ICER Response to National Pharmaceutical Council Comments on ICER Value Assessment Framework

As an organization dedicated to transparency and inclusive discussion, ICER welcomes feedback from all stakeholders on its methods and processes. Recently, the National Pharmaceutical Council (NPC) published comments on the ICER value assessment framework. We felt it important to respond to these comments and to explain our methodology in more detail where necessary. (The original comments can be found on the NPC website here: http://www.npcnow.org/commentary/npc-comments-icer-value-assessment-framework)

I. Greater Transparency is Needed

Although the general components that are considered in the care value assessment are transparent, the actual economic models that are used to evaluate treatments—and the related data points—are not readily available for public review. We recommend making the models, data, assumptions and reasoning behind why they were chosen publicly available.

ICER is committed to a completely transparent process. ICER works closely with manufacturers from the inception of its review process to get their input on key economic model assumptions and model structure issues that manufacturers feel are most important. Later, comprehensive information on the structure, inputs and assumptions used in all economic models are provided in technical appendices to the public reports and are also posted to the Open Science Foundation website (https://osf.io/7awvd/). The entire ICER review process offers multiple opportunities for questions and comments about the economic modeling, and ICER prepares a document summarizing its response to these comments.

II. "Health System Value Assessment" Should Be Renamed "Health System Budget Impact"

The health system value (HSV) assessment is an assessment of budget impact, not an assessment of value. Using the term "value" is misleading and suggests that the assessment represents the benefit of a treatment relative to its cost. In actuality, the assessment represents the short-term budget impact for payers and its label should reflect this.

We respectfully disagree. We believe that an intervention's estimated budget impact on the health care system does represent a critical aspect of value, in that high budget impacts may require eliminating or delaying services of greater value and/or unsustainable increases in health insurance costs. Patients, purchasers, payers, and providers are seeking "high value" as

defined by superior outcomes at an affordable cost. Therefore, we believe we accurately reflect the view that "health system value" must incorporate considerations of long-term value for patients and the short-term affordability for the overall health system.

III. Methods for Assessing Health System Value Need Validation

The HSV assessment sets an annual threshold for the amount a drug could cost to keep overall health expenditures from increasing faster than the Gross Domestic Product (GDP) plus one percent. Methods to determine affordability price thresholds need broader input and validation from health care stakeholders.

The use of GDP + 1% as a threshold was selected as representative of societal willingness to pay embodied in state and federal legislation. The Accountable Care Act specifies that if Medicare costs rise more rapidly that GDP + 1% the Independent Payment Advisory Board should take action to keep cost growth below this threshold. Several states, most notably Massachusetts and Maryland, have legislation specifying that some or all segments of health care cost growth should remain consistent with the growth of state GDP. Our threshold for provisional health system value, therefore, is based on public input as expressed through legislation, and is not arbitrary. However, neither is it an absolute budget cap. Our provisional health system value threshold represents an "alarm bell" for identifying when policymakers need to focus special attention to determine whether extra measures are required to manage the affordability of a new intervention. We agree that further public discussion of affordability thresholds will be important, and we hope that our methodology will spur and inform this national debate.

IV. Utilize More Realistic Adoption Rates

Under the HSV calculation, the framework relies on drug adoption rates that might not accurately reflect the uptake of a drug. Most payers tend to wait six to 12 months to see how a drug is being used and how well patients respond to it before making those types of calculations, which provides them with a more accurate adoption rate.

The adoption rates used in our value framework analyses are meant to represent the uptake that could occur in an unmanaged environment, that is, without attempts by payers or provider groups to adopt specific policies to limit adoption, such as through prior authorization or step therapy requirements. The current ICER methodology represents an evolution informed by recommendations from NPC and other manufacturers that ICER adopt a 5-year budget impact time horizon and use assumptions that less than 100% of eligible patients begin use of the new intervention within that time frame. Our uptake assumptions therefore now run from a low of 10% to a high of 75%, levels that we believe do represent likely uptake patterns in the absence of specific payer and provider policies.

We acknowledge that little data on uptake rates are available at the time our reports are created, but believe that it is imperative to the process that stakeholders have an initial value assessment of new drugs as close to introduction as possible. A delay of six to 12 months would mean that stakeholders would not have this critical information in hand as the new drug is approved, initial coverage policies are created, and price negotiations are occurring. Finally, we note that payers are reporting increasing industry pressure to make formulary decisions as close as possible to drug approval, so a 6-12 month delay in consideration by pharmacy and therapeutics committees may be a thing of the past.

V. Framework Disincentivizes Development of Treatments for Broad, Unmet Need Conditions

The method used to determine health system value creates a disincentive for the development of medicines to treat large, unmet burdens due to the ensuing budget impact. Because it is based upon the number of individuals requiring treatment, drugs that treat broad, unmet need conditions such as hepatitis C, inadequately controlled high cholesterol, or (potentially, in the near future) Alzheimer's, would be viewed as having low health system value merely because of the large treatable population, even though they may be very cost effective for individual patients.

As stated earlier, our goal is to have stakeholders engage in meaningful discussions that take into account not only the cost-effectiveness of new interventions, but also the potential impact on health care budgets. Our results, particularly related to provisional health system value, are intended to guide policy discussions, not as an algorithm to a specific coverage or pricing decision. In certain circumstances, it may be that there is uniform support to go well above the "alarm bell" threshold for short-term budget impact in order to pay for a therapy of high care value (e.g., recent new treatments for hepatitis C).

We do not believe that acknowledging the reality that short-term budget is an important consideration and component of health system value creates a disincentive for life science companies to develop treatments for large patient populations. What we intend with our approach is to signal when the potential added costs to the health care system at list price and with unmanaged uptake assumptions requires serious discussion of how to manage affordability in order to bring interventions of high care value into the health care system in a responsible, sustainable fashion. We acknowledge that our approach may provide extra incentives to price new interventions with an eye toward not only the long-term care value but also toward the ability of the health care system to manage short-term added costs. We believe these incentives are appropriate and have for too long been absent from the discussion of pricing and early coverage of new drugs and devices.

We also note that some groups have criticized our framework approach for what they believe is too generous a threshold for 5-year affordability. We will continue to welcome feedback on the important assumptions underpinning our threshold, including our assumption that

background inflation of prices for existing drugs is net neutral to budget impact, with all cost growth allocated solely to new drugs.

VI. List Price Does Not Accurately Represent Actual Price

The HSV assessment also uses the list price of a drug, which does not consider the actual discounts and negotiations made with public and private payers. Prices – for both the comparator and active drug – should be representative of actual prices. While it may be difficult to precisely quantify the net price to payers, effort should be applied to calculate a weighted estimate of net price.

Around the time of FDA approval, list price is the only universally-available metric. Complete discount information is not typically publicly available, even long after the launch of new products. Our cost-effectiveness analyses routinely explore the sensitivity of variation in drug prices on model results. In addition, our conclusions regarding value-based price benchmarks may discuss whether these results fall within the range of discounts that payers are typically able to achieve. We believe that, absent a complete, verifiable, and publicly-available source for discounts, this is an appropriate approach to inform discussions around the value of new interventions.

VII. Allow for Approval Fluctuations

HSV calculations assume that similar numbers of new drugs will be approved each year by the Food and Drug Administration and will have similar market experience. From historical experience, these numbers can fluctuate greatly from year to year.

The ICER value framework uses a running average of the number of new drug (or device) approvals over the prior two years. Our intent is to revisit these figures on an annual basis and make adjustments based on the most recent two years' experience. This will allow us to account for fluctuations from year to year as well as trends over time in the number of new FDA approvals.

VIII. A Longer Time Horizon is Needed

Typically, payers only consider a 1-2 year timeframe for calculating budget impact, although the effects of some treatments are realized over a longer time horizon. The ICER health system value assessment uses five years for calculating the budget impact, although this still might not capture the true savings realized from curative therapies. Hepatitis C cures, for example, produce economic savings well beyond five years. Ideally, a lifetime horizon would be utilized.

Our reports include assessments of both long-term care value and short-term potential budget impact. Our care value assessment uses a long-term time horizon, typically lifetime, and therefore fully captures downstream benefits, including potential cost savings, of new interventions. However, as described earlier, health systems need to integrate long-term perspectives with those related to short-term budget impact. Even though payers around the world tend to manage budgets on 1-2 year cycles, we incorporated earlier feedback from NPC and other sources and opted to stretch our time horizon out to 5 years. This will capture some, but not all, of the distant effects of a new intervention, but it goes further than a 1-2 year horizon and therefore seems a reasonable compromise. Input from other stakeholders has stressed the importance of understanding 1-2 year figures on potential budget impact as well, so we are likely to include that information in future reports.

IX. Give Quantitative Credit for Contextual Considerations

The threshold prices do not reflect key economic benefits, such as improvements in worker productivity that are valued by employers, or the savings or reductions in caregiver burdens that are important to patients and their families. These considerations should be incorporated quantitatively, rather than merely qualitatively.

As our primary approach, we have chosen not to incorporate productivity or caregiver burdens into the economic modeling, preferring to provide these analyses, where feasible, as a sensitivity analysis. Our reason for doing so is the well-known problem that incorporation of productivity (or of tax receipts, etc.) can bias results in favor of interventions for younger, working patients, particularly those in high-income occupations. We do not want to adopt an approach that puts treatments for the elderly or disabled at a disadvantage. We will therefore definitely capture this information (if present in the available evidence) and provide it so that it can be considered by our voting panels in their determination of care value, which is explicitly a judgment that includes considerations beyond pure cost-effectiveness from the payer perspective.

X. Emphasize the Breadth of Policy Solutions

Despite an acknowledgment by most health care stakeholders that we need to have a broader, constructive discussion about value, too often these conversations devolve into criticisms of price. The breadth of policy solutions beyond price must be continually presented.

We agree that, particularly when an intervention determined to have "high" care value is also found to have "low" provisional health system value, that pricing is just one of several policy levers that need to be utilized in order to maximize the actual health system value of the intervention. We will continue to emphasize this point in our reports and in the discussion of our Policy Roundtables held during the public meetings at which our reports are debated. We welcome the efforts of other stakeholders in broadening

the discussion on the mechanisms for maximizing the health system value of new interventions. ICER is not an advocacy organization and does not support a specific policy solution; rather, we intend our reports to contribute information to those discussions and believe that they provide valuable input for alternative approaches to paying for innovative technologies and ensuring they are made available to patients who need them.

XI. Bring Broader Stakeholder Representation into the Assessment Process

The current assessment process is predominantly driven by payers. The advisory board and voting panels should include broader stakeholder representation to reflect all relevant perspectives. Additionally, panel votes should be made publicly available to increase transparency.

This criticism is patently false and misleading. In fact, no payers are voting members of the CEPAC and CTAF panels. Panel members have passed strict conflict of interest provisions that include requirements that they have minimal honoraria or consultancy income from manufacturers or payers. Like FDA and CMS, we do not require each individual's vote to be identifiable so that they can feel free to vote their conscience without concern about repercussion. After each vote, however, we do invite comments from panelists on their vote in order to seek the greatest transparency possible.

The Advisory Boards for CEPAC and CTAF are comprised of a broad array of stakeholders, including representatives of provider groups, clinician specialty societies, patient-based organizations, public health agencies, academic institutions, and public as well as private payers. These groups help prioritize topics and work to broaden the impact of the reports when they are completed. They have no role whatsoever in the conduct of our reviews. It would not be appropriate to have anyone from a life science company on these Advisory Boards because of their conflict of interest in the selection of topics for review. From our inception, however, ICER has welcomed representatives from the life science industry on our own organizational Advisory Board where we have benefitted from their perspectives on our broader organizational mission and collaborations. The calumny that our process is predominantly payer driven is a canard easily used but without merit.