



National Health Council

1730 M Street NW, Suite 500, Washington, DC 20036-4561 ■ 202-785-3910 ■ www.nationalhealthcouncil.org ■ info@nhcouncil.org

BOARD OF DIRECTORS

Chairperson
Tracy Smith Hart
Osteogenesis Imperfecta Foundation

Chairperson-Elect
Cynthia Zagieboylo
National Multiple Sclerosis Society

Vice Chairperson
Robert Gebbia
American Foundation for
Suicide Prevention

Treasurer
Paul Pomerantz, FASAE, CAE
American Society of Anesthesiologists

Immediate Past Chairperson
Randy Beranek
National Psoriasis Foundation

Margaret Anderson
FasterCures –
A Center of the Milken Institute

Marcia Boyle
Immune Deficiency Foundation

Elizabeth Fowler, PhD, JD
Johnson & Johnson

James Greenwood
Biotechnology Innovation
Organization

Kevin Hagan
American Diabetes Association

Barbara Newhouse
The ALS Association

Ann Palmer
Arthritis Foundation

Harold Paz, MD, MS
Aetna

Richard Pops
Alkermes

Eric Racine, PharmD
Sanofi

Gary Reedy
American Cancer Society

J. Donald Schumacher, PsyD
National Hospice and
Palliative Care Organization

Susan Sherman
The LAM Foundation

Steven Taylor
Sjögren's Syndrome Foundation

Stephen Uhl
PhRMA

Ex Officio Member
Marc Boutin, JD
Chief Executive Officer
National Health Council

September 12, 2016
Institute for Clinical and Economic Review
Steven D. Pearson, MD, MSc, President
Two Liberty Square
Ninth Floor
Boston, MA 02109

RE: ICER National Call for Proposed Improvements to Value Assessment Framework

Dear Dr. Pearson,

The National Health Council (NHC) appreciates the opportunity to provide input on the 2017 update to ICER's Value Assessment Framework. The NHC is the only organization that brings together all segments of the health community to provide a united voice for the more than 133 million people with chronic diseases and disabilities and their family caregivers. Made up of more than 100 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 professional and membership associations, nonprofit organizations with an interest in health, and representatives from the insurance, pharmaceutical, generic drug, medical device, and biotechnology industries.

Understanding and defining the value of health care treatments and interventions has become a national priority. The patient community is eager to take part in the value discussion. Patient¹ perspectives on value can differ significantly from other groups such as clinicians and payers. These perspectives often integrate considerations beyond clinical outcomes and cost, such as a treatment's ability to help patients achieve personal goals.

The NHC recognizes ICER's recent efforts to engage the patient community by, for example, outlining a plan for gathering patient input in the scoping documents that inform ICER's reviews and appointing a patient representative to the governance board. However, we strongly recommend ICER adopt an open and collaborative process for identifying and appointing additional patient representation in your governance as well as create additional opportunities for patient engagement.

The NHC offers comments on the four areas ICER has identified as the highest priorities for potential revision to the framework.

¹ Throughout this letter, the term "patient" refers to patients and their family caregivers.

1. Methods to integrate patient and clinician perspectives on the value of interventions that might not be adequately reflected in the scientific literature, elements of value intended to fall in the current value framework within “additional benefits or disadvantages” and “contextual considerations”

We commend ICER for including methods for integrating patient and clinician perspectives as a high-priority area for improving ICER's value assessment framework. The NHC agrees there is a significant gap in appropriate, validated methods to integrate patient and clinician perspectives into value assessments and appreciates ICER's effort to solicit more input in this area.

We are concerned, however, that the scope of this priority as articulated in the call for comments is too narrow and assumes that relevant patient-centered data is widely available for assessment. Specifically, the current scientific literature does not adequately incorporate patient and clinician perspectives, which underscores the need for a paradigm shift in how research is both conducted and evaluated. To imply that the current literature in any way includes appropriate incorporation of patient perspectives misrepresents the state of the field and, unfortunately, downplays the underlying need for gathering and considering these perspectives and the potential impact their inclusion can have on value assessments. This will result in the need for ICER to rely on other means for capturing this information, either directly or through partnerships that include patients and patient groups. We encourage ICER to more openly acknowledge the fundamental deficiencies, gaps, and challenges in this area, which can help set the tone and appropriately frame ICER's efforts to credibly incorporate the consideration of the patient voice in its value assessment process.

Because of these existing deficiencies, gaps, and challenges, it is of utmost importance that ICER develop a robust, systematic process for incorporating the patient perspective into its reviews. We urge ICER to develop this process in partnership with patients, patient organizations, and other experts in the field and make the process transparent and understandable to patients. Doing so will greatly improve the output of ICER's work and lead to greater credibility for the organization.

To facilitate the integration of patient perspectives in value discussions, the NHC convened a multi-stakeholder roundtable to develop a Patient-Centered Value Model Rubric², a tool that the patient community, physicians, health systems, and payers can use to evaluate the patient centeredness and to guide developers on the meaningful incorporation of patient engagement throughout their processes. The roundtable participants identified six domains that encompass patient centeredness:

1. **Patient Partnership**, involving patients in every step of the value model development and dissemination process
2. **Transparency to Patients**, disclosing assumptions and inputs to patients in an understandable way and in a timely fashion
3. **Inclusiveness of Patient**, reflecting perspectives drawn from a broad range of stakeholders, including the patient community.

² Learn more at: <http://www.nationalhealthcouncil.org/patient-centered-value-model-rubric-released>

4. **Diversity of Patients/Populations**, accounting for differences across patient subpopulations, trajectory of disease, and stage of a patient's life.
5. **Outcomes Patients Care About**, including outcomes that patients have identified as important and consistent with their goals, aspirations, and experiences.
6. **Patient-Centered Data Sources**, including data sources that reflect the outcomes most important to patients and capture their experiences to the extent possible.

The NHC strongly recommends ICER develop a formalized patient-engagement process as part of its value assessment framework that addresses the six domains outlined in the Rubric to ensure its value assessments are patient centered. Part of this formalized process could include an open process for stakeholders to nominate individuals from the patient and clinician communities to serve on relevant committees. The NHC would welcome the opportunity to work with ICER on this effort.

The NHC recommends that as part of each assessment, ICER explicitly describe how patient input and preferences were considered and incorporated to help ensure accountability to patients, demonstrate responsiveness to patient input, and help patients better understand the information ICER finds useful. Specifically, ICER should publicly make available its rationale for including or not including submitted comments. We recognize ICER has already made efforts in this area, for example in seeking patient perspectives for the draft scoping document on multiple sclerosis treatments and indicating specifically the aspects of the scoping document (such as choice of interventions or outcomes) that were informed by that input. Understanding why certain patient considerations were included and others were not underpins constructive collaboration. We urge ICER to produce outputs like this to demonstrate the impact of its engagement efforts.

Finally, we understand through recent public comments by ICER staff that the Institute is currently developing a roadmap for advocates to engage ICER. We commend you for this work and encourage you to seek input from the patient community on the roadmap. The NHC and our members look forward to working with you to further strengthen this document and all of ICER's patient-engagement activities.

2. Incremental cost-effectiveness ratios: appropriate thresholds, best practice in capturing health outcomes through the QALY or other measures

The NHC recognizes the importance of evaluating treatments and services to understand their comparative clinical and cost effectiveness. However, we stress that the appropriateness of outcomes selected is critical to the relevance and accuracy of determining value to patients. Whether in the context of QALYs or other measures, ICER should aim to gain a better understanding of whether a QALY and or the data it is based on, are from relevant patients and are meaningful to patients. We caution that endpoints that are translated into value assessments must be derived directly from information provided by patients to be relevant for value determination. Reliance on population-based assessments that do not reflect the heterogeneity of disease subpopulations and patient treatment responses, and patient preferences run the risk of mischaracterizing the imputed value of the treatments being compared. In addition, meaningful endpoints specific to patients and a disease state, such as alleviation of symptoms or the ability to be productive in work or home settings, may not be reflected by global or specific clinical measures that feed into a QALY, losing the opportunity to assess value on patient-centric outcomes. Again, input from the appropriate patient populations for identification of outcomes

that are important to them is critical to support a value assessment approach that is meaningful and has utility for patients.

3. *Methods to estimate the market uptake and “potential” short-term budget impact of new interventions as part of judging whether the introduction of a new intervention may raise affordability concerns without heightened medical management, lower prices, or other measures.*

We are concerned that this priority appears to focus solely on identifying methods that would help assess short-term affordability from the payer perspective and result in restricted access to treatments as an unintended consequence for patients. The NHC urges ICER to, at minimum, consider long-term outcomes and impacts from the patient and payer perspective. While many interventions may have high short-term budget impacts, they may not only greatly improve patient outcomes but can reduce the costs for a patient and the health care system over a longer period of time by reducing the likelihood of more costly interventions and/or poorer outcomes such as frequent hospitalizations and/or surgeries. Many of these cost savings will not be realized for years and will likely be spread between patients – alleviating potential financial hardships – and entities such as private health plans, Medicare, Medicaid, and other social safety net programs.

Focusing on short-term (5 years or less) budget impacts in isolation, de-coupled from approaches that consider longer-term impacts over a lifetime horizon, is not an appropriate or meaningful patient-centered approach to assessing the impact and value of interventions and services. As articulated, this priority appears to focus too narrowly on the short-term impact on siloed costs.

4. *Methods to set a threshold for potential short-term budget impact that can serve as a useful “alarm bell” for policymakers to signal consideration of whether affordability may need to be addressed through various measures in order to improve the impact of new interventions on overall health system value.*

Again, the NHC has serious concerns with focus on short-term budget impact models. We urge ICER to acknowledge through its value assessment process that the measure of value to patients inherently extends beyond the short-term perspective that payers often adopt. We are concerned that emphasizing the budget impact of treatments using assumptions and arbitrary thresholds for short-term budget impact may be used as a rationale to restrict patient access, particularly when they are established without the context of any offsetting long-term benefits that are important not only to payers, but to patients and their families. This is particularly important in cases of chronic conditions, which impact patients during the course of their lifetime.

The NHC also suggests ICER move away from the terminology, “alarm bell,” which might incite knee-jerk reactions that lead to inappropriate access restrictions and other unintended consequences. Shifting to something like “indicator for proactive management to improve impact and outcomes” would be more conducive to collaborative, patient-centered approaches.

The NHC is eager to continue to partner with ICER as it works toward promoting greater patient engagement and integrating patient perspectives in the value assessment process. We would like to thank you for this opportunity to share our comments. Please do not hesitate to contact Eric

September 12, 2016

Page 5 of 5

Gascho, our Vice President of Government Affairs, if you or your staff would like to discuss these issues in greater detail. He is reachable by phone at 202-973-0545 or via e-mail at egascho@nhcouncil.org.

Sincerely,

A handwritten signature in black ink, appearing to read "MBoutin", with a long horizontal flourish extending to the right.

Marc Boutin, JD
Chief Executive Officer