October 15, 2019

Steven Pearson, MD
Institute for Clinical and Economic Review
2 Liberty Square, Ninth Floor
Boston, MA 02109

RE: 2020 Value Assessment Framework Proposed Changes

Dear Dr. Pearson:

Aimed Alliance is a 501(c)(3) non-profit health policy organization that seeks to protect and enhance the rights of health care consumers and providers. Aimed Alliance respectfully submits the following comment in response to the "2020 Value Assessment Framework Proposed Changes" ("Proposed Changes") published by the Institute of Clinical and Economic Review (ICER) on August 21, 2019.

## I. ICER Should Revise Its Value Assessment Framework to Provide an Adequate Mechanism for the Inclusion of Real-World Evidence

Real-world evidence is emerging as an important consideration in drug development, regulatory approval decisions, and coverage decisions. The uses of real-world evidence include measuring adherence, establishing effectiveness among subpopulations, and establishing clinical and cost effectiveness within a health plan's specific population.[1] ICER explains that it will assess the validity of real-world evidence and how such evidence should be incorporated into an assessment. ICER also intends to generate new real-world evidence for incorporation into its reviews.

Aimed Alliance is concerned that the Proposed Changes do not provide an adequate mechanism for the inclusion of real-world evidence into ICER's cost-effectiveness review. ICER's value assessments often occur before or shortly after the U.S. Food and Drug Administration (FDA) approves a therapy. As such, there is simply not adequate real-world evidence available for meaningful inclusion in a cost effectiveness assessment. Moreover, if a therapy is prematurely deemed not cost-effective, the likelihood of third-party payers covering the treatment without imposing significant benefit utilization management policies increases, creating barriers to access for patients who need innovative and life-saving therapies. Without market uptake, real-world evidence and its inclusion in subsequent cost effectiveness evaluations will be limited. As such, in addition to reaffirming its commitment to real-world evidence, we recommend that ICER refrain from making a determination about the cost effectiveness of new therapies until mature real-world evidence emerges in order to ensure its inclusion in ICER's value assessments.

[1] https://www.futuremedicine.com/doi/full/10.2217/cer-2018-0066

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## II. ICER Should Incorporate Data Related to Indirect Costs to Patients into Its Value Assessment Framework

We thank ICER for seeking to work with the patient community as a partner through its Proposed Changes. The creation of a new "Patient Perspectives" chapter is a valuable addition to ICER's evidence reports. The first-hand experience of living with a condition provides important cost-effectiveness data. Patient-reported outcomes, for example, are increasingly gaining importance in clinical research as a means of measuring changes to quality of life.[2]

However, the Proposed Change still does not incorporate meaningful data regarding the direct and indirect costs of therapies to patients into its calculations of value-based benchmark prices and potential budget impact. Such data provides valuable information about patient-based considerations for innovative therapies, such as measuring adherence to complex treatment regimens and indirect costs to caregivers. The exclusion of such information would certainly impact an accurate assessment of the value of innovative treatments and should be included. Aimed Alliance requests that ICER revise its framework to include such data.

## III. ICER Should Consistently Include Patients and Medical Specialists in its Evidence Appraisal Council Membership

Patient advocates are included in ICER's public meetings and have an opportunity to comment to provide input on cost-effectiveness evidence. Yet, they are notably absent from ICER's voting evidence appraisal councils. Patients and caregivers provide a unique perspective about the value of new therapies about how living with a condition affects their quality of life. Though they are the only people who can provide this first-hand knowledge, their current role in ICER's Value Assessment Framework is minimal.

Moreover, while specialists in the therapeutic area that is under analysis are included in the Value Assessment Framework and are often available to ICER's voting councils at public meetings, they are not consistently included as members of ICER's voting evidence appraisal councils. Medical specialists are uniquely positioned to provide insight into the intricacies of treating specific medical conditions and may better understand the challenges that their patients face regarding treatment access and adherence.

As such, Aimed Alliance recommends that ICER alter its council membership to establish minimum requirements for the inclusion of representatives from the patient community and medical specialists in the therapeutic area under review on its voting evidence appraisal councils. This will better ensure that specialists, patients, caregivers, and patient advocates are consistently included on its voting council memberships to provide meaningful patient engagement in its cost-effectiveness assessments.

[2] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3227331/

## IV. ICER Should Provide Greater Transparency About the Evidence Evaluated Through the Value Assessment Framework

Aimed Alliance requests that ICER provide more transparency regarding the evidence being evaluated through its Value Assessment Framework, including information on study limitations, assumptions made, endpoints chosen, and model design used in its assessments. In particular, ICER has not provided any transparency on how it determines value-based benchmark prices and its potential budget impact analysis. ICER has not made its methodologies for clinical or economic evaluations transparent in such a way that outside researchers could test and validate its approaches. As such, we recommend that ICER make such information available.

## V. ICER Should Not Rely on QALY to Evaluate the Value of a Treatment

Aimed Alliance reiterates its longstanding recommendation against relying on quality adjusted life year (QALY) measures to evaluate any treatment. The use of QALY measures to evaluate the value of a treatment raises significant ethical concerns. QALY measures put a price tag on the value of human life that merely reflects the individual's diagnosis and deems those with chronic, debilitating, and rare conditions as being worth less than those with common conditions. They treat individuals' lives and health as a commodity and ignore patients' and practitioners' individualized concept of the value of treatment.

QALYs are often used to justify coverage limitations and utilization management policies, such as prior authorization and step therapy programs, that prevent individuals from obtaining treatments that are most appropriate for their individualized needs. Prior authorization requires providers or insured individuals to obtain approval from the insurer or its pharmacy benefit manager before the plan will cover the cost of a prescribed health care product or service. Step therapy requires insured individuals to try and fail on alternative treatments, sometimes with adverse effects, before the payer will cover the prescribed treatment. Such policies can be unethical and inconsistent with standards of care, interfere with the patient-doctor relationship, and result in significant delays to prescribed treatments. For these reasons, we recommend against using QALYs.

Thank you for providing us with the opportunity to comment on the Proposed Changes. Please contact us at policy@aimedalliance.org or (202) 559-0380 if you would like to discuss any of the recommendations herein.

Sincerely,

John Wylam
Staff Attorney