



April 3, 2017

Steven D. Pearson, MD, MSc
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Dear Dr. Pearson:

The Partnership to Improve Patient Care (PIPC), along with the undersigned organizations, appreciates the opportunity to provide feedback on the proposed revisions to the Institute for Clinical and Economic Review's (ICER) value framework. Amidst the debate about assessing and measuring the value of interventions and treatments, it is important to remember the reason we are having those conversations – the patient.

Patients and their advocates will be the first to say that value is critical – improving health outcomes and making care efficient is a priority for our communities as well. We agree with ICER that stakeholders at all levels of health care require frameworks and tools to support their decision-making and achieve value, including payers. But regardless of the purpose of the framework or tool, or its intended audience, whether it's payers, providers, or patients, it is crucial that the framework is patient-centered.

Although we appreciate that ICER has solicited comments from stakeholders on its value assessment framework, we also believe there are additional steps that ICER must take beyond its proposed revisions to ensure that its framework achieves true patient-centeredness. Those steps include:

- Take additional steps to meaningfully engage stakeholders, particularly patients and people with disabilities, in its value assessment process as an ongoing dialogue, such as through meaningful engagement of patients and clinical experts in the voting process as well as early pro-active engagement beginning with the scoping process.
- Refrain from relying on cost-per-QALY based cost effectiveness analyses, which undervalues the lives of patients and people with disabilities.

- Recognize that populations are comprised of individual patients and take steps to account for variability among patients and people with disabilities in terms of preferences and clinical characteristics.
- Emphasize a long-term perspective that benefits patients and people with disabilities, payers and society.
- Devise a patient-centered, transparent ratings system to communicate value that is consistent with the recommendations of the patient and disability communities.
- Broaden the types of evidence relied upon, including patient-reported data, and update frameworks based on real world evidence.
- Develop a valid methodology for additional benefits and disadvantages that is updated based on input from patients and people with disabilities.

As it stands, ICER's framework risks impeding patient access by encouraging payers to impose blunt, one-size-fits all standards on patient-provider decision-making. Below we describe our outstanding concerns and propose steps ICER should take to address those concerns.

ICER should take additional steps to meaningfully engage stakeholders, particularly patients and people with disabilities, in its value assessment process.

We appreciate that ICER has made some progress in engaging stakeholders, particularly patient organizations, but remain concerned that, in many ways, patients and clinical experts continue to be disenfranchised from meaningfully engaging in the value assessment process. We describe here several areas where we believe there is room for improvement in ICER's engagement infrastructure.

First, it is a step in right direction to allow patient representatives and clinical experts to join the independent appraisal committee for the entire meeting. It is important that a range of perspectives, and the right perspectives, are represented. We are therefore concerned that it is not clear from the proposed revisions or the patient engagement guide what expertise is required to serve on an independent appraisal committee, such as level experience with the condition related to the treatment under consideration. In addition to ensuring that the qualifications required to participate and the selection process is transparent, it is also important that a range of perspectives is represented. Where possible, a range of patient representatives that have expertise as patient organizations representing the affected patient population as well as individual patients should bring their personal experience to the discussion. Additionally, the clinical experts should be recommended and supported by the patient representatives as truly having the relevant expertise needed to inform the independent committees about the condition being treated.

We also appreciate that patient groups will be given the opportunity to present the results of their own evidence generation through patient-reported outcomes and surveys on other benefits or

disadvantages. It is not clear from ICER's draft at what stage that information is considered. For patient data to be meaningfully considered, it should be sought out and considered early in the scoping and topic prioritization stage so that it is informing the focus of ICER's work, as well as being incorporated into the evidence synthesis from the beginning of the process, and then again as ICER determines the benefits and disadvantages that represent outcomes that matter to patients. Engagement of patients and people with disabilities is not a "one off" activity and should instead be considered an ongoing dialogue.

Lastly, ICER outlines a process for value assessment that includes independent public appraisal committees, and further input from patient representatives, clinical experts and other stakeholders. Yet, it appears that ICER will not permit patients and relevant clinical experts to vote on the value of treatments under review. We strongly encourage ICER not to isolate patients and clinical experts from the ultimate determination of what is valuable and what is not.

Recommendations: We urge ICER to formalize its engagement process with the following specific steps:

- Provide a timeframe for input from patient representatives and clinical experts that allows for input to be considered and incorporated in a timely manner to inform its topic selection, evidence synthesis, measured outcomes, draft report and final report.
- Invite to independent appraisal committee discussions a range of patient representatives that have expertise as patient organizations representing the affected patient population as well as individual patients with personal experience, and give them a vote.
- Ensure clinical experts are recommended and supported by the patient representatives as truly having the relevant expertise needed to inform the independent committees about the condition being treated, and give them a vote.
- Propose criteria for those participating in independent public appraisal committees so that the committees are made up of individuals directly experienced with the conditions being treated.
- Seek out and meaningfully consider and give weight to patient data throughout the value assessment process, beginning with the scoping process.

ICER should refrain from relying on cost-per-QALY based cost effectiveness analyses, which undervalues the lives of patients and people with disabilities.

We are pleased that ICER is considering supplementing its cost per quality-adjusted-life-year (QALY) calculation with additional scenario analyses and cost consequence analysis. However, we remain concerned that these changes alone are not enough to overcome the inherent limitations of QALY-based cost effectiveness analyses which fails to capture the value of treatments for people with disabilities and patients with serious chronic conditions. While the QALY is

theoretically based on patient preferences, it is widely accepted that the generic-preference based measures that form the foundation of traditional QALYs are inadequate at capturing preferences among patients with chronic health conditions and people with disabilities, or even how patients' preferences may change over time as they become more familiar with managing their health.¹

We remain concerned that the theoretical underpinning of the QALY is that something as ephemeral as quality of life can be measured and distilled down to a single number. Of course, quality of life is a concept that philosophers, scientists, and policy-makers have struggled with through the ages, making it hard to imagine that the QALY could capture the value of a person's life in a single number. Thus, while the simplicity of the QALY makes it a deceptively attractive metric for researchers, this very simplicity means it is, by definition, an inherently limited measure of the complexities surrounding patient preferences and values. Indeed, because the QALY was developed as a theoretical tool for academia, it allows for "states worse than death," which is at odds with the more pragmatic way that most individuals view their own health.^{2 3}

The disconnect in using an academic tool to influence real-world policy can be seen in how QALYs are measured and calculated. Individuals surveyed about theoretical scenarios may be unable to imagine the value of their lives in a particular state of health or what they are willing to trade to treat a hypothetical health condition or symptom. A recent survey found that among more than 2,000 American adults who were asked to imagine losing their vision, nearly half considered blindness to be worse than death, HIV infection, or the loss of a limb.⁴ While blindness can undoubtedly have a significant impact on quality of life, it is also generally accepted that with support and training, individuals who are blind can "be as happy and lead as full a life as anybody else".⁵

Recommendations:

- Given the limitations of QALY-based cost effectiveness analyses, explore other more transparent, patient-centered methods for calculating the benefit of treatments, other than QALY-based cost effectiveness.

¹ M. Janssen, A. S. Pickard, D. Golicki, C. Gudex, M. Niewada, L. Scalone, P. Swinburn and J. Busschbach, "Measurement properties of the EQ-5D-5L compared to the EQ-5D-3L across eight patient groups: a multi-country study," *Qual Life Res*, November 2012.

² M. J. Harrison, L. M. Davis, N. J. Bansback, M. J. McCoy, T. M. Farragher, S. M. M. Verstappen, A. Hassell and D. P. M. Symmons, "Why Do Patients With Inflammatory Arthritis Often Score States "Worse than Death" on the EQ-5D? An Investigation of the EQ-5D Classification System," *Value in Health*, vol. 12, no. 6, pp. 1026-1034, 2009.

³ S. J. Whitehead and S. Ali, "Health outcomes in economic evaluation: the QALY and utilities," *British Medical Bulletin*, vol. 96, no. 1, pp. 5-21, October 2010.

⁴ A. Scott, N. Bressler, S. Ffolkes, J. Wittenborn and J. Jorkasky, "Public Attitudes About Eye and Vision Health," *JAMA Ophthalmology*, vol. 134, no. 10, pp. 1111-1118, October 2016.

⁵ Washington State, "Department of Services for the Blind," [Online]. Available: <http://www.dsb.wa.gov/resources/dispelling-myths.shtml>.

- Articulate the significant limitations of a cost-per-QALY analysis in ICER reports, and emphasize the range of treatment value that may be experienced by real patients and people with disabilities with unique characteristics.

ICER should recognize that populations are comprised of individual patients and people with disabilities.

ICER acknowledges that its value framework takes a “population” level perspective, and is intended to support payer-level decision making, as opposed to trying to serve as a shared decision-making tool to be used by individual patients and their clinicians. Although payers must make decisions at a population level, those decisions do not have to ignore patient differences, and they do not have to conflict with the movement toward individualized patient care. We live in an era of personalized medicine, in which there is increasing recognition of the significant diversity in patient preferences, goals and treatment effects. We urge ICER to take steps to ensure that its framework is accounting for variability and heterogeneity within patient populations, and recognize the limitations of its use for payer decision-making. If applied inappropriately, ICER’s framework could have the effect of bluntly limiting patient access to important treatments that suit a specific patient’s medical needs. In the long run, patients and people with disabilities are better served in a patient-centered health system that allows for access to care tailored to the individual, not to the average patient. While ICER acknowledges the intended use of its reports, ICER does not iterate a strategy to prevent misuse of its reports and the perhaps unintended implications for individualized patient care.

Recommendations:

- Acknowledge the limitations of its analyses in recognizing patient differences, and take steps to account for variability among patients and people with disabilities in terms of preferences and clinical characteristics.
- Consider steps to mitigate the unintended and potentially devastating implications of its reports for patients and people with disabilities that do not fit neatly into its model of averages.

ICER should emphasize a long-term perspective that benefits patients, payers and society.

We are pleased that ICER now recognizes that the benefits for patients and potential cost offsets for new treatments might take many years to be seen. As indicated by ICER, payers often view the value of treatments through the lens of actuarial value – a short time-frame – making it incumbent upon value framework developers to view the value of treatments through the lens of chronically ill patients and people living with disabilities whose conditions are not necessarily resolved in 1-2 years. We appreciate that ICER’s short-term assessments were extended to 5 years, and urge ICER to consider a stronger emphasis on the long-term/lifetime perspective.

Factors such as the potential of a treatment to avoid disability or further disability, or to enable a person to live independently, or to work or to act as a caregiver have significance for the personal and societal costs of disease and disability. As a society, we have an ethical obligation to support individuals to live to their own full potential and our assessments of treatment value should underscore the ethical considerations for treatment as well. In doing so, we would also potentially avoid the long-term costs of further disability, unemployment and loss of independence. Yet, it is important that we look directly to impacted patients for insights on the long-term costs and outcomes that represent the real-world tradeoffs they experience and prioritize. Those tradeoffs may vary over time spent with condition, so it is vital to ask patients and people with disabilities about their priorities on multiple occasions over time.

Recommendation: We appreciate that ICER’s short-term assessments were extended to 5 years, and recommend ICER to consider a stronger emphasis on the long-term/lifetime perspective.

ICER should devise a patient-centered, transparent ratings system to communicate value that is consistent with the recommendations of the patient and disability communities.

We are very concerned that although ICER claims it separates “long term value for money” and “budget impact” for the purpose of its framework, it continues to merge the concepts in its value-based price benchmark. A single number cannot capture the value of a treatment across the entire population. Every patient is different and will weigh the benefits and disadvantages of a treatment differently. We urge ICER to consider the National Health Council’s Patient-Centered Value Rubric⁶, the Patient-Perspective Value Framework created by FasterCures⁷, and PIPC’s Roadmap to Increased Patient Engagement in Value Assessment⁸ as guides for assessing value in a manner that is more patient-centered.

ICER asserts that it takes into consideration net budget impact across the health system. To truly do so, it is important that ICER recognize that health system costs may not just be costs borne by the payer. Patients may be paying out-of-pocket for certain services that allow them to manage their condition or adhere to treatment (over-the-counter products, transportation, wellness services, etc.), or that payers do not recognize as being related to a certain treatment and therefore not considered to be related to that treatment’s bundle of health costs (mental health, services related to comorbid conditions, etc.) To achieve a more holistic view of costs associated with treatment, a broad scope of patients must provide input.

⁶ See <http://www.nationalhealthcouncil.org/sites/default/files/Value-Rubric.pdf>

⁷ See <http://avalere.com/expertise/life-sciences/insights/releasing-the-draft-avalere-fastercures-patient-perspective-value-framework>

⁸ 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

Recommendations:

- Devise a more patient-centered ratings system than the value-based price benchmark.
- We urge ICER to consider the National Health Council’s Patient-Centered Value Rubric⁹, the Patient-Perspective Value Framework created by FasterCures¹⁰, and PIPC’s Roadmap to Increased Patient Engagement in Value Assessment¹¹ as guides for communicating value in a manner that is more patient-centered as its framework evolves.

ICER should broaden the types of evidence it relies upon, including patient-reported data.

ICER states that it has a “flexible and ecumenical approach to sources of evidence and...ICER’s methods incorporate multiple sources and types of evidence, seeking the evidence that is most helpful in understanding the long-term net health benefits for patients of different care options.” We appreciate that ICER is beginning to recognize the value of patient-reported data and long-term registries and that “all too often what matters most to patients is poorly captured in the available clinical trial data.” Academia tends to place more significant weight on the randomized clinical trial (RCT), which, although rigorous, tends to represent a narrow patient population, underrepresent patients with multiple comorbidities, and measure only certain clinical outcomes.

Additionally, it is important to consider how quickly RCT’s become outdated as eventually real-world evidence gives us more timely and accurate evidence on which to base decisions. As discussed above, we must recognize that such challenges impede our very ability to assess value of a treatment, and instead focus on partnering with patients and people with disabilities to improve the real world evidence base.

We remain concerned that ICER has a very academic view of the evidence that may be relied upon for assessing value. ICER stated its plans to include an evaluation of the heterogeneity of treatment effect for key clinical outcomes where possible. Where not possible, we hope that ICER will look to patient organizations for the best available evidence, even if it is not from an academic source, to inform its work and explicitly identify such evidence gaps to inform future research priorities.

Recommendations:

- Look to patient-centered sources of data for the best available evidence on the real-world implications of treatments for those outcomes. Patient organizations with large networks have the capacity to survey those networks and collect information on patient-reported outcomes that can provide more timely data on the experiences of patients in the real world.

⁹ See <http://www.nationalhealthcouncil.org/sites/default/files/Value-Rubric.pdf>

¹⁰ See <http://avalere.com/expertise/life-sciences/insights/releasing-the-draft-avalere-fastercures-patient-perspective-value-framework>

¹¹ 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

- Where evidence is lacking on heterogeneity of treatment effect, look to patient organizations for insights and clearly identify the evidence gap for future research priority-setting.
- Continuously update value frameworks to represent emerging real-world evidence.

Develop a valid methodology for additional benefits and disadvantages that is updated based on input from patients and people with disabilities

While we appreciate the intent, we are very concerned that incorporation of additional benefits and disadvantages and the 10 factors is fundamentally flawed. It is not clear that the method that ICER proposes is rigorous considering that it is feeding a “single number” quantitative analysis, nor has it been tested before to ensure that it is valid. We are also very concerned that the result will not incorporate the views of the patient and disability communities, who are not permitted to vote as part of the independent appraisal committee.

It is also not clear how the list of 10 factors of additional benefits and disadvantages may be updated and improved by the input of impacted patients and relevant clinical experts. It will be important that the factors are specific to diseases, conditions and the population of patients and people with disabilities, as no two conditions or illnesses are alike.

Recommendations:

- Seek more patient-centered strategies for measuring and incorporating additional benefits and disadvantages into a value framework that reflect the range of priorities for care outcomes.
- Test this methodology, and any others under development, to demonstrate its effectiveness before it is used for publicly available value assessments that will be used by payers.
- Look to patient organizations for insights from their patient communities on outcomes that matter to patients and people with disabilities, and that could supplement the 10 elements of value.

Conclusion

We remain concerned with the potential use of value assessments that are based on cost assessments of long term value for money and budget analyses. We recognize the effort put forth by ICER to address the concerns of patients and people with disabilities and recognize that culture change is not easy. ICER has an opportunity to lead a culture change that drives research and care delivery models designed to prioritize the needs, values and preferences of the individual patients and people with disabilities whom the health system is intended to serve. No one benefits if patients are forced to fail on a treatment before accessing the treatment that will work for them – there is no value for money or budgets or people when that happens. Helping people live life to their fullest is not just the right thing to do, but also is ultimately cost effective as we promote

treatment adherence, decrease adverse events and hospitalizations, and promote increased productivity and participation in society.

We also have provided a copy of PIPC's Roadmap for Patient Engagement in Value Assessment which provides additional information about models for patient engagement for your review in this process. Thank you for your consideration of our recommendations. We look forward to continuing to provide input on the evolution of ICER's process to be more patient-centered.

Sincerely,

Tony Coelho
Chairman, Partnership to Improve Patient Care

Signed by:

[Alliance for Patient Access](#)

[ALS Association](#)

[American Association of Neurological Surgeons](#)

[American Autoimmune Related Diseases Association](#)

[American Foundation for the Blind](#)

[Arthritis Foundation](#)

[Association of University Centers on Disabilities](#)

[Autism Society of America](#)

[Autistic Self Advocacy Network](#)

[Center for Autism and Related Disorders](#)

[Christopher & Dana Reeve Foundation](#)

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[Immune Deficiency Foundation](#)

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[Lupus and Allied Diseases Association](#)

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[National Alliance on Mental Illness](#)

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[Parent to Parent USA](#)

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[Spina Bifida Association](#)

[The ARC of the United States](#)

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