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Board of Directors**

June 10, 2019

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Institute for Clinical Effectiveness
and Health Policy
Buenos Aires, ArgentinaSteven D. Pearson, MD, MSc
President, Institute for Clinical and Economic Review
Two Liberty Square, Ninth Floor
Boston, MA 02109**President-Elect**Nancy J. Devlin, PhD
University of Melbourne
Melbourne, Australia

Dear Dr. Pearson:

ISPOR is pleased to respond on behalf of its membership to the call for comments on ICER's overview of planned update to its value framework.

Past PresidentShelby D. Reed, RPh, PhD
Duke University
Durham, NC, USA

ISPOR is a scientific and educational society with many of its members engaged in some aspect of health economics and outcomes research (HEOR) related to evaluation of pharmaceuticals. Our membership includes over 20,000 individuals across a range of disciplines, including health economics, epidemiology, public health, pharmaceutical administration, psychology, statistics, medicine, and more, from a variety of stakeholder perspectives, such as the life sciences industry, academia, research organizations, payers, patient groups, government (including some HHS employees), and health technology assessment bodies. The research and educational offerings presented at our conferences and in our journals are relevant to many of the issues and questions raised in this request for information.

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This response was formulated with the assistance of ISPOR's most senior and representative Council, the Health Sciences Policy Council. It was reviewed by and approved by our current President and myself. Given the somewhat limited response period and its overlap with our Annual International Meeting, however, we were unable to conduct the poll of membership that we typically do for such consultations, and responded only to a limited set of items. The entire value framework is of great interest to ISPOR and its members and we would be happy to engage in further consultation in this area, and will endeavor to respond more fully when the full revision is released in August. We would also welcome conference submissions or other suggestions for broadening the discussion about these issues.

ISPOR would be happy to answer any questions about our response. Please consider Richard Willke, PhD, our Chief Science Officer, as the contact person in this area.

Sincerely,

Nancy S. Berg
CEO & Executive Director
ISPOR**Treasurer (2013-2020)**Zeba M. Khan, RPh, PhD
Celgene Corporation
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ISPOR responses to specific aspects of ICER's value framework updates

4. Other Benefits or Disadvantages and Contextual considerations

We encourage ICER's plan to continue consideration of contextual factors of value in a pilot fashion. In its recent report, ISPOR's Special Task Force on US Value Assessment Frameworks encouraged consideration of many of these same "novel" elements of value, as well as the deliberative processes that could help enable their use in decision-making, while also acknowledging that they generally need further research and testing (Lakdawalla et al, 2018; Phelps et al, 2018; Garrison et al, 2018). We note a few things, however. Your set of potential elements does not include the aspect that is often termed the "value of hope," ie, the situation where a therapy may not help a majority of patients (so it doesn't improve median survival), but does significantly help, or cure, some proportion of patients – the "thick tail" phenomenon. It has been shown that patients show significant willingness to pay for that feature of therapy, and ASCO has included that consideration in its value framework (Shafrin et al, 2017). Another value element not directly mentioned is the value of risk protection, though that value does seem to be highest for diseases with a high burden of illness, a factor that you do include (Lakdawalla et al, 2017). In many cases these factors can be quantified in an augmented cost-effectiveness analysis or net monetary frameworks; it may be useful to build up a set of case examples here to learn more about them. We would also recommend reconsideration of piloting an MCDA-like approach to help quantify the influence of such factors – while those weights can vary by approach, such processes can provide insight into the relative importance of those factors. Finally, the Second Panel on Cost-Effectiveness in Health and Medicine provides an "Impact Inventory" which should serve as a reference point for other types of societal benefits (eg, educational, legal) in selected cases.

6. Report Development

While this comment may go beyond the current bounds of the "Report Development" part of your value framework, we believe there is another scientific aspect of your report development process that merits discussion, even though it is also related to the scoping process described in other ICER documents.

Specifically, ICER typically starts their process in their "scoping" phase with an existing disease state model in mind (in part, presumably, because the evaluation process can be done more quickly if based on an existing model). However, there may be situations where novel, new treatments have a significant impact on patient outcomes and mortality through a mechanism that is not considered in the existing model, so a new or revised model is warranted to properly evaluate the new treatment. This information comes out in the manufacturer interactions during the scoping phase and in the manufacturer's comments to the Scoping draft. The challenge for ICER and the modeling team is that their standard timelines are based on the assumption that they will use an existing model. We would suggest, given an important priority being the relevance and accuracy of the model being used for evaluation, that it would be helpful to get input as early as possible on whether ICER has the right model for the novel, new treatments BEFORE the draft Scoping document ... and/or build in some flexibility with respect to report development timelines.

7. Patient Engagement

With regard to this section and ICER's efforts to include patients and the public in the value assessment process, we commend ICER for directly addressing this important component of value assessment. We recommend that ICER continue to build upon the vehicles for incorporating patient input into the value assessment process, recognizing and honoring that patients with critically important perspectives are not necessarily well-grounded in the concepts of health economics, or even in the existence of ICER as a body. It is imperative that communication and outreach efforts are co-designed by patient partners, to ensure that they are understandable and relevant to patients. We also suggest that, just as highlighting in the Draft Evidence Report indicates where changes have been made *based on* patient and other input, it would be incredibly useful to highlight areas where patient input was collected but did not change the end result, and why that was the case, including a discussion about the nature, construct, and source of the PGHD and what the assessors found lacking. Finally, providing well-advanced notice to patients and facilitating travel for patients to attend in-person meetings is important. Without assistance, only patients with financial resources will be able to attend and the discussion will lack a critical voice—particularly within the ambit of a cost conversation.

8. Identification of low-value services as part of evidence review process

In general we agree with and encourage this approach. Of course, there are some guideposts here, as expressed in our Special Task Force Report (Willke et al, 2018):

“An efficient way to address budget constraints would be to reduce spending on, or to replace, technologies with less favorable cost-effectiveness ratios in favor of budget-expanding but more cost-effective technologies. This could be achieved by price reductions on new technologies, by utilization management targeting less cost-effective subgroups of patients, or by disinvestments in less cost-effective treatments. A lower cost-effectiveness threshold could be set (given some uncertainty about pending new treatments and the success of price reduction and other efforts) that would help achieve the needed overall budget [32]. Any new products (including the new budget-expanding technology in question) as well as existing technologies that could be subject to disinvestment, could be held to that new standard. Ideally, an affordability strategy should examine the entire medical care portfolio subjecting all technologies to the same opportunity cost criterion, rather than assuming that budget savings can be achieved by restricting the price or utilization of technologies that meet the affordability criteria. Barriers to reducing price or to disinvestment include high transaction costs associated with reducing the use of established technologies within health systems and equity concerns if the technologies of interest are the only effective options for patients with specific conditions.”

Ideally, one would be able to utilize real world costs and outcomes for existing technologies to identify those services that have turned out to be low value in practice. Over time, use of some technologies may evolve to their most efficient uses relative to initial approval, or, by contrast, spread too widely to largely inefficient uses, and prices often change after initial studies are done. We realize that such studies are not done as much as they should be, and in the absence

of randomization, careful analysis is necessary, so reliable real-world evidence may not be easily available, but we encourage consideration of real-world evidence when feasible.

References

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