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September 6, 2019

BY ELECTRONIC DELIVERY

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 Value Assessment Methods for Single or Short-Term Transformative Therapies (SSTs)

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 feedback on the, "Value Assessment Methods for Single or Short-Term Transformative Therapies (SSTs)." Founded in 1920, the 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 diverse, 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.

We envision a society in which all people have access to quality health care that respects personal goals and aspirations and is designed around the patient experience to promote their best possible health outcomes. We agree with ICER that methods adaptations are necessary for value assessment of SSTs.

Many of these new therapies have the potential to cure or substantially modify diseases, giving patients hope of a better life. They also come with significant upfront costs with the potential for significant downstream savings. Unfortunately, the organization that pays for the treatment today will rarely be the organization that realizes the future

savings without innovative contracting and financing mechanisms. Thus, traditional value assessment may not truly capture the value of products with longer-term, downstream advantages, creating the need for adapted approaches. Therefore, we appreciate ICER's effort to capture these issues and provide suggested solutions.

Below, we provide our comments on the set of proposed adaptations and recommendations to ICER the proposals. Our comments follow the organization of ICER's August 6, 2019 document.

Introduction

ICER's additional proposed models, sensitivity analyses, and opportunities to engage will add complexity for researchers developing models, but also for stakeholders to provide information for building and providing feedback on the assessments. We strongly suggest ICER partner with members of the patient and research communities to understand realistic timeframes for engaging, providing input, and preparing comments. Since ICER's recommendations may impact patients' access to care in the real world, it is critical that ICER emphasize high-quality methods and not impose unnecessarily aggressive timelines on either the researchers who must conduct the work, nor stakeholders interested contributing valuable insights. Whenever possible, we recommend that a comment period of at least 90 days be offered to allow for the patient community to have adequate time to prepare a thoughtful response. Patient groups may need to convene scientific or medical advisory boards of volunteers or engage large numbers of patients to gather sufficient data to be responsive.

1. Determining those treatments for which adapted assessment methods will be used

We appreciate ICER's effort to offer a definition for SSTs. This is a critically important starting point for this dialogue. We also appreciate that the patient community is an acknowledged partner and that formal public comment will be sought. **It would also be beneficial to have a very clear process articulated that delineates how the patient community will be engaged and at what point(s) in time in the process this will happen, specifying what the patient community role will be.** Since SSTs include those therapies that produce a "transformative health gain," it should be those people and families experienced with living with the condition every day that define what "transformative" means in each context. Patient, caregiver, and family-member input will be a necessary requirement in this definition for each condition considered. We recommend that a clearer pathway for how that will happen be codified and are happy to help collaborate on what that process could look like.

2. Assessing and describing uncertainty

This section describes the use of incremental cost-effectiveness analysis scenarios at multiple time horizons. While we understand the desire to develop a consistent and predictable time horizon, **we believe that it will important to establish time periods that are meaningful to the specific condition and population to be treated.** The examples provided at five or 10 years may or may not be meaningful to a given

condition. It also indicates that, “decision makers may wish to apply their own judgment on the time horizon.” These judgments should not be made independently by payer decision-makers. The time periods should be established with patient and clinical community input to be relevant to the condition and sensitive to meaningful change. This should be part of the process ICER uses when defining what is curative or transformative. Curative or transformative at what time point(s) from the patient and clinician perspective should be part of the earliest dialogue. We recommend these time points be established as part of defining what is curative or transformative for the specific condition.

In section 2.3, introducing a new economic review section on “Controversies and Uncertainties,” we suggest that the phrase, “data on patient outcomes,” be changed to, “data on patient-centered outcomes.” We believe it is also important to indicate which outcomes are important to patients, which typically includes but often extends beyond quality of life. For example, this section would make it transparent that a particular assessment is focused on specific endpoints (e.g., clinical trial endpoints) as data on them are available from clinical trials. But, this section would point out that they are not patient-centered endpoints as patients did not prioritize their importance. We recommend that this clarification be included for transparency to the reader and potential user of the information.

As noted in our 2017 report, “Policy Recommendations for Reducing Health Care Costs,”¹ outcomes-based contracting can be helpful in creating patient access to new therapies. We believe this is especially true of SSTs. However, it is unclear whether ICER’s proposed cut off [of 25% of probabilistic sensitivity analysis (PSA) simulations over \$200,000/QALY threshold] is appropriate or if outcomes-based contracts should be more broadly recommended.

It seems, as well, that PSA is being used narrowly here, and it could inform users by elucidating uncertainty throughout the various inputs to the model across the board. As also mentioned elsewhere in the document, there can be a “most conservative scenario” and “a most optimistic scenario.” Rather than narrow the PSA to one use, to only encourage outcomes-based contracts, which we believe can be very positive for patients, ICER should take advantage of PSA to capture what could be a range of realistic scenarios given the outcomes and time points captured in early patient and clinician engagement. We recommend ICER consider the use of PSA and other appropriate methods to transparently capture and articulate implications of uncertainty about any model input.

3. Additional elements of value

The NHC supports consideration of “additional elements of value.” However, we are concerned these additional elements will be disregarded by decision makers unless they are either considered quantitatively or specifically and transparently highlighted as important/critical caveats to interpreting the entire assessment. For example, NHC

¹ National Health Council. Policy Recommendations for Reducing Health Care Costs. Available from: <http://www.nationalhealthcouncil.org/sites/default/files/NHC%20Policy%20Proposals%20for%20Reducing%20Health%20Care%20Costs%20UpdatedFINAL052217.pdf>

members have seen instances where information or recommendations included in various parts of an ICER value assessment document (such as the section on “Contextual Considerations”) have been ignored by payers since the information was not included in the value-based price calculation. Thus, we recommend ICER consider an approach that either quantitatively considers these elements or sufficiently conveys to potential value-assessment users what the contribution or impact is as a caveat to interpretation of the base case.

We suggest that ICER provide additional information and rationale for the proposal to add “a potential disadvantage for therapies that, if not successful, could reduce or even preclude the potential effectiveness of future treatments.”

While the technical document provides additional detail on many of the other suggested methodological adaptations, we did not find additional data related to this recommendation. We are concerned with it potentially reducing the availability of approved medicines based on attributes of treatments that are not and may, if fact, never be approved. We recommend that ICER reconsider this proposal at this time until its implications can be better understood.

4. Affordability and fair sharing of economic surplus

We appreciate ICER’s effort to “stimulate a broader societal discussion on the use of cost-effectiveness analyses to guide value-based pricing.” We believe this is a discussion that needs to happen in general, not just for SSTs. Here, the conversation is directed at what “appropriate sharing” of the economic surplus from an SST between the innovator and the health system. We believe the conversation should be broader.

We recommend that the term, “shared savings,” not be used in this context. This is a term used by the Centers for Medicare and Medicaid Services (CMS) to refer to some of its value-based payment programs. In the CMS vernacular, this is the savings to CMS generated when providers agree to value-based payment rather than fee for service payment. CMS then shares the savings CMS incurs with those providers who generated the savings. We believe using this term in the circumstance described by ICER will lead to confusion and different term should be used.

We are concerned that potential impact on innovation is not sufficiently considered, which could have significant implications for patients and the potential for having future “choice among treatments with a different balance and timing of risks and benefits.” It would be important to understand how that would also be incorporated into the analysis and its implication for surplus.

Since the discussion on fair sharing of economic surplus must be in a broader societal context, it is not in alignment with ICER’s general approach or approach to SSTs, which focuses on a base case scenario conducted from the payer perspective. It seems that these offsets would actually be retained by the payer in the current payment system and not shared with providers or patients. This discussion would be more in alignment with a base case from the societal perspective. It is incongruent to produce a value-assessment report that primarily provides findings on value to the payer (cost effectiveness from only the payer perspective) and to then insert a tangential discussion

for policymakers where cost offsets are retained by the system. A base case that focuses on the societal perspective better captures outcomes important to the patient community and would be in alignment with a discussion on providing policymakers with information about economic surplus, with the surplus made relevant to society and not only payers.²

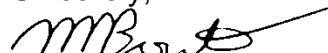
For these reasons, **we believe inclusion of a discussion on fair sharing of economic surplus in ICER value assessment reports is premature and recommend ICER not include the analyses or this section at this time.** That is not to say that we do not think it is important. However, we suggest additional exploration of this topic, to include public dialogue; development of case examples that include SSTs, as well as treatments for rare and chronic conditions; and discussion of how economic surplus has implications for patients in terms of access to current treatments, out-of-pocket costs, and access to future SSTs and “choice among treatments with a different balance and timing of risks and benefits.” The NHC would be happy to collaborate in exploration of these topics.

The NHC welcomes additional opportunities for members of the patient community to engage with ICER. As previously recommended, the impact of patient input and patient-group-submitted data should be clearly articulated in value assessment reports. The current document describes that patient input will be sought, but not how it will be sought or how its impact on the assessment will be described. We believe this is an important aspect of patient-centered value assessment and recommend more detail be provided and added to all future reports.

Our recommendations are intended to increase patient centrality in value assessment. Patient-centered value assessment exists when patients have been engaged, heard, understood, and respected throughout the entire process, and their input is incorporated and guides decision-making. We hope to see even greater impact of patient engagement on value assessment moving forward.

We at the NHC are happy to discuss these comments and recommendations with you, to clarify any suggestions we have 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 Dr. Elisabeth Oehrlein, NHC’s Senior Director of Research and Programs at eoehrlein@nhcouncil.org or 202-973-0540, with any questions.

Sincerely,



Marc Boutin, JD
Chief Executive Officer
National Health Council

² Asthma and Allergy Foundation of America. Asthma and Allergy Foundation of America Responds to Premature ICER Review of New Peanut Allergy Treatments. Available from: <https://www.aafa.org/media/2439/aafa-statement-on-icer-final-report-for-peanut-allergy-therapies.pdf>