September 12, 2016

Submitted electronically to: publiccomments@icer-review.org

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President
Institute for Clinical and Economic Review
Two Liberty Square, Ninth Floor
Boston, MA 02109

Re: Feedback on ICER's Value Framework

Dear Dr. Pearson:

On behalf of the Institute for Patient Access, I thank you for the opportunity to provide feedback on the Institute for Clinical and Economic Review's value framework.

The Institute for Patient Access (IfPA) is a physician-led policy research organization dedicated to maintaining the primacy of the physician-patient relationship in the provision of quality healthcare. To further that mission, IfPA produces educational materials and programming designed to promote informed discussion about patient access to approved therapies and appropriate clinical care. IfPA was established in 2012 by the leadership of the Alliance for Patient Access, a national network of nearly 800 physician advocates committed to patient access. IfPA is a 501(c)(3) public charity non-profit organization.

Matters of patient access are increasingly influenced by third-party evaluations of what medications, diagnostics and devices are worth for patients and the overall health care system. Far from an end unto itself, a calculation such as ICER's value-based price benchmark goes on to inform crucial health plan features such as formulary design, cost-sharing ratios and the use of utilization management tools such as prior authorization and step therapy. These factors often determine whether a patient can access the treatment prescribed by his or her physician, and whether a physician can direct patient care as needed.

In light of the ICER value framework's impact on health care, patients and physicians across the United States, we offer the following 12 suggestions for the updated framework to be implemented in 2017. These suggestions focus primarily on two areas for which ICER requested feedback: 1) Integration of patient and clinician perspectives on the value of interventions, and 2) Incremental cost-effectiveness ratios and thresholds.

Please see the attached report for a full description of these recommendations and related background.

1. **Formally and transparently involve patients and clinician stakeholders throughout the valuation process.**

Patient and clinician stakeholder engagement is necessary throughout the ICER valuation process, and needs to be more formally and transparently described in the valuation framework. ICER should consider reviewing the research and findings supported by PCORI in the US and active patient engagement strategies currently implemented in Australia, Canada and the UK [i, ii] for examples of effective engagement methods that meaningfully involve patients in the valuation process. At the University of Leeds, UK, patients are directly involved in all elements of clinical research including the study design (including identification of outcomes and comparators groups), development of protocols, study implementation and dissemination of the findings of clinical studies [iii].

In addition to the areas previously identified by ICER, engaging patients and clinicians may also be useful during: (1) Construction of the overall model; (2) Review and evaluation of evidence; (3) Determination of the quality of life inputs; (4) Estimation of care value; (5) Assessment of other aspects related to the treatment; and (6) Rating care value. The experience in Leeds [iii] demonstrates that patient engagement during the research process can be effectively implemented and results in improvements in clinical studies.

2. **Incorporate patient reported outcomes in the comparative assessment of clinical effectiveness.**

In the ICER framework, it is uncertain how and, in some cases, even whether patient-reported outcomes (PRO) are incorporated into the value assessment. Patients should participate in identifying which outcomes are to be used to evaluate the effectiveness of a new treatment compared with existing treatment options [iii, iv, v, vi].

In addition, methods other than quality-adjusted life years are needed to evaluate treatments for particular diseases so that they incorporate effectiveness, adverse effects and survival. For example, the Q-TWiST (quality-adjusted time without symptoms or toxicity) approach [vii, viii, ix] may be effectively applied to evaluate the overall effectiveness of treatments for cancer, where apart from progression-free survival and overall survival, there may be treatment-related toxicity of varying severity that should also be evaluated.

More comprehensive approaches to evaluating treatment effectiveness should be identified and assessed. The clinician perspective is also important in understanding and incorporating clinical experience across multiple patient-related health states and trajectories for the target medical condition.

3. **Consider benefits and disadvantages other than the clinical effectiveness and adverse effects of new treatments.**

Patient and community groups should be asked about intervention-related benefits and disadvantages that may be less directly related to the clinical effectiveness of the intervention. Issues to be considered include route of treatment administration (i.e., oral, subcutaneous injection, infusion, etc.); aspects of the treatment that may reduce or improve adherence; other treatment-administration related characteristics; and other benefits and disadvantages to the broader community (e.g., herd immunity conveyed by vaccination, other interventions aimed at reducing transmission of infection).

These factors can be considered at the treatment and care level, and may be directed at the broader community level and improvement in public health.

4. **Explicitly incorporate contextual factors into the valuation process.**

The ICER process does not formally incorporate the contextual factors into the valuation process. Possible contextual factors are examined by the independent public appraisal committee, but little detail is provided on the methods used to incorporate them in the care value rating. In reports on specific topics, little information is included on the deliberations of these committees and how contextual factors were considered in the valuation process.

Contextual considerations may include legal, ethical, and other aspects that influence the priority of an illness or treatment. For example, for a particular disease, are alternative treatments available or is there an under-served need for interventions for the targeted condition? Issues related to the prevalence and severity of the illness may make the intervention a priority for the community. Are there ethical considerations related to the intervention?

Patients, their clinicians, and perhaps the general public should be involved in discussing these more intangible issues associated with new interventions, working toward consensus on the valuation of an effective, targeted intervention.

5. **Clearly describe the methods used for achieving consensus on care value.**

Based on the ICER webinar and slides describing its methods, the elements of care value are publically discussed and then treatments are rated as low, intermediate or high value. The methods employed in arriving at these recommendations are not well articulated. While it may not be possible to provide exact details, the approach for achieving consensus on care value, taking into consideration existing clinical evidence on effectiveness and adverse effects, other factors, and contextual issues need to be

described fully. These methods must ensure that patient and clinician stakeholders are well represented and are formally involved in this process of review, evaluation, and discussion, leading to the eventual consensus on level of care value.

6. **Use multi-criteria decision analysis more formally to assess care value.**

ICER evaluates the care value of products using four types of criteria: the strength of the evidence, the efficiency, the existence of other benefits and "contextual" factors such as ethical or legal aspects. Each product is rated in each category and this information is presented to an appraisal committee for discussion and voting on the overall rating (low, intermediate or high value). This process constitutes an informal multi-criteria decision analysis (MCDA) [x].

Following a more formal MCDA process would offer some benefits, chief among them the increased rigor involved. In addition, the emerging guidelines for good practices can be leveraged to further substantiate the recommendations that are made. A formal process will help members of the appraisal committee understand each other's position, assist in resolving discrepancies, and, in any case, it will increase transparency and facilitate reporting.

7. **Replace the general efficiency threshold with therapeutic-area specific ones.**

Using a single threshold is problematic. It imposes the idea that all products must abide by the same efficiency requirement, regardless of the severity of the illness, the unmet need or ICER's own rating of value. It has been repeatedly shown that most citizens do not agree with this [xi]. Moreover, a single threshold is impossible to support empirically—the UK researchers who spent an enormous amount of time and money trying to establish an empirical basis found, instead, an enormous range of actual efficiencies in the health care system [xii] and resorted to recommending an unsupported mean.

Instead of a general efficiency threshold, ICER should switch to therapeutic area-specific thresholds. This would accord much better with reality, where efficiency differs substantially across therapeutic areas.

8. **Derive specific thresholds by constructing efficiency frontiers in each area.**

Deriving an efficiency threshold is difficult in the absence of a legitimate market because it requires establishing what is reasonable to pay for a given benefit and there is no good way to do that. An approach that circumvents this conundrum is to rely on the actual market: what are we paying for benefits in a given area? This value provides the area-

specific threshold assuming that one should be reluctant to accept a product with lower efficiency.

These area-specific thresholds can be easily obtained by deriving the efficiency frontier in each area [xiii]. The frontier reflects the best extant efficiency at particular levels of benefit.

9. **Increase transparency by making models available to reviewers.**

Although ICER has formally expressed a commitment to transparency, the economic models that underlie much of the work are not open and available for review. This is unacceptable and contrary to the guidelines on good modeling practices [xiv]. In line with those guidelines, any intellectual property rights claimed by the developers of the models can be protected via appropriate non-disclosure agreements that must be signed before access is provided. Failure to do this will render all of the estimates suspect and raise questions about the commitment to transparency.

10. **Report expected budget impact but don't use it to derive acceptable price.**

There is no basis for constraining every product to the same amount. A major breakthrough should not be held to the same standard as a product that provides little advantage. There needs to be flexibility in the amount of budget allowed to be consumed by a new product. Moreover, an overall budget impact threshold is an unsound idea in the context of a health care system like the American one, which does not have anything like a global budget. Budget impact is a difficult aspect to assess in an overall manner without a specific payer in mind. Each budget holder must be able to address in its own context the strain a new product may apply.

The impact of a new product on a payer's budget is difficult to forecast because it depends on the frequency with which candidates for treatment appear in that jurisdiction, and the rate at which they are prescribed the new product; neither quantity being readily estimated, particularly for the entire health care system. Instead of deriving an "alert" price, the ICER evaluation should facilitate the budget holder's deliberations by providing a tool for estimating the impact to their budget under assumptions that make sense for them, including coverage modalities and other aspects that affect the budget impact.

11. **Involve patients and clinicians in deliberations regarding the budget impact ("health system value").**

Patients and clinicians should be directly involved in determining the health system value of new treatments and interventions. Methods are available for involving patients and

other stakeholders in the design and implementation of clinical studies [iv, vi, vii]. These methods are directly generalizable to engaging patients (and others) in determining value to the health care system. While this may be challenging, meaningful engagement of patients and clinicians in the process of valuing these new interventions ensures that assessments are transparent and that they consider comprehensively the range of interested stakeholders and perspectives. This involvement also ensures that all relevant and important outcomes are at least considered in the valuation process.

12. **Provide formal methodological guidelines or citations to existing ones for every aspect.**

The work that ICER does is exposed by its very nature to review and critique by many different stakeholders. In this context, it is extremely important that every step of the process be conducted following solid methodological guidelines. For many aspects, guidelines that have been developed and vetted by experts already exist and can be cited. Where there are gaps in the guidelines, or in places where ICER wishes to introduce its own approach, this needs to be carefully documented. As some of the work is performed on behalf of ICER by other institutions, they should also be held to strict standards. At the very minimum, they should be required to provide detailed technical documentation of what they do, and this information should be provided to all stakeholders upon request.

In conclusion, the Institute for Patient Access appreciates your consideration of the 12 recommendations listed above and detailed in the attached report. If we may provide further detail or aid the Institute for Clinical and Economic Review in incorporating any of the above recommendations, please contact us at 202-499-4114.

Sincerely,

Brian Kennedy
Executive Director

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