

December 13, 2022

Stephen Pearson, MD, MSc
President
Institute for Clinical and Economic Review
4 Beacon Street, Suite 800
Boston, MA 02108

Dear Dr. Pearson:

We appreciate that the Institute for Clinical and Economic Review (ICER) is convening stakeholders to address the methodological flaws of health technology assessment (HTA) and its implications for health equity by advancing work on a report funded by the Commonwealth Fund on “Evaluating and Advancing Health Technology Assessment Methods that Support Health Equity.” At its basis, high value care must also be equitable care. It is our hope that ICER will consider the recommendations developed in the attached report entitled, “Aligning Health Technology Assessment with Efforts to Advance Health Equity” as part of its report to be published in March 2023. In this letter, we summarize the report’s findings and recommendations for ICER’s consideration.

Researchers and health economists face a difficult history surrounding the use of quantifiable metrics and algorithms to value health care that are developed based largely on data derived from white populations and do not prioritize the delivery of equitable care. We urge ICER to reconsider current definitions of value to clarify the answer to the question, “Value to whom?” Treatments should bring value to the end users of our health care system – the patients – rather than just payers. It is important to define value consistent with the value of reducing health disparities and promoting health equity to ensure everyone receives high quality care. This will require development of new methods and algorithms to calculate value that inherently prioritize health equity and that incorporate elements of value unique to different subpopulations.

Our recommendations underscore the importance of addressing three significant challenges for value assessment: inclusive and representative data, methodologies that value health equity, and engagement of all people impacted directly by the condition being assessed. A true commitment to health equity will require ongoing engagement in this work as new questions arise as part of this process.

Inclusive and Representative Data

The foundational step toward health equity is improving the research and science that drives our health care system. Certain populations are excluded, intentionally or unintentionally, from much of the research that feeds value assessment models, instead relying on population-level averages. Comparative and cost effectiveness algorithms are derived from health utilities

reflecting white males. Clinical trial data also frequently lack diversity.^{1 2} Methods and data must catch up to the nation’s health equity goals.

Recommendations

Explicit Reference to Limitations of a Value Assessment: While ICER may recognize the limitations of data in its contextual considerations, we are concerned that ICER does not qualify the reliability of its base case value assessment and policy recommendations when entire subpopulations or outcomes are excluded from the data. Disclaimers should be included throughout ICER’s reports and in ancillary materials targeting stakeholders — such as policymakers — and the media as well. Where data gaps exist, ICER should have a process for further data collection. It is not enough for ICER to only cite the limitations of their results and still perpetuate inequity by treating those results as conclusive for use by payers and others.³

Invest In and Provide Resources to Fill Data Gaps in Advance of a Value Assessment: We understand that ICER has, in the past, provided limited grants for patient organizations to conduct surveys during their assessment process that are not used in ICER’s base case value assessment. We recommend ICER work with patient groups and other stakeholders to generate the data needed to inform a credible value assessment well in advance of its conduct. We also recommend ICER consider whether resources slated to be invested in a planned value assessment may be better invested in data generation. It is essential to allot time to data generation where gaps exist before advancing a value assessment that would otherwise fail to represent all patients.

Methodologies that Value Health Equity

Without improved methods, ICER will be limited in its ability to incorporate factors such as social and structural determinants of health and the intersectionality of social identities (such as Black and female). Real world research supports the conclusion that overlapping social identities — such as race and class — have real effects on health behaviors, measured preferences, and economic costs. We encourage ICER to capture this heterogeneity in benefits and costs within a value framework, as omitting it fails to provide adequate information to decision-makers to evaluate the value of treatments for socially disadvantaged populations and biases their conclusions.⁴

¹ Willyanne DeCormier Plosky, et al, “Excluding People with Disabilities from Clinical Research: Eligibility Criteria Lack Clarity and Justification,” *Health Affairs*, published October 1, 2022, <https://www.healthaffairs.org/doi/10.1377/hlthaff.2022.00520>.

² National Minority Quality Forum, “Traditional Value Assessment Methods Fail Communities of Color and Exacerbate Health Inequities White Paper,” *National Minority Quality Forum, Inc.*, published 2019, <https://www.nmqf.org/nmqf-media/traditional-value-assessment-methods>.

³ Don Husereau, et al, “Consolidated Health Economic Evaluation Reporting Standards (CHEERS) 2022 Explanation and Elaboration: A Report of the ISPOR CHEERS II Good Practices Task Force,” Item 20, *Value Health* 25:1, published June 2022: 10-31, <https://doi.org/10.1016/j.jval.2021.10.008>.

⁴ McRae, 1365-1372

For example, when ICER reviewed treatments for sickle cell disease and took steps to incorporate Medicare claims data, the survey of sickle cell disease patients provided insight that most patients were being served by Medicaid – not Medicare.⁵ Therefore, the data from Medicare were not necessarily reflective of the patient experience. Additionally, the survey found that pain crises were managed largely at home and not in a setting that would be captured by claims data.⁶ ICER did not reflect pain events outside the hospital in its analysis of the cost effectiveness and value of treatment despite qualitative input from sickle cell disease patients.⁷ Incorporating this knowledge into the base case would have produced a more accurate and nuanced assessment.⁸

Recommendations

Recognize Heterogeneity Among Patients and Incorporate into Value Assessment: The value of treatments may vary based on social identities, geographic communities, and other factors such as access to health facilities. We recommend that ICER use methods to stratify its work to identify the different impacts among treatments based on the varying social identities of patients and incorporate that knowledge into the base case.^{9 10} We also urge ICER to explicitly recognize factors that may skew the validity of value assessment conclusions — such as, how lower costs associated with standard care could result from underutilization of care, higher costs could result from overuse of emergency room services for populations without access to primary care, and differential access to pharmacies and community-based health workers could drive challenges for adherence to prescribed treatment and therefore impact associated treatment cost and health outcomes.¹¹

Incorporate the Values of Patients and People with Disabilities: We recommend that ICER partner with patient organizations familiar with the research literature in the conduct of systematic reviews far in advance of conducting a value assessment. ICER would gain insights from the literature about patient preferences and values that could be incorporated into the earliest scoping document on which patients, people with disabilities, and other stakeholders

⁵ Sick Cells, comment letter to ICER on draft evidence report for sickle cell disease, Feb. 2, 2020, page 1. https://sickcells.org/wp-content/uploads/2021/06/Sickle-Cells-Public-Comments-to-ICER_final.pdf

⁶ Sick Cells My Life with SCD Patient and Caregiver Survey Results: Inputs for the 2019 ICER Review, presentation to SCDA National Convention, 2020. https://sickcells.org/wp-content/uploads/2022/08/SCDAA-Convention-2020_Presentation-Slides.pdf

⁷ Linthicum, page

⁸ Bradt P, Spackman E, Synnott PG, Chapman R, Beinfeld M, Rind DM, Pearson SD. Crizanlizumab, Voxelotor, and L-Glutamine for Sickle Cell Disease: Effectiveness and Value. Institute for Clinical and Economic Review, January 23, 2020, p. 89. https://icer.org/wp-content/uploads/2020/10/ICER_SCD_Evidence_Report_031220-FOR-PUBLICATION.pdf

⁹ Jacquelyn McRae and Eberechukwu Onukwugha, “Why the Gap in Evaluating the Social Constructs and the Value of Medicines?” *PharmacoEconomics* 39:12, published August 30, 2021: 1365–1372, <https://doi.org/10.1007/s40273-021-01075-w>.

¹⁰ McRae, “Why the Gap in Evaluating the Social Constructs and the Value of Medicines?” 1365 – 1372.

¹¹ McRae, 1365-1372

will provide input. ICER's methods should be updated to allow for the values of a represented subgroup to be incorporated into the base case value assessment.

Avoid Discriminatory Metrics such as QALYs: ICER has an opportunity to develop, identify, and incorporate new methods for value assessment that allow for identifying and interpreting the heterogeneity of treatment impact, including the distributional impacts of a cost effectiveness analysis in relation to a population's experience of social disadvantages, consistent with recommendations from ISPOR — a professional society for health economics and outcomes research.¹² Innovative methods should deviate from averages and instead consider intersectionality based on social identities and, where data gaps preclude such conclusions, ICER should explicitly acknowledge its inability to make reliable conclusions about value for a particular subpopulation.

Conduct Retrospective Reviews of Previous Value Assessments: We recommend ICER partner with impacted patient stakeholders to conduct retrospective reviews of their past reports with an overlay of real-world evidence — such as ICER did in 2021 for hereditary angioedema¹³ — and consideration of factors impacting health equity such as social and structural determinants of health. This is especially important when value assessments are launched before the availability of real-world evidence and where earlier conclusions may have been biased. An updated assessment could change the conclusions, improve health care decisions that rely on the value assessment, and allow for a stronger understanding of the limitations of the conclusions for certain subpopulations that are useful to decision-makers.

Increase Transparency of Methods: We recommend ICER increase the transparency of its methods and consider strategies such as an open-source data model. We urge ICER to consider the recommendations of ISPOR related to study findings, limitations, generalizability, and current knowledge, as well as take steps to report key findings, limitations, ethical or equity considerations not captured, and how these could affect patients, policy, or practice.¹⁴

Engagement and a Meaningful Voice for Stakeholders

ICER is not alone in its work to advance health equity and will find many best practices in the engagement processes of participatory researchers, social work researchers, and entities such as the Patient-Centered Outcomes Research Institute (PCORI). Building on a background of robust engagement practices, in 2021, the PCORI Patient Engagement Advisory Panel (PEAP) published Equity and Inclusion Guiding Principles that provided more specific recommendations, calling for “critical engagement with historically disenfranchised groups whose interests have not been consistently centered due to systemic devaluations based on race, ethnicity, income, geography, age, sexual orientation, disability, and other

¹² Husereau, et al, Item 19.

¹³ ICER, “Assessment: Hereditary Angioedema,” *Institute for Clinical and Economic Review*, last modified August 24, 2021, <https://icer.org/assessment/hereditary-angioedema-2018/>.

¹⁴ Husereau, et al. Items 18, 19 and 26.

characteristics.” Incorporating these best practices into ICER’s value assessments would advance a culture shift that values the input of people with lived experience in the health system. Improved data and methods are difficult to achieve without this shift in culture.

For value assessment, meaningful engagement is especially challenged by the limited resources of patient and disability stakeholders that stand to benefit from the studied treatment. Advocacy organizations serving communities that are largely underserved in our health care system tend to be underserved themselves. Therefore, mounting a data collection process that is professionally designed with well-constructed questions and disseminated in a manner that is powered to get the input needed to be broadly representative is not an easy task, typically being far outside their normal scope of business. Yet, it is precisely the organizations representing people with lived experience that should be driving the data collection process because they are trusted and credible in their communities.¹⁵

ICER has an opportunity to recognize the investment, partnership, technical assistance, and other resources that would enable organizations representing people affected by the studied treatment to themselves collect, curate, and generate the necessary data to participate in a value assessment process effectively and productively.

Recommendations

Use Best Practices to Develop Protocols for Engagement that Prioritize Equity and Inclusion: We recommend ICER consider best practices — such as PCORI’s Engagement Rubric, Compensation Framework, guidelines to budget for stakeholder engagement, and Equity and Inclusion Guiding Engagement Principles — as potential models for developing protocols for meaningful engagement of people with lived experiences. Building trust begins with recognizing the past harm posed by HTA and committing to work collaboratively to assure future HTA is centered on improving health equity.

Provide Engaged Patient and Disability Partners with Resources for Capacity: We understand that ICER has started to provide stipends for participation to some engaged stakeholders. We urge ICER to equitably compensate engaged patients and people with disabilities for their participation in a value assessment process, modeled by the PCORI Compensation Framework. Due to the complexity of value assessment, we urge ICER to go further by providing sufficient resources to engaged organizations representing the impacted populations of patients so that they are able to hire outside experts — such as a health economist — to work in patients’ best interest during the value assessment process. ICER should encourage engaged stakeholders representing people with lived experience to access training and resources, such as the National Health Council’s Value Classroom, that will increase their capacity to meaningfully

¹⁵ Mark Linthicum, MPP, et al, “Finding Equity in Value: Racial and Health Equity Implications of U.S. HTA Processes, published 2022,” page 20, https://sickcells.org/wp-content/uploads/2022/10/IVI_Sick-Cells_Equity-in-Value_2022.pdf.

engage in a value assessment process and assure that they are broadly representing those people with lived experience that are not participating.¹⁶

Correct Systemic Disadvantages to Engaged Partners and Share Engagement Strategies in Final Assessment: We encourage ICER to evaluate the partner experience and make modifications as needed throughout the value assessment process to ensure that systemic disadvantages to any participants are addressed in real time. We urge ICER to state clearly as part of its published HTA how affected patient and disability stakeholders were engaged in the design of the study, consistent with ISPOR's recommendation.¹⁷

Conclusion

ICER has pledged to be part of change, stating, "We hear the clarion call ringing out — to be part of the change needed to ensure that race is no longer a determining factor in life expectancy, that medical research dollars flow to combat conditions that impact communities of color, and that we achieve health equity by addressing the negative impact of social determinants of health."¹⁸

To gain the trust of patient and disability stakeholders partnering with ICER in this work, ICER has an opportunity to prioritize increased transparency of its methods, the data that informs its assessments, and the algorithms that are used to make conclusions. This level of transparency would allow its models to be accessible and usable to all stakeholders. ICER also has a meaningful opportunity to participate in the co-creation of non-biased tools in health care, to purposefully correct the value assessment of the past that has perpetuated health inequities, and to do better in the future. Otherwise, its work will continue to be marginalized by dependence on flawed data and biased methods that perpetuate health inequity.

We appreciate this opportunity to provide input as ICER advances its work to address health equity. Please direct your response to Sara van Geertruyden at sara@pipcpatients.org.

Sincerely,

Alliance for Aging Research
Allies for Independence
ALS Association
Alstrom Syndrome International
American Association of Kidney Patients (AAKP)
American Behcet's Disease Association (ABDA)
Asthma and Allergy Foundation of America

¹⁶ NHC, "Education: Value Classroom," *National Health Council*, published 2021, <https://nationalhealthcouncil.org/education/value-classroom/>.

¹⁷ Husereau, et al, Item 21.

¹⁸ ICER, "ICER Statement on Racism in America," *Institute for Clinical and Economic Review*, published 2020, https://icer.org/news-insights/commentaries/race_in_america/.

Autistic Women & Nonbinary Network — AWN
Axis Advocacy
Cancer Support Community
CancerCare
Caring Ambassadors Program
Center for Autism and Related Disorders
Coalition of State Rheumatology Organizations
Color of Crohn's and Chronic Illness
Crohn's & Colitis Foundation
Cystic Fibrosis Research Institute
Davis Phinney Foundation
Derma Care Access Network
Diabetes Leadership Council
Diabetes Patient Advocacy Coalition
Disability Rights Oregon
Ellen Leigh
Epilepsy Alliance America
Epilepsy Foundation
Familia Unida Living with MS
Global Liver Institute
GO2 for Lung Cancer
Health Hats
Heart Valve Voice US
Hypertrophic Cardiomyopathy Association
ICAN, International Cancer Advocacy Network
Immune Deficiency Foundation
Lupus and Allied Diseases Association, Inc.
Multiple Sclerosis Foundation
National Organization of Nurses with Disabilities
Not Dead Yet
Partnership to Advance Cardiovascular Health
Partnership to Fight Chronic Disease
Partnership to Improve Patient Care
Preparedness and Treatment Equity Coalition
RetireSafe
Rosie Bartel
The Bonnell Foundation: Living with cystic fibrosis
The Coelho Center for Disability Law, Policy and Innovation
The Headache and Migraine Policy Forum
The Hepatitis C Mentor and Support Group, Inc. - HCMSG
Treatment Action Group (TAG)
United Spinal Association