IVI INNOVATION AND VALUE INITIATIVE

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RE: Public Comments ICER's 2023 Value Assessment Framework: Proposed Changes

Dear Dr. Pearson:

Thank you for the opportunity for the Innovation and Value Initiative (IVI) to comment on your proposed changes to the ICER Value Assessment Framework. IVI is a 501(c)3, non-profit research organization committed to advancing the science, practice, and use of patient-centered health technology assessment to support decisions that make healthcare more meaningful and equitable.

IVI's comments to ICER can be summarized as falling under three major themes:

- Aspects of true patient engagement are still missing from the framework. Full partnership with patients and patient organizations is a necessity going forward. Patients should be included as co-leaders and true partners throughout the HTA process (from before topic selection to final recommendations).
- More information, both in quantity and substance, is needed regarding health equity and its incorporation into the revised framework. Beyond initial inclusion, we urge continuous improvement in how health equity measures are included in HTA processes, data, and methods (i.e., no static solutions will suffice).
- There is a need for more explicit processes for formal incorporation of non-traditional data (e.g., qualitative lived experience and mixed methods) as well as formal processes for what to do when quantitative data are missing (i.e., emphasize gaps rather than "building models to the data").

1. Introduction

In revising its value assessment framework, IVI recommends that ICER consider the comments below related to proposed methodological changes, patient engagement strategies, and methods for handling data gaps.

1.1. Overarching Purpose and Principles of the ICER Value Assessment Framework

We appreciate that ICER is incorporating metrics from its recently published white paper, "Advancing Health Technology Assessment Methods that Support Health Equity," into its proposed revised value framework. But we encourage further incorporation of patient engagement, data diversity, and mixed method approaches to ensure the cultivation of health equity within HTA. In addition, we encourage ICER to continue advancing and incorporating research to make health equity an integral part of HTA methodology and the entire HTA process.

1.2. Population Perspective and Intended Uses of the ICER Value Assessment Framework

ICER should ensure that patient representatives are fully included as co-leaders throughout the HTA process, including in decision-making and scoping, analytic and reporting decisions, and final recommendations. This patient involvement should be representative of the diversity of underlying disease populations, and should include patients from groups that may not be adequately represented in clinical trials or other available data. Effort should also be made to include patients with lived experience beyond those who are formally affiliated with patient advocacy organizations, to help ensure diversity of opinion.

ICER should continue to expand and deepen the ways in which it engages patients with lived experience and patient advocacy groups in the HTA process. For true engagement, ICER should incorporate more patients as equals on HTA evaluation teams, by including them in decision-making from the selection and scoping of topics to the conduct of analyses and reporting of results, not just in advisory positions.

2. Comparative Clinical Effectiveness

2.1 Clinical Trial Diversity

We commend ICER for its plan to evaluate the diversity of participants in clinical trials. However, it is unclear exactly how these changes will be reflected in ICER's reports and recommendations. It would be useful if ICER would explicitly commit to specific reporting requirements and formats for inclusion of these equity-related factors. It is not yet clear how ICER's diversity ratings will be weighted in or incorporated into overall assessments. Additional transparency into how ICER's diversity ratings will be incorporated into overall assessments would be helpful. ICER should explicitly clarify how the diversity rating will be used in ICER's reports and by its voting panels.

We would also encourage ICER to follow and align its actions with efforts that the US Food and Drug Administration (FDA) already has begun in this area, to avoid redundancies or misaligned recommendations. In addition, while race/ethnicity, sex, and age are important dimensions to consider in health equity, it is also important to use a data-driven approach to uncover subgroups that have worse outcomes that we might not necessarily know of *a priori*. Engagement with patients and stakeholders can shed light on this.

2.2 Subpopulation Analyses

While ICER will consider issuing different evidence ratings for a single intervention if robust, high-quality evidence supports substantial differences in the evidence ratings of the intervention across different populations or subgroups, it would be helpful to have more explicit details on how evidence ratings will be handled when evidence across subgroups is inconclusive, mixed, or missing. In addition, ICER should consider analyses that measure the opportunity to reduce health disparities across subpopulations affected by the disease and treatments being evaluated.

3. Long-Term Cost Effectiveness

3.1 Perspective in Economic Models

ICER should consider following the recommendations of the Second Panel on Cost Effectiveness in Health and Medicine[1] to include societal perspective as part of its base case analyses in all assessments.

We commend ICER's plan to include "non-zero" inputs for impacts on productivity. In addition, ICER's assessments should strive to calculate a broader definition of economic burdens and financial impacts on patients, beyond direct and indirect medical costs. IVI's recently published Economic Impacts framework[2] can provide guidance on how to systematically catalog and consider these costs in a comprehensive manner.

3.2 Dynamic Pricing Scenario

IVI encourages the use of dynamic pricing scenarios for all assessments, not just those "predominantly targeted to Medicare-eligible populations." The current approach does not account for the fact that generic entry may occur for non-Medicare targeted drugs, making them likely to be much cheaper over time. We also encourage ICER to revisit its assumptions around likely price reductions as the relevant IRA provisions are implemented, to ensure they reflect the policies actually implemented.

The proposed dynamic pricing modeling also focuses on one cohort of patients. The societal benefits of novel health technologies may also benefit many future generations, who would be able to enjoy the technologies at a much lower price.

3.3 Quantifying Additional Dimensions of Value

We commend the use of shortfalls to consider severity. We would also encourage ICER to explore the use of GRACE-type analyses, as a framework that allows for consideration of risk aversion and disease severity. We also believe that more attention should be given to quantification of caregiver burden, as the impact of this may be larger than expected in many cases.

More details around the use of the Health Improvement Distribution Index (HIDI) would be helpful. It is unclear what the implications of using the HIDI will be, and how it will be incorporated into the deliberative process (e.g., does this imply that we will allow for a higher price for treatments because of the potential to lower disparities?). Also, the HIDI may be helpful to decision-makers, but it does not sufficiently capture what needs to be considered to incorporate health equity in HTA. While this index may be considered a good starting point, considering only prevalence is not enough, so we encourage ICER to continue exploring additional equity-related metrics.[3]

We also point out that the opportunity cost-based approach to estimate cost-effectiveness thresholds is subject to important limitations in methodology and data (see Sampson 2022[4] for a

[1] Sanders GD, Neumann PJ, Basu A, et al. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine. *JAMA*. 2016;316(10):1093–1103. doi:10.1001/jama.2016.12195
[2] https://thevalueinitiative.org/wp-content/uploads/2023/06/05-2023-Economic-Impacts-Framework-Report_FINAL.pdf
[3] https://thevalueinitiative.org/wp-content/uploads/2023/03/No-Value-Without-Equity_Synthesis-Insight.pdf
[4] Sampson C, Zamora B, Watson S, et al. Supply-side cost-effectiveness thresholds: Questions for evidence-based policy. *Applied Health Economics and Health Policy* 2022;20(5):651-667.

detailed examination of these limitations). Having one threshold for all disease states and all subpopulations may also ignore heterogeneity across populations and might risk worsening equity and efficiency trade-offs (Hernadez-Villafuerte 2022[5]). This is even more of a concern in a system like the US with multiple payer types with different opportunity costs.

Lack of a clear cost-effectiveness threshold in these cases should not completely block off the consideration of additional value elements. As pointed out by the framework document, cost-effectiveness is just one of many factors to be considered in a deliberative process for HTA. All these point to the need for development of deliberative methods that consider all these factors.

Mixed methods, including quantitative and qualitative analyses, should be considered as part of deliberative processes used in HTA by learning from methods used in other research areas. For example, ICER should continue to test and operationalize methods such as MCDA that will allow consideration of a broader set of value elements that matter to society. How assessors weigh these additional value elements beyond traditional CEA is an important area for exploration.

We would also like to emphasize that analysts cannot wait for perfect data to incorporate certain elements of value. Rather, it may be necessary to develop scenario analyses that explore changes to results using plausible assumptions for missing data. This can show where the provision of missing data has the potential to change decisions (or not), and to guide further research into missing data.

3.4 Health Benefit Price Benchmarks

To further allay concerns about the quality-adjusted life year (QALY), ICER should consider the use of alternative measures beyond equal value life-years gained (evLYG), such as Health Years in Total (HYT). Please also see our comments above regarding the opportunity cost perspective for determining cost-effectiveness thresholds.

The specific scenario analyses for SSTs seem arbitrary, so ICER should consider providing a range of scenario analyses that use different shared saving proportions or cost offsets, and more explicit guidance on when these will be applied to the Health Benefit Price Benchmarks (HBPB)

3.5 Other Changes

In updating assessments with RWE, accountability for equity-related data collection and incorporating this data into assessments is a necessity. We strongly encourage ICER to consider sensitivity or scenario analyses using plausible ranges when preferred data are not available.

4. Potential Other Benefits or Disadvantages and Contextual Considerations

4.1 List of Voting Questions and Voting Format

ICER should more explicitly discuss how its assessments will consider the socioeconomic impacts of new health technologies. How will such impacts be measured and reported, and how

[5] Hernandez-Villafuerte K, Zamora B, Feng Y, Parkin D, Devlin N, Towse A. (2022). Estimating health system opportunity costs: The role of non-linearities and inefficiency. *Cost Effectiveness and Resource Allocation* 2022;20(1):1-13.

are ICER's voting panels expected to account for these? Will panels be given explicit, detailed instructions for how to consider these issues or will this be more subjective?

In addition, ICER's panel voting and report recommendations should be worded to more explicitly ensure that their recommendations are not construed to unintentionally reduce access to care for patients.

ICER Processes for Conducting Value Assessments

A2. Topic Selection

ICER should ensure that patient representatives are fully included as co-leaders throughout the HTA process, including them in the topic selection and scoping processes. Patient engagement should begin as early in the HTA process as possible, before the point of deciding the disease area and topic selection, and before scoping.

A3. Stakeholder Engagement

We commend ICER's efforts to enhance their patient engagement processes, and their commitment to ensure patients' time and contributions to assessments are fairly compensated. As those most directly affected, patients and patient groups should especially be involved in the HTA process. For true and valid engagement, HTA programs must incorporate patients as equals on HTA evaluation teams by including them in decision-making, including the selection and scoping of topics, not just in advisory positions. An inclusive, transparent, and participatory process with a large and diverse set of stakeholders, especially those with lived experience, should be used throughout the HTA process, to ensure multiple, meaningful opportunities for feedback. Importantly, ICER's reports, and other publications should note how patient and other stakeholder input guided and changed the assessment process.

For "Share Your Story," ICER should consider that using an online form to collect patient input might not capture subgroups with challenges in accessing the internet. It would be helpful to have more explicit mention of ways in which ICER will try to identify and recruit under-represented subgroups for this.

We appreciate the opportunity to provide input to ICER's 2023 Value Assessment Framework: Proposed Changes. Please do not hesitate to contact me for further discussion.

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Innovation and Value Initiative