

May 26, 2017

Richard J. Willke  
Chief Science Officer  
International Society for Pharmacoeconomics and Outcomes Research  
505 Lawrence Square Blvd. South  
Lawrenceville, NJ 08648

Dear Dr. Willke:

I am writing on behalf of the Partnership to Improve Patient Care to express our strong concerns about the recent draft of a U.S. Value Assessment Frameworks Initiative led by the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) whose mission “advances the policy, science, and practice of pharmacoeconomics (*health economics*) and outcomes research (*the scientific discipline that evaluates the effect of health care interventions on patient well-being including clinical, economic, and patient-centered outcomes*).”<sup>1</sup> It is therefore troubling to see ISPOR endorsing methodologies for assessing treatment value that reflect the past and not the future. The recommendations do not reflect advancement as is indicated to be ISPOR’s mission.

Since its founding, the Partnership to Improve Patient Care (PIPC) has been at the forefront of applying principles of patient-centeredness in comparative effectiveness research (CER). Central to our mission is ensuring that the patient voice is heard in judgments about care value – whether in the context of comparative effectiveness research or emerging “value-based” payment incentives. From this experience, PIPC continues to work to bring the voices of patients, people with disabilities, and their families to the discussion of how to advance patient-centered principles throughout an evolving health care system. We understand the challenge of assessing value in health care in a manner that is centered on the characteristics, needs and preferences of the individual patient versus defining value based on what is cost effective for an “average” patient, and appreciate that the work that the ISPOR Special Task Force on Value Assessment has undertaken is difficult. That said, we have significant concerns regarding the recommendations that ISPOR outlines in its draft report.

### **The Shortcomings of Quality-Adjusted-Life-Years (QALYs) Are Well-Recognized Among Patients, Disabled Persons, and Policymakers**

PIPC was hopeful that the Special Task Force was an opportunity to further advance novel, patient-centered methods for assessing treatment value. We are disappointed that ISPOR instead doubled down on the use of the cost-per-QALY metric in direct contradiction to the patient-centeredness movement. Although the full paper rightfully explores alternatives to QALYs, including Multi-

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<sup>1</sup> <https://www.ispor.org/about-ispor.asp>

Criteria Decision Analysis, the top-line recommendations move in the opposite direction to fully endorse the use of QALYs. Those recommendations only serve to undermine the real work of thoughtful leaders to advance a patient-centered health system by developing a new paradigm for assessing the value of health care from a patient perspective.

PIPC is particularly concerned about the implications of health policy built on population-based value assessments on people with disabilities and patients with chronic conditions who may or may not be cured, but regardless are seeking access to treatments and health interventions that improve their quality of life. People with disabilities and patients with chronic conditions have a long history opposing the use of quality-adjusted-life-years (QALYs) as the benchmark to measure the value of health care interventions. As applied to real-world policy and decisions about access and coverage, QALY-based analyses often conclude that the lives of patients and people with disabilities are not valuable. This in turn leads to unnecessary and harmful limitations on access to care. Such simplistic average measures of value are perceived to reinforce the old paternalistic system of health care and work against the movement toward more personalized, individualized health care.

Policy-makers recognize the dangers of misusing cost-effectiveness standards in ways that undermine high-quality, individualized care. For example, the Patient Protection and Affordable Care Act (ACA) explicitly prohibits the Patient-Centered Outcomes Research Institute (PCORI) from “[developing] or [employing] a dollars per quality adjusted life year (or similar measure that discounts the value of life because of an individual’s disability) as a threshold to establish what type of health care is cost effective or recommended” and further restricts the use of QALYs by mandating that “the Secretary [for Health and Human Services] shall not utilize such an adjusted life year (or similar measure) as a threshold to determine coverage, reimbursement, or incentive programs” in the Medicare Program.<sup>2</sup>

Alternatively, PIPC aims for policymakers to focus on health care payment and delivery reforms that activate and engage patients and people with disabilities and that support shared decision-making between them and their providers. We believe that solutions that center on patients and people with disabilities are the best approach to improving overall health care efficiency and quality. We are very excited about the work underway to develop alternative and more patient-centered methodologies for assessing value to the patient.

With this background, we are particularly concerned that the ISPOR process for this initiative lacked consistent and meaningful patient and stakeholder engagement. While PIPC’s Executive Director was invited to provide input early in the process, we are unable to track any engagement since the initial survey. Our views about value assessment are front and center on our website and our concerns about the use of QALYs as a measure of treatment value have been consistently communicated in our work, yet are not reflected in your recommendations.

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<sup>2</sup> 111th Congress of the United States of America, *H.R. 3590 The Patient Protection and Affordable Care Act*, vol. Section 1182, Washington, DC, 2010.

## Recent Efforts to Drive Patient-Centered Approaches to Value Assessment

As the Special Task Force moves forward with its work, PIPC recommends that ISPOR review the robust and thoughtful value assessment-related work currently underway by members of the patient community. This work could inform the work of ISPOR's Special Task Force and help guide them in a more patient-centered direction. A few examples of work being done to truly advance health economics and outcomes research to reflect principles of patient-centeredness include:

- **PIPC Roadmap to Increased Patient Engagement in Value Assessment:** PIPC's issue brief builds on existing calls for patient engagement by providing a guide to developers seeking to create value frameworks and tools that can become the foundation of a truly patient-centered health system. The paper describes best practices in procedures for conducting value assessments that are patient centered; it does not address the equally important issues related to methods used for value assessment. Specifically, we identified seven key opportunities for improvement through increased stakeholder engagement:
  - Obtaining Stakeholder Input in Priority Setting
  - Identifying and Employing Patient-Relevant Outcomes and Endpoints
  - Structure and Use of Advisory Panels
  - Soliciting and Responding to Stakeholder Comments During Conduct of Value Assessments
  - Ensuring Transparency in Approach and Methods
  - Engaging Patients in Dissemination of Results
  - Structured Assessment Review Cycles<sup>3</sup>
- **Roadmap to Consumer Clarity:** The National Patient Advocate Foundation (NPAF) is developing a Roadmap to Clarity in Consumer Decision Making (the Roadmap), a patient-centered framework that will increase awareness of the full spectrum of changes needed for consumers to have the information and support to engage in health care decisions effectively. Through literature reviews, work with patients, and guidance from an advisory group, NPAF is developing the Roadmap and raising awareness about the Roadmap and help other groups working in the field to understand and use it. PIPC has participated as a stakeholder in the Roadmap advisory group, which has consisted of a transparent and engaged process lasting over a year. The Roadmap proposes models to foster patient-centered care and shared decision making. We would encourage ISPOR to engage with NPAF and also incorporate their expertise on how patients assess value in health care.<sup>4</sup>

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<sup>3</sup> See [http://www.pipcpatients.org/uploads/1/2/9/0/12902828/pipc\\_value\\_frameworks\\_white\\_paper\\_-\\_a\\_roadmap\\_to\\_increased\\_patient\\_engagement\\_in\\_value\\_assessment.pdf](http://www.pipcpatients.org/uploads/1/2/9/0/12902828/pipc_value_frameworks_white_paper_-_a_roadmap_to_increased_patient_engagement_in_value_assessment.pdf)

<sup>4</sup> see <http://www.rwjf.org/en/library/grants/2016/02/working-to-provide-patients-with-high-quality-health-care-inform.html>

- **National Health Council:** The National Health Council (NHC) has several initiatives to advance a patient-centered health system. The NHC has undertaken an initiative to address current barriers to patient engagement through a multi-stakeholder approach by building a consensus-based conceptual framework for patient engagement and agreement on best practices in research and development in drug development. The NHC also developed an information collection tool to help patient advocacy organizations systematically capture and organize patient concerns and comments about the benefits and risks of treatment options. With stakeholder input, NHC has also created a Value Model Rubric to help evaluate the patient centeredness of value models and to guide value model developers on the meaningful incorporation of patient engagement throughout their processes. Their expertise should be central to your work on value frameworks.
- **Patient Perspective Value Framework:** *FasterCures* and Avalere Health have partnered to develop the first patient-centered value framework. PIPC is proud to participate in an advisory role in their work. The newly released Patient Perspective Value Framework (PPVF) incorporates measures of benefits and costs in the context of patients' personal goals and preferences to assess the value of different health-care treatment options. The PPVF has five broad domains of value, each of which contributes different types of information important to patient decision-making. This work truly advances the conversation about how treatments are valued in a manner that is consistent with the goals of personalized medicine and patient-centeredness, and should be a reference for your work as well.<sup>5</sup>

The above examples reflect the commitment of the patient community to participating in a meaningful way in the evolution to value-based payment models. We believe that value and patient-centeredness are not conflicting goals. We know that there are positive implications for the overall cost of care when patients get the right care at the right time, thereby avoiding unnecessary care, increasing adherence and decreasing adverse events and hospitalizations. It is exciting that so much work is being directed to ensuring that value and patient-centeredness are aligned in new models of payment and delivery, including the increased emphasis on comparative clinical effectiveness research and shared decision-making.

Yet, at this stage of ISPOR's process, we are faced with differing views among our members as to how to proceed. Some urge us to stay engaged and to work to improve the outcome of the initiative to reflect patient perspectives. Others have advised that our best path forward is to focus our attention on initiatives that are consistent with our principles for patient centeredness and that meaningfully engage patients. In reality, we will not make an impact on your work if you choose to dismiss our input.

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<sup>5</sup> see <http://www.fastercures.org/reports/view/66>

Therefore, PIPC urges ISPOR to develop and implement a process for patient engagement that reflects the best practices modeled by organizations like PCORI, FDA, and others described above. With a meaningful patient engagement infrastructure in place moving forward, PIPC is excited to continue to be an engaged stakeholder in the U.S. Value Assessment Frameworks Initiative. If ISPOR has no intention for meaningful engagement, we will focus our energy on initiatives that seek to advance a patient-centered health system.

Please also note that PIPC plans to publish an issue brief related to use of QALY's in the near future. Unfortunately, we were unaware of ISPOR's pending publication of its draft and the recommendations in it. We hope that ISPOR will consider the views communicated in that paper when we publish it in the near future as part of its engagement.

I look forward to learning more about the path forward that ISPOR chooses in this work. Please reach out to me or to Sara van Geertruyden ([sara@pipcpatients.org](mailto:sara@pipcpatients.org)) if ISPOR chooses a path of meaningful engagement as we are eager to share our work and our views with your team.

Sincerely,



Tony Coelho  
Chairman, PIPC