

September 12, 2016

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

Dear Mr. Pearson:

The undersigned organizations are pleased to join the Partnership to Improve Patient Care (PIPC) on this letter providing input in response to the national call for input issued by the Institute for Clinical and Economic Review (ICER). In a debate that often is dominated by the perspectives of other stakeholders – payers, manufacturers and researchers – we seek to elevate the voices of those who might otherwise not be heard – those of patients, many of whom have serious and life-threatening chronic conditions, their caregivers and people with disabilities. Simply put, if we aren't paying for care that patients' value, we aren't really paying for value-based care.

As noted by ICER and many other organizations, rising health care costs, as well as changing benefit designs, place increased pressure on care access and affordability. In this environment, it is more important than ever to address the issue of value, and to make sure these efforts are centered on care and outcomes that patients value. One important element of this is making sure that patients, providers and other decision-makers have sound information and decision-support tools available to them.

ICER's particular approach to value assessment underscores long-standing concerns that many patients and consumers have about how the value of individual patient care will be judged, and whether these judgments will be applied in ways that ignore individual patient differences and needs or deny access to treatments or services that are valued by individuals or patient subgroups. In recent months, these concerns have been amplified as proposals have emerged that would misapply assessments like ICER's in ways that would impede patient access to optimal care by imposing one-size-fits-all value judgments.¹

While we recognize the steps your organization recently took to improve the procedures it uses to conduct value assessments, it is our hope that the changes to ICER's process lead to the meaningful incorporation of the voice of patients, people with disabilities, and those who care for them throughout its work. We are concerned that the effect of identifying the concerns of patients and people with disabilities as myths per ICER's recent report entitled "Addressing the Myths About

¹ CMS Proposed Part B Drug Payment Model, 81 FR 13229

ICER and Value Assessment” is to dismiss their very real concerns about both ICER’s methods and, more broadly, the standards to be used to judge the value of patient care. We urge ICER to give sincere and careful consideration to the input of all organizations representing patients and people with disabilities, many of which have direct experiences with ICER’s process and whose input would be very constructive. We may not always agree, but we should be able to agree that all voices matter, especially the voices of patients and people with disabilities that are directly impacted by your work.

Our input is informed not only by long-standing concern about the need to advance more patient-centered approaches to value assessment and value-based decision-making, but also by in-depth work that many of our organizations have participated in over the past year on the topic of value assessment. As you may know, PIPC held an in-depth roundtable discussion earlier this year with organizations representing patients and people with disabilities, also including Avalere Health and the Patient-Centered Outcomes Research Institute (PCORI). The PIPC roundtable report elicits and captures diverse perspectives on patient-centeredness in value assessment. We share the concerns in the PIPC report and support ICER’s use of the National Health Council’s Patient-Centered Value Model Rubric, as discussed below. We look forward to ICER’s incorporation of patient perspectives systematically and consistently throughout its assessments.

We would like to bring to your attention to several significant concerns from patients related to the process, methods, and end use of value assessment. Where appropriate, we also recommended steps ICER could take to address these concerns.

Process: ICER Should Make Substantial Improvements to Its Process for Conducting Value Assessments to Ensure It Receives and Considers Input from Patients and Their Caregivers

It is imperative that ICER proactively reach out to patients, patient advocates and clinical experts for their input, and explain the process through which assessments are developed. Based on our experience with organizations such as the Patient-Centered Outcomes Research Institute (PCORI), we know that patients and people with disabilities, and the organizations who serve them, are able and willing to offer input and expertise. The data, information, and perspective that these groups bring to the conversation around value assessment is vital to driving value in health care, as is supporting their capacity to contribute. For example, patient groups offer expertise on the conditions they represent, both from the perspective of patients, but also via their close collaboration with the medical community. Specifically, we propose the following changes to ICER’s process.

- ICER should be engaged with organizations representing the impacted patient communities and clinical experts in the specific treatment area under consideration in advance of scoping

their projects to ensure that ICER's assessments are achieving consensus on the assumptions (such as predicted uptake of the treatment), definitions and underlying questions. We suggest that ICER conduct briefings for the organizations representing patients that are candidates for the potentially studied treatment, as well as the clinical experts that provide services to those patients, as part of the scoping process.

- ICER should provide stakeholders representing patients and clinical experts with a meaningful role in developing the construct and operation of ICER's advisory panels to ensure that participants have specific expertise on the treatments under consideration.
- ICER should develop realistic timeframes to provide comments throughout their process. We applaud ICER's recent efforts to extend certain comment deadlines.
- ICER should respond to feedback by making it clear what input was incorporated and why certain input was not incorporated into its final reports. Meaningful engagement requires not only getting input from patients, people with disabilities and clinical experts, but also *incorporating that feedback* into your final reports. It is a positive first step that ICER is making comments to its draft reports publicly available.

Methods and End-Use: ICER Should Significantly Revise Its Methods To Achieve the Goal of Ensuring Patient Needs and Preferences Are at the Center of Its Work, Even When Assessments Are Intended for Payer Decision-Making

We recognize that ICER's assessments are not generally intended for use by patients and people with disabilities. However, we are concerned that ICER's work could hinder efforts to advance best practices in shared and supported decision-making that are being developed for patients. We would ask that you consider methodological changes that would help individual patients or providers disaggregate your assessments in ways that help them understand how the assessment relates to them and the decisions they are making, similar to the manner in which Congress called on PCORI to make its research findings relevant to individual patients.² If ICER's work is to be considered a reference for private payers in determining coverage and value of treatment options, then ICER must take responsibility for the impact its work will have on real patients and people with disabilities. Therefore, PIPC recommends several changes to ICER's methods that would support decisions by payers that reflect value as defined by patients and people with disabilities, thereby improving health outcomes and reducing costly adverse events.

- *ICER should avoid the "one-size-fits-all" mentality that does not recognize diversity among patients and people with disabilities by adopting alternative approaches to the use of quality-adjusted-life-years (QALYs).* Patients and people with disabilities do not support the use of QALYs in research related to the assessment of treatment value. Additionally, a societal impact

² 42 U.S.C. 1320e(d)(8)(A)(ii)

analysis based in cost-per-QALY may not account for the value of substantially improving the life of a person with a disability or serious medical condition. ICER acknowledged it is “aware of this possibility” of the discriminatory potential of QALYs in its recent report, making it disingenuous to call the concerns of patients and people with disabilities about QALYs a “myth.” Alternative approaches could better enable patients and people with disabilities to understand how the evidence on clinical and economic value relates to them, and enable payers to better develop policies that reflect value from an individual perspective (not just at the population level).

- *ICER should expand the sources and types of data it relies upon for its evidence reports, thereby reinforcing the importance of changing the culture of research to recognize the value of patient data sources, without viewing such a change as an assault on evidence-based medicine.* Randomized Clinical Trials (RCTs) provide limited data, representing only a small part of the population and do not represent real-world treatment impacts as do other sources such as patient and clinical data registries. While RCTs provide strong assurance of validity within the study, they do so at the expense of offering limited insight on value for patients or endpoints beyond the study. ICER should also provide clear consistent guidance on the range of studies relied upon by ICER to help all stakeholders assess its usefulness for decision-making.
- *ICER should be transparent about the evidence on which its assessments are based, as well as the limitations presented by that data such as the limited populations and outcomes captured by the evidence.* Stakeholders should understand the evidence used to develop value assessments, the quality and source of evidence, and the limitations of the evidence. By better articulating the limitations of the data that informs the development of ICER’s value framework, it will be clear where evidence gaps exist to inform future research efforts. For example, PCORI was called upon by Congress to articulate the limits of its research and could provide a useful model for consideration.³
- *ICER should better reflect patient-centered outcomes.* Quantifying value in a way that is useful and meaningful to patients and people with disabilities requires a basic understanding of their values and preferences. Doing so will benefit both patient and payer as they identify and integrate the appropriate patient-centered criteria in assessing the value of treatments for a particular condition. ICER’s assessments should not conflate value considerations at the population level with value considerations experienced at the individual level, where real-world personal and financial cost considerations differ from population-based models.
- *ICER should not develop assessments that result in a single universal “value score” for patient populations. Patient sub-groups, and individual patients, define value differently based on their particular disease mutation, their preferences and their unique characteristics that result*

³ 42 U.S.C. 1320e(d)(8)(A)(iii)

in very real differences in the impact of treatments. We are concerned that payers use ICER determinations of value to restrict access to treatments without consideration of the varying value presented by a treatment or technology for the individual patient or person with a disability. Treatments impact patients differently based on their comorbid conditions as well. Value to patients and people with disabilities simply cannot be captured in a single number.

- *ICER should recognize a holistic cost perspective.* By focusing on short-term costs, ICER limits the relevance of its reports only to the short-term actuarial analysis of a payer. ICER's assessments have a limited perspective on the economic component of value, while there are long-term, personal and societal costs that are not being considered in its value assessments, such as risk of disability and the potential need for caregiving. Broader costs should be considered rather than focusing only on short-term costs so that ICER's work is relevant to decision-making by patients, people with disabilities and their providers.

We support the work of the National Health Council to provide a guide to evaluate the patient-centeredness of value models and to guide value model developers on the meaningful incorporation of patient engagement throughout their processes. The rubric outlines the domains that must exist for a value framework to be patient-centered:

- **Patient Partnership.** Patients should be involved in every step of the value model development and dissemination process.
- **Transparency to Patients.** The assumptions and inputs into the value model itself – and each step in the process – should be disclosed to patients in an understandable way and in a timely fashion.
- **Inclusiveness of Patients.** The value model should reflect perspectives drawn from a broad range of stakeholders, including the patient community.
- **Diversity of Patients/Populations.** The value model should account for differences across patient subpopulations, trajectory of disease, and stage of a patient's life.
- **Outcomes Patients Care About.** The outcomes integrated into the value model should include those that patients have identified as important and consistent with their goals, aspirations, and experiences.
- **Patient-Centered Data Sources.** The value model should rely on a variety of credible data sources that allow for timely incorporation of new information and account for the diversity of patient populations and patient-centered outcomes, especially those from real-world settings and reported by patients directly. The data sources included should reflect the outcomes most important to patients and capture their experiences to the extent possible.

Following NHC's rubric as a guide to improve ICER's framework would be a constructive first step in addressing the concerns of patient groups. We appreciate your consideration of the views

and concerns above, and look forward to being engaged in a version 2.0 of ICER's framework for assessing value.

Sincerely,



Tony Coelho
Chairman, Partnership to Improve Patient Care

And the Undersigned Organizations:

ALS Association
American Association of Neurological Surgeons/ Congress of Neurological Surgeons
American Association of People with Disabilities
American Association on Health and Disability
American Autoimmune Related Diseases Association, Inc. (AARDA)
American Foundation for the Blind
Association of University Centers on Disabilities (AUCD)
Asthma and Allergy Foundation of America
Autistic Self Advocacy Network
Brain Injury Association of America
CancerCare
Christopher & Dana Reeve Foundation
Cognitive Compass
Cutaneous Lymphoma Foundation
Depression and Bipolar Support Alliance
Epilepsy Foundation
Global Liver Institute
Hepatitis Foundation International
Hydrocephalus Association
International Cancer Advocacy Network
Kidney Cancer Association
Lakeshore Foundation
Lung Cancer Alliance
LUNgevity
National Alliance for Hispanic Health
National Alliance on Mental Illness (NAMI)
National Council on Independent Living (NCIL)



National Viral Hepatitis Roundtable
No Health without Mental Health
Not Dead Yet
Parent Project Muscular Dystrophy (PPMD)
Parents Reaching Out in New Mexico
Patient Services, Inc.
Patients Rising
RetireSafe
Spina Bifida Association
The Arc of the United States
The diaTribe Foundation
The Hepatitis C Mentor & Support Group, Inc.
Tuberous Sclerosis Alliance
United Cerebral Palsy
United Spinal Association
VHL Alliance