



# **2023 Value Assessment Framework**

**Response to Public Comments**

**September 25, 2023**

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# 1. Introduction

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ICER thanks the 32 organizations who gave feedback on the draft proposals released in June 2023. We deeply appreciated the time, thought, and effort that went into each of these submissions. Here we provide a general overview of the comments received and our response to those general themes. Readers of this document are encouraged to view it as a complement to the justifications provided in the [draft revisions](#) proposed in June 2023, as well as the rationale provided in the final [2023 Value Assessment Framework](#) itself.

## 2. Comparative Clinical Effectiveness

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### Clinical Trial Diversity

***Commenters shared general support for ICER's clinical trial diversity proposal while recommending that we ensure efforts align with FDA Guidance for Industry. Commenters also suggested broadening the demographic characteristics evaluated, proposed different approaches to multinational trials, and recommended that we re-consider the approach for evaluating trials conducted for rare conditions.***

We have been closely following FDA's effort to improve clinical trial diversity. Our clinical trial diversity rating (CDR) framework was largely guided by FDA guidance. For example, the FDA recommends that sponsors should set enrollment goals based on the epidemiology of the disease, particularly when data indicate that the medical product may perform differentially across different populations based on factors associated with demographic characteristics such as race or ethnicity.<sup>1</sup> As such, our framework uses the epidemiology of the disease as the benchmark for evaluating clinical trial diversity. ICER's framework expands on this guideline by defining thresholds to judge adequate or inadequate representation. For example, we defined a participation-to-prevalence threshold of 0.8 to judge adequate representation to account for the limitations of epidemiology estimates. Furthermore, the demographic characteristics and categories included in our framework are based on the reporting requirement by the FDA. We received several comments about the limitations of the current Office of Management and Budget (OMB) race and ethnicity categories, however, our framework uses these categories to evaluate racial and ethnic diversity to match the current FDA standard for collecting race and ethnicity in clinical trials.<sup>2</sup> We will continue to follow the efforts of the FDA in this area closely and will ensure that our CDR framework remains updated in alignment with FDA guidelines.

We agree with many commenters who highlighted the importance of other demographic characteristics. We are committed to promoting conversations around equity in clinical trials of new drugs. As such, the clinical trial diversity section in our report will descriptively cover information on the relevant demographic characteristics. However, ICER's quantitative evaluation of clinical trial diversity can only focus on the demographic characteristics, such as race and ethnicity, sex, and age, that are consistently captured and reported in clinical trials.

We received conflicting comments on how to approach multinational trials. While some commenters strongly supported limiting the evaluation of racial and ethnic diversity to patients enrolled in the United States (US), others were concerned this would penalize global trials that attained representation but recruited too few US patients to attain adequate representation. Since our approach seeks to promote conversations around equity in clinical trials of new drugs in the US, we will continue to focus on rating the subpopulation of patients recruited in the US. However,

information on the racial and ethnic diversity of the overall patient population will be provided for context as needed. We know that, in most cases, the baseline characteristics of the US subpopulation will not be published; therefore, this information will be requested as part of the routine data request sent to manufacturers. If these data are not published and not provided to us, we will rate the entire trial population.

Finally, we agree with many commenters who cautioned against using the US Census estimate as a benchmark to evaluate trials of rare diseases when there are no reliable prevalence estimates. We have now revised our approach. Trials of rare diseases with no reliable disease-specific prevalence estimate will not be rated on clinical trial diversity. Instead, a qualitative description of the demographic characteristics of participants in the clinical trial will be presented in this section. For other conditions (not considered rare) with no reliable disease-specific prevalence estimates, when appropriate, consideration would be given to comparing clinical trial participants to population estimates (US census demographic breakdown) and interpreting the finding accordingly.

## Subpopulation Evaluations

***Commenters said we should not limit subpopulation evaluations to only those defined by race/ethnicity, age, or sex. Furthermore, several commenters wanted us to clarify how subpopulation findings will be translated into the cost-effectiveness evaluation.***

We do not plan to limit subpopulation evaluation in our reviews to only those defined by race/ethnicity, age, or sex. This has now been clarified in the VAF document. ICER's approach is to consider subpopulations defined by race/ethnicity, age, sex, and other characteristics that are relevant to the topic as presumptive subpopulations of interest at the start of a review. The final list of subpopulations that will be evaluated will ultimately be determined by information gathered during scoping. During scoping, we conduct targeted literature reviews to evaluate the existing evidence base and consult with clinical experts, manufacturers, patients, patient groups, and other stakeholders. Investigations of a presumptive subpopulation during topic scoping may result in a conclusion that further consideration of that subpopulation is not warranted or that additional information is needed to proceed. Information gathered during scoping may also lead us to include another subpopulation of interest. The rationale for including a subpopulation will be described in the scoping document and/or research protocol.

On the topic of cost-effectiveness, [ICER's Reference Case](#) calls for the inclusion of different subpopulations when clinical effectiveness data suggest plausible and important differential effects, especially when these subpopulations are identified *a priori* at the time of trial design. *Post hoc* analyses of subpopulations defined solely by race/ethnicity or socioeconomic status are vulnerable to unknown confounding clinical and socioeconomic variables, raising the risk of misleading results. Therefore, in each report, we will provide the rationale for why we have performed or avoided cost-

effectiveness analyses of subpopulations defined by characteristics other than known clinical markers of risk or outcome.

## 3. Incremental Cost Effectiveness

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### Perspective

***Commenters shared general support for ICER’s “non-zero” proposal within its modified societal perspective. Some commenters also advocated that we adopt a co-base case approach for all assessments with results from the health care system perspective and a modified societal perspective. Some commenters, however, emphasized the potential for discrimination with societal perspective analyses that grant additional value to treatments for conditions affecting younger, non-disabled patients.***

ICER’s value framework continues to rely on the health care system perspective for the conventional base case, sharing with nearly all health technology assessment (HTA) organizations the view that the most relevant perspective for decision-making by the majority of insurers, provider groups, and policymakers in the United States. We also believe that default inclusion of a modified societal perspective raises important risks for discrimination against treatments for conditions affecting older populations or those with chronic underlying disabilities. Nonetheless, we agree that estimates of a broader societal perspective remain important for all policymakers to understand, particularly when the results of a societal perspective analysis differ markedly from that of the health system perspective. As proposed, we will adopt a new method to enhance the relevance of our societal perspective analyses by including indirect estimates of patient and caregiver productivity time when innovator companies fail to include these outcome measures in their clinical trials.

ICER’s value framework remains consistent with the prior version in terms of when to include the societal perspective analysis as a co-base case along with the health care system perspective (i.e. when the societal costs of care are large relative to the direct health care costs and the impact of treatment on these costs is substantial). Due to added uncertainty of including indirectly calculated “non-zero” inputs for patient productivity and caregiver time, we will not include the modified societal perspective to be a co-base case in ICER’s health benefit price benchmarks.

## Dynamic Pricing Scenario

***Commenters were generally favorable to ICER's proposal to perform dynamic pricing scenarios but some warned of the lack of consensus on how to operationalize this concept.***

As we stated in our original proposal, many academic and HTA health economists have questioned whether value assessments intended to inform current decision-making should factor in potential price increases or decreases for drugs many years in the future. Arguments for not adopting a “dynamic pricing” paradigm are not solely based on the substantial uncertainties of the magnitude and timing of these price changes, but also focus on whether society should accept a price that produces negative overall health outcomes in the short-term through opportunity cost effects for the promise of a shift toward increased social value many years in the future.

Despite these concerns, we agree with the majority of commenters that it is reasonable to move forward with developing methods to perform dynamic pricing scenarios. In large part our decision is driven by the advent of the Inflation Reduction Act (IRA), which includes both a mechanism to block price increases greater than the rate of inflation and a process for price negotiation for high-cost drugs that have no active generic/biosimilar competition. However, many questions remain, and some commenters warned that there are no consensus methods on how to operationalize dynamic pricing scenarios. Among the many uncertainties remaining are:

- Whether the IRA itself will be implemented following legal challenge.
- Which drugs will be subject to drug price negotiation, at what time point in their market lifespan, and with what percent decrease, if any, from their net price at launch.
- Which drugs will have substantial sequential price increases in the private insurance market despite the impact of the IRA on price increases for drugs paid for by Medicare.
- Which drugs will avoid generic competition and/or price negotiation by “product hopping”.
- Which newly launched drugs will become obsolete through the introduction of better options by the time drug price negotiation or genericization occur.
- What the price dynamics will be for the drug(s) used as the comparator to new drugs.
- Whether dynamic pricing should be modeled using a standard cohort or a stacked cohort approach.

After consideration of feedback on our proposal, we will not launch an official dynamic pricing scenario at this time, but instead will pursue a focused collaborative effort to develop and test methods for this approach. As now stated on page 35 of the finalized VAF:

“Prior to changing our approach to include price dynamics within a mandated scenario analysis, we commit to engaging our Health Economics Council, Methods Advisory Group, and other researchers and stakeholders including international Health Technology Assessment bodies to test the feasibility and impact of how best to include pricing dynamics within cost-effectiveness analyses.

Although academic contributions are emerging in the dynamic pricing arena including those published as a themed section on prescription drug pricing at Value in Health,<sup>3</sup> best practices across health technology assessment entities do not exist. Further, public comments received on this topic supported additional deliberation on the methods prior to implementing them in ICER's Value Assessment Framework. We are willing to make updates to our Value Assessment Framework on this topic if and when engagement and testing support making a change."

## Outcomes (Quantifying Additional Dimensions of Value)

***Several commenters said that we should expand the measures of value and include modifiers related to severity and other elements. Commenters pointed to emerging methods such as the Generalized Risk-Adjusted Cost-Effectiveness Analysis as an approach to weight severity and risk aversion.***

We recognize the efforts of the International Society for Pharmacoeconomics and Outcomes Research Task Force that explored the potential for quantifying a broader set of elements related to value. As the Task Force [wrote](#),<sup>4</sup> "Other value elements including value of insurance, severity of disease, value of hope, and real option value have been shown to modify [quality-adjusted life year] QALY estimates, but are not commonly used in [cost-effectiveness analysis] CEA. Further research to evaluate their potential for more standard use is warranted." After ongoing consideration of the potential to perform quantitative analyses of these types of additional elements of value, we believe at the current time that there are still many methodological concerns that make it more appropriate for us to consider these elements through deliberation focused on a broad cost-effectiveness threshold range. There are concerns related to potential double counting between these potential additional domains and the health gains captured by the equal value of life years (evLY)/QALY. There is a lack of academic consensus on how to conceptualize or measure the value of hope, real option value, or scientific spillover effects. There are also concerns about whether these potential value domains, even if measurable, should be factored into the calculation of a "fair price" for drugs and other health care interventions, or whether these potential benefits should accrue to society as part of the broader social contract between society and health care innovators. Lastly, but very importantly, current opportunity cost thresholds are built on measurements of tradeoffs in health gain alone. It may be that a new health care intervention conveys additional benefits outside of health gain, but if these additional benefits are not also known for those services that would be "lost" due to opportunity cost effects, it seems most appropriate to assume that any additional benefits gained would be matched by those that are lost through opportunity cost effects.<sup>5</sup>

Although the incremental cost-effectiveness findings will not be modified by severity in ICER's Value Assessment Framework, as described in detail later in this document, we will begin measuring "unmet need" by calculating absolute and proportional evLY shortfalls, and we will incorporate



consideration of these quantitative measures as part of the deliberation and voting during public meetings.

More research is needed to understand the implications of modifying health gains quantitatively, including understanding the potential budget impact of paying more for health unit gains in more severe disease and considering whether patients, payers, and society agree that treatments for conditions such as psoriasis, asthma, and diabetes – which may be ranked as less “severe” – would receive lower value-based pricing for the same health gains. Despite these uncertainties, we received numerous comments urging us to consider the potential advantages of the Generalized Risk-Adjusted Cost-Effectiveness (GRACE) set of methods. We have been aware of GRACE and are interested in exploring its potential advantages and unintended consequences further. GRACE is still very new, and neither the academic nor HTA community have experience with it. Therefore, after consideration of feedback received and our further consideration, while we will not implement GRACE as a standard part of our methods at this time, we will have a focused program to explore its use for potential future adoption. As stated on page 28 of the finalized VAF:

“...with this update to the Value Assessment Framework, we signal that we will begin a special focus in coming months on considering novel ways to quantify preferences related to severity, methods that often are framed as abandoning an assumption of a linear relationship between health gain value and replacing it with a formula that can capture risk aversion, severity, and the value of insurance. We will focus on exploring the Generalized Risk-Adjusted Cost-Effectiveness framework<sup>6,7</sup> and methods adopted by several international HTA programs that now weight health gains in relation to severity. In this effort to examine these methods, we will engage our Health Economics Council, Methods Advisory Group, and other researchers and stakeholders including international Health Technology Assessment bodies prior to testing the feasibility and impact of shifting to differentially weighting cost-effectiveness findings. We will also continue to monitor advances in methods as well as monitor changes made in the health technology assessment ecosystem on this topic. And, as a result of this special focus, we may entertain making an interim update to our Value Assessment Framework on this topic prior to the next overall update.”

## Health Benefit Price Benchmarks

***Commenters generally opposed any consideration of lower cost-effectiveness thresholds as the foundation for health benefit price benchmarks. Most commenters also opposed the ongoing use of shared savings scenarios within analyses of high-impact single or short-term therapies such as gene therapies. Commenters also encouraged the consideration of both opportunity-cost and willingness-to-pay approaches to determine operational thresholds for price benchmarks.***

Our original proposal did not include the idea of moving to a lower cost-effectiveness threshold range to frame health benefit price benchmarks. Instead, it was noted that ICER’s current threshold range was framed around the best existing research on the opportunity-cost threshold (\$104,000

per QALY), with the relatively expansive additional range up to \$150,000 per equal value of life years gained (evLYG) representing the way that additional value elements and modifiers could be considered, especially given that policymakers and the media tend to focus on ICER's benchmark price point at \$150,000 per evLYG instead of the lower end of the range. We noted in our proposal that advocates for quantifying additional elements of value within a modified QALY or evLYG should be aware that doing so would raise the real prospect that ICER's cost-effectiveness range should be lowered to reflect the impact on opportunity costs. In addition, if priority is to be given in some way to equity concerns about the opportunity costs faced by individuals with lower incomes, cost-effectiveness thresholds used to suggest fair pricing would be lowered, i.e. services should be made available at a lower cost and overall health care insurance premium growth should be constrained further. However, there is also value in retaining a consistent threshold range as a level playing field for all stakeholders, especially as an incentive for future innovation. Therefore, for now, we will be making no change to our threshold range. In practice, we generally characterize our HBPBs as the range between \$100,000 per QALY as the lower bound and \$150,000 per evLYG as the upper bound, but we continue to emphasize that HBPBs based solely on the \$100,000-\$150,000 per evLYG range are presented in our reports and available for use by decision-makers wishing to avoid any calculations involving the QALY.

We received some comments encouraging a shift from an opportunity-cost paradigm to that of a "willingness to pay" paradigm for determining operational cost-effectiveness thresholds. However, we believe that there is a confluence of results between research exploring opportunity cost thresholds and willingness to pay thresholds in the US setting, and for conceptual and ethical reasons, we continue to favor an opportunity cost paradigm with a single threshold range for all new health care interventions. While there is a case for multiple thresholds based on willingness-to-pay which may differ by payer type, there is also a widely accepted ethical goal in the US to have a common standard of care available for all patients, albeit with acknowledged differences in access due to network constraints, out-of-pocket payment, and other benefit design features. That the US does not yet achieve the goal of a common standard of care available for all patients does not imply, in our view, that we should abstain from framing a cost-effectiveness range that should apply broadly across many, if not all, health insurance systems in the US.

On the topic of shared-savings analyses, some commenters felt that it is unwise to continue using our current methods to set a lower health benefit price benchmark for treatments with large cost offsets that are based on prices for comparators that greatly exceed conventional cost-effectiveness ranges. Some comments supported an alternative method in which preliminary analysis to "re-price" the comparator at a price aligning with a cost-effectiveness threshold, but this approach faces both practical and conceptual barriers when there are multiple generations of treatment modalities. Our current method of capping cost offsets at \$150,000 per year is supported by the argument that any comparator versus its alternative should not yield more than one QALY or evLYG per year in terms of comparator gains. After extensive public feedback on ICER's valuing a cure

framework as it was developed several years ago, and further consideration of public comment on this year's VAF update, we have decided to continue applying our shared savings methodology when a large percentage of the traditional health benefit price benchmark comes from cost offsets and when the comparator therapy price is deemed as failing to meet common cost-effectiveness thresholds.

## 4. Benefits Beyond Health and Special Ethical Priorities

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The fields of health economics, ethics, and political science have all seen work over more than three decades on considerations of how health technology assessment should account for elements of value that in some way may modify the “weighting” of health gains or that represent novel dimensions that have not been adequately integrated into broader judgments of value. This work continues in earnest today, and has been complemented over many years as well by the practical experience of HTA organizations at all health system levels and in countries with widely varying social and political cultures. Because of the importance of this context, before addressing public comment on our proposed changes, we present below some of the background from our original proposal of changes to ICER’s methods in integrating “potential other benefits” and “contextual considerations” into our assessments.

The inclusion of explicit domains of value labeled “benefits beyond health” and “special ethical priorities” are critical features of the ICER Value Assessment Framework. These elements of the framework force the ICER appraisal committees and all external stakeholders to consider broader domains of value than those that are core to clinical trial evidence and cost-effectiveness modeling. All too often what matters most to patients is poorly captured in the available clinical trial data. Sometimes this occurs because surrogate outcome measures do not reflect true patient-centered outcomes; but even when trials do capture the clinical outcomes that matter most to patients, there are other aspects of value related to the complexity of the treatment regimen or the impact of care options on the ability to return to work, on the negative impact of the condition on family and caregivers, on public health, or on other aspects of the health system or society. The ICER value framework identifies these “benefits beyond health” as important elements of any overall judgment on long-term value for money, and all ICER reports have separate sections in which evidence and information pertaining to these elements are presented.

Similarly, decisions about value do not happen in a vacuum. Stakeholders may have special ethical priorities related to the severity, or “unmet need” related to the condition. Similarly, societies have an ethical priority to give some degree of preference to interventions that can provide health gains that reduce historical disparities in outcomes often due to discrimination of one kind or another. The ICER value framework includes this domain of value and it is explored in a separate section of each ICER report.

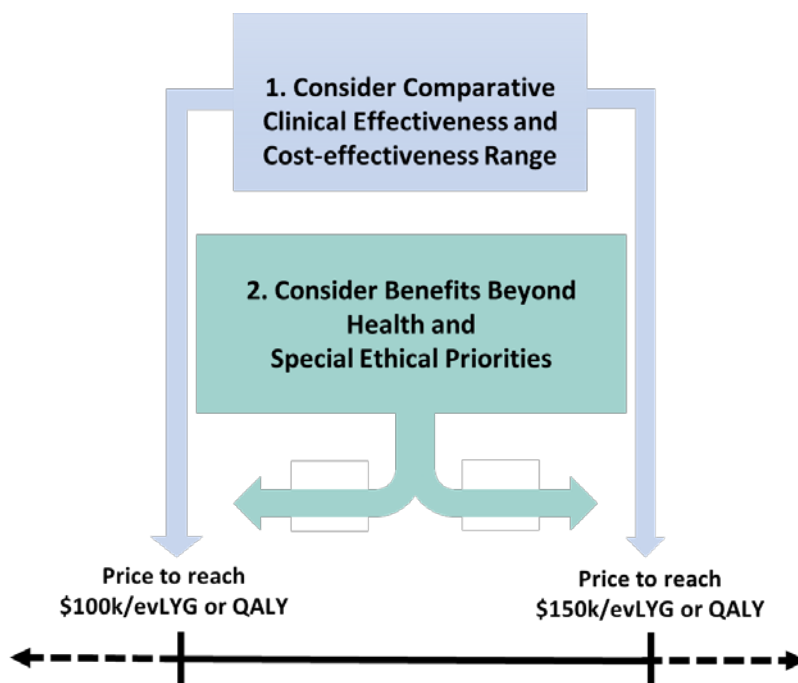
Researchers and policymakers continue to explore different ways to identify specific benefits beyond health and special ethical priorities and apply them in a formal quantitative fashion to weight health gains or to adjust cost-effectiveness thresholds. However, current methods for algorithmic integration of these factors carry important risks. Attempts to measure benefits that

accrue to patients in their ability to fulfill their life goals, or to achieve greater personal dignity, are likely to represent some degree of double counting of the benefits captured by conventional measures of health gain, such as the evLYG or QALY. Some potential benefits beyond health, such as the scientific “spillover” of new science to other treatment areas, cannot easily be distinguished from the spillover of investments in other areas of health or societal wellbeing. And routine quantitative inclusion of productivity gains raise the specter of discrimination against people with chronic disabilities who may never achieve a health status that allows them to contribute as much traditional economic output as others. These examples demonstrate that the general impulse to recognize and account for benefits beyond health and special ethical priorities needs to be tempered by methodological and ethical concerns.

Thus, it is not surprising that most health technology assessment groups around the world do not attempt to quantify these domains of value, believing that consideration of these domains is essential in a judgment of value, and yet should be left qualitative and integrated into decision-making through public deliberation.

We have considered over many years the evolving methodological options for quantitative or mixed-methods approaches to enhance the explicit integration of these considerations in value assessment. Formal multi-criteria decision analysis (MCDA) has been considered but rejected because we do not believe that the methods for weighting individual elements are robust enough to add to reliability of value judgments. We have attempted formal MCDA with ICER’s independent committees on several occasions in the past and found the technique too complicated for reliable use. Based on discussions with stakeholders, benchmarking other value frameworks around the world, and the input of public comment, ICER reports will continue to use a variation on MCDA that makes benefits beyond health and special ethical priorities explicit and gives clear guidance on their relevance to judgments of value, but that does not attempt an overly facile quantification. Decision-makers will be given guidance, however, that consideration of these factors should guide part of their thinking about how to use the cost-effectiveness threshold range, with higher ends of the range more applicable when there are important positive contributions related to these factors, and lower ends of the range reflecting relatively less consequential added value considerations. Figure 4.1 on the following page summarizes this conceptual approach to integration of benefits beyond health and special ethical priorities into considerations of long-term value for money of a health intervention.

**Figure 4.1. Conceptual Guide to Application of “Benefits Beyond Health” and “Special Ethical Priorities” to Judgements of Value**



evLYG: equal value life-years gained, QALY: quality-adjusted life years

## Dimensions of Value

*Several commenters expressed concerns that in the new set of report and voting categories, we seemed to be eliminating some of the value elements it included in the past such as mechanism of action, complexity, lifetime impact, etc. We also received comment suggesting that benefits beyond health and special ethical priorities be presented in a consistent table or graphic format that would emphasize the importance of these factors in value judgments.*

We believe that commenters may have misunderstood the new proposed list of what we will now call “benefits beyond health” and “special ethical priorities.” Lifetime impact was part of our conceptualization of severity, with short-term risk of death or progression of serious disability reflecting the other component. Our experience with this splitting of concept of severity was that it seemed to place a burden on patient advocates to describe their lived experience in ways unfamiliar to them, created difficulties for the appraisal committees in distinguishing the issues, and may have reduced the ultimate impact of deliberation on this important potential modifier of health gains. With prior field testing with our appraisal committees, we believe that a single question on “unmet need,” supported with quantitative findings on health shortfall, will prove

more consistent with patient community goals in sharing their view of the severity of the condition in the context of current treatment options.

Similarly, we have not eliminated consideration of the potential benefit of a new mechanism of action. We have placed this issue within the framing of whether a mechanism of action can offer the opportunity to improve access and outcomes. We did eliminate complexity of regimen as a separate vote because questions about what the appropriate comparator should be for this question made it difficult for appraisal committees or others to find the discussion and vote helpful. However, importantly, we will retain the flexibility of adding additional benefits beyond health to the votes on a case by case basis, and we will seek input from manufacturers and patient groups on this issue well in advance of the public meeting.

We agreed with one commenter's suggestion and will summarize benefits beyond health and special ethical priorities in a table in the report and also present this information in a table or graphic side-by-side with the results of comparative effectiveness ratings, long-term cost-effectiveness analyses, and short-term affordability estimates within our Report-at-a-Glance.

***Several commenters asked ICER to shift consideration of these factors from a qualitative/deliberative method to a more quantified method that would directly modify the measure of health gain and lead to a different cost-effectiveness result.***

In the section above on incremental cost effectiveness we describe the theme in many public comments of the importance of quantifying benefits beyond health and special ethical priorities so that their impact is seen in the calculation of the health benefit price benchmark range instead of as qualitative factors that should be integrated into judgments of value within that range. We note here again that health economists, ethicists, political scientists, and HTA organizations have been examining this area for many years, and that there are important conceptual as well as practical barriers to a simple quantification approach without risking double counting, unintended consequences with ethical ramifications, and the need to revisit the opportunity cost threshold. We will continue to evolve our methods, including an ongoing closer look at the GRACE methodology, but we strongly believe that having dedicated sections of each report on these issues, along with formal deliberation and separate votes on each, serves as a useful means of highlighting the relevance of these factors and encourages their incorporation in value judgments made by decision-makers.

***Several commenters suggested technical improvements to the way we will present and perform our Likert scale votes.***

We will change our approach so that we will define each number on the Likert scale, present the mean as well as the full range of voting for each question.

***We received several comments urging reconsideration of multi-criteria decision-analysis (MCDA) as a way to integrate conventional cost-effectiveness findings along with other factors into decisions.***

As noted in our proposal, we have attempted MCDA previously and found it unsuitable for use by our appraisal committees. Research is ongoing on ways to make MCDA more amenable to public deliberation, but requires quantification and priority-setting among multiple factors that can be extremely complicated and that, to an important extent, remains the task of the ultimate decision-maker. ICER reports and the results of public meetings and votes are meant to support decision-making by a wide variety of payers and other decision-makers, raising the question of whether it is appropriate for ICER to even attempt MCDA. Nonetheless, we remain actively interested in considering academic and practical experience with MCDA and other methods for incorporating additional decision factors in a way that can be transparent and useful to diverse decision-makers.



## 5. Topic Selection

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***Commenters shared support for incorporating health equity considerations into topic selection; however, some also shared concern that it is too early to incorporate health equity considerations. Comments also encouraged ICER to work with manufacturers and patients in the topic selection process.***

We agree that the incorporation of health equity considerations is a new and evolving aspect of value assessment methodology, but we do not feel that concerns related to the maturity of these methods should prevent us from considering relevant health disparities within the topic selection process when possible.

We welcome any topic suggestions from outside individuals or groups through the use of our [topic suggestion form](#), found on ICER.org. In the course of evaluating our topic options, we may seek input on potential role in therapy, ideal set of comparators, and other factors relevant to determining whether the most appropriate timing and focus of an HTA. For this input we may reach out to clinical experts, payer organizations, clinical societies, patient organizations, and manufacturers themselves; however, to maintain the independence of our evaluations, the final selection of which topics to pursue is ICER's alone. In addition, our Director of Patient Engagement coordinates discussions with a variety of patient groups throughout the year during which ideas for new topics are always welcomed; these suggestions are then relayed back to ICER's topic selection team. Formally incorporating patients/patient groups into the topic selection process may be complicated by the variety of disease/therapeutic areas where drugs are being developed and the small number of topics we are able to review in a given year. Once a topic has been selected, we have a robust engagement strategy that is designed to encourage active collaboration with manufacturers throughout a review. We also have a robust engagement strategy that is designed to encourage active collaboration with patients and patient groups throughout a review.

## 6. Patient Engagement Program

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***Commenters were supportive of the new proposals to expand ICER’s patient engagement program. The proposals for patient compensation, small group patient and caregiver discussions, and the formation of the ICER Patient Council received the most positive feedback. Commenters proposed that we produce patient-friendly educational and summary materials to better engage the patient community in our process. In addition, we received comments requesting greater transparency around how patient engagement impacts the ICER assessment and around the activities and impact of the Patient Council.***

We thank the commenters for their feedback and support of our proposals to update the current patient engagement program. The evolution of our patient engagement process and the strategic updates to our engagement approach are largely a reflection of the input we have received from many patient groups and patients over the years. We look forward to continued iteration and expansion of this program so that we can engage the patient community in a manner that is accessible, equitable, and impactful.

Based on comments identifying the need for more patient-friendly educational materials, we have revised our VAF update to also include the creation of patient-friendly resources that will better explain our review process, how the patient community can participate, and how the patient perspective was incorporated into our report findings. We have piloted this initiative by creating a patient-friendly summary of our [2023 Final Evidence Report for Gene Therapies for Sickle Cell Disease](#), published in August of 2023. These new summaries and guides are intended to be co-created with our patient community partners when appropriate, and also reviewed by our Patient Council.

Following the public launch of our Patient Council in July of 2023, we have published a new page on our [Patient Portal](#) to introduce the members of the Council and describe the objective and aims for their current three-year term. We will explore how to best communicate the progress and impact of the Council, and feature these updates on the Patient Council webpage. We hope to also work with the Patient Council to develop new approaches for reporting on the impact of patient engagement in ICER reviews.

With regards to the new Share Your Story Form, we have clarified that this form will be available on our Patient Portal and also shared with each new patient group following onboarding. The Share Your Story Form aims to replicate many of the questions we ask during a scoping call with our review team. Responses to these questions, even on the individual level, help shape our thinking about the scope of our research, patient-important outcomes, and important context or challenges to prioritize in our understanding of the patient lived experience. We recognize the limitations and

barriers of the Share Your Story Form, and intend for it to serve as only one method of providing input into an ICER review.

We have also revised our framework to include a new opportunity on drug reviews for manufacturers to describe how they incorporated the patient perspective into their drug development programs. We will invite manufacturers to submit their methods for identifying patient-important outcomes for their clinical trial programs, and we will include this written description in our Evidence Report.

We have included the following clarifications to address a few comments that did not require an update to our existing patient engagement program:

- Patient advocates do already participate as voting members of all three of our independent appraisal committees: <https://icer.org/who-we-are/people/independent-appraisal-committees/>
- The current ICER patient engagement process does consistently engage the patient community throughout the review cycle from topic launch to scoping, report development, public meeting, and final report: <https://icer.org/patients/>
- We disagree that patient input is only of value when incorporated into the economic model. Health technology assessment incorporates clinical, economic, social, and ethical dimensions of value, and the patient perspective meaningfully informs all of these dimensions, even if no quantitative data exist.
- We are pleased to share that ICER has been utilizing the National Health Council's Fair Market Value calculator to determine the appropriate level of payment for our patient participants.

We again thank the commenters for their thoughtful feedback and suggestions for our 2023 Value Assessment Framework, and hope the implementation of the proposed updates will enhance our patient engagement program to be more inclusive and transparent.

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