limitations of the assessments should be made clear.

In summary, CSC recommends the following:
1. Work with patients and patient advocacy groups to disseminate results in a manner that is clear and understandable.
2. Provide clear instructions for implementation and warnings again unintended use.

Conclusion
In conclusion, CSC sincerely thanks ICER for the opportunity to comment on this iteration of the Value Assessment Framework and provide patient-informed input as we both seek to best serve the millions of people impacted by illness each year. We believe that ICER is taking important steps to fine-tune the value assessment framework. We are acutely aware of the rising costs of treating cancer and other diseases, and we support efforts that contain costs while ensuring the provision of truly comprehensive care. As such, we reiterate our strong belief that patients should be at the core of all of ICER’s work and we are prepared to partner to ensure that the voices of patients and their loved ones are incorporated in value assessments that have the potential to impact their lives in a significant way. We stand ready to suggest patient participants for panels and activities, partner to develop patient-centered language for potential participants, or offer feedback or information to help inform and shape the process as ICER seeks to enhance this process. 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,

[Signature]

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