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October 18, 2019

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

RE: Comments on Proposed Changes to ICER's 2020 Framework

Dear Dr. Pearson:

The National Health Council (NHC) is pleased to provide comments on the Institute for Clinical and Economic Review's (ICER) solicitation for comments on the proposed 2020 Value Assessment Framework update. Founded in 1920, the National Health Council (NHC) brings diverse organizations together to forge consensus and drive patient-centered health policy. The NHC provides a united voice for the more than 160 million people with chronic diseases and disabilities and their family caregivers. Made up of more than 125 national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient advocacy organizations, which control its governance and policy-making process. Other members include health-related associations and nonprofit organizations including the provider, research, and family caregiver communities; and businesses representing biopharmaceutical, device, diagnostic, generic, and payer organizations.

The NHC appreciates the opportunity to comment on ICER's proposed changes to its 2020 framework. We believe that while much progress has been made in recent years, there is still significant work needed to be done to fully integrate the patient voice into value assessment.

In response to ICER's proposed value framework updates, the NHC has the following suggestions and comments to ensure the framework is truly patient centered. We note and appreciate that several proposed changes are responsive to our comments submitted on June 10, 2019. Under the general topic areas below, we also offer recommendations on how to strengthen the 2020 framework and provide specific comments on particular sections of the proposed updates.

I. Promote Meaningful Patient Engagement

Public comment periods included in the revised timeline remain insufficient to facilitate meaningful patient engagement

The proposed update outlines an extended timeline for “large class” reviews by nine weeks, but this includes only one additional week for public comment. One added week is still insufficient to promote meaningful stakeholder engagement. Tables 1 and 2 below provide an overview of recent comment periods for an Asthma “Large Class Review” and an ongoing single-intervention review.

ICER provided stakeholders with a mere 4-weeks to digest and review 132 and 148-page documents filled with complex materials, analyze it, develop comments, circulate comments to their scientific advisory boards and membership, all while potentially hiring an expert consultant for assistance. This is an impossible request for patient organizations with a small staff, limited resources, and who must juggle an ICER review with other critical, mission-related daily tasks.

Table 1. 2018 ICER Asthma “Large Class Review” Timeline

Document	Public Comment Period for Asthma Large Class Review in 2018 ¹
Asthma: Draft Scoping Document	05/15/2018 – 06/05/2018
Asthma: Draft Evidence Report • 132 pages ²	09/24/2018 – 10/22/2018

Table 2. Ongoing Type 2 Diabetes Single Intervention Review Timeline

Document	Public Comment Period for Type 2 Diabetes Review in 2019 ³
Type 2 Diabetes: Draft Scoping Document	05/02/2019 – 05/22/2019
Type 2 Diabetes: Draft Evidence Report • 148 pages ⁴	09/11/2019 – 10/08/2019

Recommendation: We feel strongly that a 90-day comment period should be provided to review all draft evidence reports – not just large-class reviews. This timeframe would bring the ICER comment period more in line with the timelines of other organizations that seek to engage the patient community, such as the Food and Drug Administration (FDA). For example, recently released FDA draft patient-focused drug development guidance provides a 90-day comment period.

Comment periods should also be extended when dates fall over holidays. For example, the already limited public comment period for the asthma large-class review coincided with the Memorial Day Weekend.

Patient-facing educational materials should be co-developed with patient organizations

As part of ICER’s commitment to facilitating effective stakeholder engagement, ICER proposes to develop a series of webinars on the principles of health technology

assessment and economic modeling for a general audience. The NHC process to educate patients involved conducting a needs assessment to identify which resources needed to be developed in this area. As a result, we created an online educational series, *In the Pursuit of Value: An Introduction to Health Economics and Value Assessment*.⁵ Our modules were developed with patient community, academic researcher, and also ICER staff input. The NHC would be happy to consider developing modules on additional topics ICER might recommend.

Recommendation: Ultimately, it is critical that any patient-facing materials or trainings are co-developed with members of the patient community.

Provide clearer guidance on what patient-submitted data has been impactful and what would be useful for patients to collect and submit

We thank ICER for being responsive to our feedback that the Patient Population, Intervention, Comparison, Outcome, Timing, and Setting (PICOTS) framework serves as a platform for gathering information from and communicating with patient groups. ICER's plan to incorporate PICOTS elements into the patient survey is a welcome first step. We also appreciate ICER's proposal to develop a new "Patient Perspectives Chapter." The decision to include this chapter at the very beginning of the report is aligned with our 2018 recommendation that "VA bodies can open a VA report by leading with patient-experience input to provide context and set the stage for interpretation of the assessment. Patient groups can work with VA bodies to develop this section."⁶

Recommendation: We recommend that this new chapter should not only include *what* information was submitted by patients, but also *how* it informed the review. This would provide important lessons learned for the patient community.

Additionally, we appreciate ICER formalizing the debriefing process with patient groups. However, the proposed update states, "ICER's practice, which has been the same for many years, is to respond to draft-report comments with this degree of detail and will continue to do so; scoping documents currently describe suggestions we have accepted under a 'Stakeholder Input' heading, and we propose to include details of why some suggestions have not been adopted." We must point out that ICER has conducted dozens of reviews over recent years, and it is impractical for patient groups to sift through past reports about unrelated diseases to identify potential insights of what was useful/not useful. Thus, we remain concerned that patient groups do not have direct, clear guidance on data that would be helpful for them to collect.

Recommendation: We suggest that a helpful resource for patient groups would be if ICER collated these responses in one place and identified key themes of patient input that was impactful or not impactful and why. This would be instructional for tailoring patient-group input. Translating this information may also provide useful insights to ICER and the patient community regarding "lessons learned." The NHC stands ready to assist to help ensure insights and lessons learned are shared broadly with the patient community.

Patient groups have become more sophisticated regarding topics related to value assessment and will increase their knowledge over time, especially from the "lessons

learned.” Thus, we also recommend that full economic models are made available to patient groups upon request.

II. Promote Value Assessment Methods Advancement and Transparency

Multi-criteria decision analysis (MCDA) has evolved substantially over the past decade

The Proposed Changes document states that “in 2009-2010 ICER attempted on several occasions to use a formal MCDA process in its appraisal committee deliberations. We found, as have others, that it was very difficult for participants to identify mutually independent factors in their decision-making, much less to give weights to them.”⁷ However, MCDA methodologies have evolved significantly over the past decade. In addition to ISPOR’s two task force documents,^{8,9} the Innovation & Value Initiative and the University of Colorado’s P-Value Center are each working to advance the field.^{10,11} International health technology assessment (HTA) bodies have also successfully piloted MCDA.¹²⁻¹⁵ For example the Belgian Health Care Knowledge Centre (Belgium’s HTA) concluded that “the results show that the proposed MCDA is feasible and acceptable for the unmet needs commission.”¹⁵

Recommendation: Given substantial researcher- and broader stakeholder-community interest in advancing MCDA, possibly as a more transparent approach to HTA, we recommend that ICER revisit MCDA by committing to at least one MCDA pilot study over the coming year to assess the ability of MDCA to capture value elements important to patients.

Greater transparency regarding shortcomings and limitations of value assessment findings

We reiterate our recommendation that quality-adjusted life year-based approaches are insufficient for capturing value from the patient perspective. In the absence of alternative approaches, the shortcomings and caveats to conclusions and recommendations stemming from these methods must be very clearly articulated. The patient community has observed “cherry picking” on the part of value assessment report users; that is, only giving attention to final cost-per-QALY findings of a report that fit a user’s agenda and ignoring those more illustrative parts that run counter to their agenda. We acknowledge that ICER has publicly stated that these kinds of actions run counter to ICER’s intent.

Recommendation: We ask ICER to continue to be responsible in calling out such actions. We highly recommend that ICER in presenting assessment findings be extremely clear in the presentation of results and blatantly transparent regarding uncertainty and assumptions. Critical caveats around interpretation cannot be located elsewhere in a report or in other documentation. Presenting results and caveats transparently also will assist stakeholders in identifying which assessment users are “cherry picking” the recommendations they adopt or ignore.

Results from the societal “co-base case” should also be presented alongside the healthcare sector perspective analysis within an evidence report, and highlighted in press releases, report-at-a-glance documents, and other decision-maker-facing materials (e.g., JMCP commentaries).

The societal perspective should be provided as the co-base case

In indicating why ICER does not present the societal perspective as a co-base, the document states that US decision-makers are not responsible for making trade-offs that involve broader societal resources. In some instances, this is true. However, most patients are employees and their employer is providing a health insurance plan as a benefit to keep the employee and his or her family members healthy and productive. It is those plans that potentially make use of value assessment report findings in their decision-making. We would counter when an employer funding a health insurance plan benefit has a contract with a plan does not see itself as having responsibility for keeping employees and their families healthy and productive, that employer should find a plan that does.

Similarly, ICER also states in the report with regard to committee voting that “It has always been our intention to use these votes as a way to signal to *decision-makers* that the “*right*” cost-effectiveness threshold to be applied in any individual situation should be a judgment that benefits from integration of cost-effectiveness results with an intervention’s potential other benefits (or disadvantages) and broader contextual considerations that include ethical dimensions of priority setting.”

Recommendation: We recommend that a societal perspective be presented as a co-base case to provide more than a signal regarding many of the broader contextual considerations. Providing both the societal co-base case and unambiguous caveats for interpretation alongside the findings can support users, mitigate cherry picking, and emphasize critical contextual considerations.

Transparency regarding ICER policies and approaches

ICER has long stated that for value assessments to be useful, they need to be conducted around the time of launch. However, expediting reviews before sufficient evidence to conduct an assessment, which will be used into the future, is irresponsible. For example, regarding ICER’s assessment of Zolgensma, ICER’s website notes that “An update was added to this report on May 24, 2019, to reflect the FDA label and new clinical data for Zolgensma.”¹⁶ While the updated report states that overall conclusions remain unchanged, it does raise important questions regarding how ICER determines when a sufficient amount of evidence is available to conduct an initial review, and when sufficient new evidence is available to conduct a re-review. The Proposed Changes document states that ICER wants to use the “best available evidence at the time.” However, it is uncertain how ICER determines if there is a sufficient amount of evidence at the time.

Recommendation: We recommend that ICER clearly state how it determines that sufficient evidence is available to initiate an assessment – whether for an initial assessment or “reassessment”

Expanded use of real-world evidence

We appreciate ICER stating that real-world evidence (RWE) will play a greater role in upcoming reviews. Patient-provided RWE plays an important role in providing insights into patient perspectives.

Recommendation: We encourage ICER to continue to partner with patient groups to incorporate these types of RWE, which are critical to understanding the patient perspective. We encourage ICER to continue to use RWE found in the published literature and from other reputable sources. We also encourage ICER to focus its efforts in identifying, assessing, and utilizing reputable RWE rather than generating RWE *de novo*.

Cross-over to German Evidence Ratings is a distraction from the important improvements needed

There is a tremendous amount of important work to be done to improve value assessment methods. It is unclear what the value of this experimental, unvalidated crosswalk with German Evidence Ratings would be or its potential impact on US stakeholder decision making.

Recommendation: We recommend that instead of ICER expending resources to develop a crosswalk to German Evidence Ratings, ICER refocus efforts on advancing the purpose of its value assessment framework: “to form the backbone of rigorous, transparent evidence reports that, within a broader mechanism of stakeholder and public engagement, will help the United States evolve toward a health care system that provides fair pricing, fair access, and a sustainable platform for future innovation.” For example, ICER could focus its efforts piloting an MCDA approach, advancing patient-engagement methods, or studying the impact of value assessment on payer decisions, patient access, and/or utilization management.

III. Conclusion

The NHC appreciates the opportunity to comment on ICER's initiative and agrees that in this emerging field, methods must evolve and will need to be updated/adapted as experience in this space grows. The recommendations made above are offered with the goal of increasing patient centricity in health technology assessment. The NHC appreciates ICER's work to more proactively involve the patient community in value assessment. Just as opportunities to engage have increased in recent years, we hope to see a greater impact of patient engagement on value assessment moving forward.

We at the NHC are happy to discuss these recommendations with you, to clarify any suggestions made and to hear from you about how we can be supportive of their implementation. As always, please do not hesitate to reach out to us by contacting Elisabeth Oehrlein, PhD, MS, our Senior Director of Research and Programs, at 202-973-0540 or via email at eoehrlein@nhcouncil.org.

Sincerely,



Marc Boutin

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