



2023 Value Assessment Framework

Proposed Changes

June 5, 2023

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Executive Summary

This document describes proposed updates to the ICER value assessment framework. These proposals build on several years of experience with the current framework, which was updated in 2019 from a prior version first posted in 2017. This update will include key changes identified in our 2023 white paper on Advancing Health Technology Assessment Methods that Support Health Equity.¹

In this Executive Summary, we describe proposed changes to the current value assessment framework; the full text contains additional discussion of the rationale behind the proposed changes. Elements of the framework that will remain unchanged are generally not discussed in detail in this document, and full descriptions of the entire framework can be found in the 2020 - 2023 Value Assessment Framework² and its adaptations posted to [ICER's website](#).

The changes to the value assessment framework proposed in this document will be subject to a public comment period from June 5 through June 30, 2023. After reviewing all public comments, ICER will reflect further and make any final changes before releasing its Final 2023 Value Assessment Framework on or around September 25, 2023.

Comparative Clinical Effectiveness

Clinical Trial Diversity

1. ICER's reports will include a new subsection called "Clinical Trial Diversity". This section will present information on the demographic diversity of participants in the clinical trials that inform ICER's assessments.
2. ICER will provide an overall diversity rating for the following demographic characteristics: race/ethnicity, sex, and age, specifically, adults aged 65 and older. To do this objectively and consistently across all ICER assessments, ICER will apply a new framework for evaluating clinical trial diversity based on the best practices described in our white paper on HTA methods and health equity.

3. In recognition of the potential barriers for multinational clinical trials to reflect the racial and ethnic diversity of the disease population in the US, the racial and ethnic diversity rating will focus only on the subgroup of patients recruited exclusively in the US if these data are available; if these data are not published and not provided to ICER, ICER will focus on the diversity of the entire trial population. Trials conducted exclusively in other countries will not be rated on race and ethnicity, as they are unlikely to be representative of the racial and ethnic diversity of the US population.

Subpopulation Analyses

1. To ensure that our reviews focus on evaluating the most relevant subpopulations, ICER will include an *a priori* list of the subpopulation(s) of interest and the scientific rationale for evaluating these subpopulations in the scoping document and research protocol.
2. ICER will consider race, sex, and age as a presumptive subpopulation for every review. During topic scoping, we will evaluate the current evidence base and consult with clinical experts, patients, patient groups, and other stakeholders to investigate the relevance of subpopulations defined by these characteristics for the topic under consideration. Information gathered during scoping may lead us to conclude that further consideration of subpopulations defined by these characteristics is not warranted or that additional information is needed to proceed. In such cases, our scoping document and research protocol will describe our rationale for not including these subpopulations.
3. ICER will continue to include the subsection “Heterogeneity and Subgroups” in all its reports to present the primary discussion of subgroup effects or highlight the other sections of the evidence review that discuss subgroup effects. In addition, to ensure the information on the credibility of subgroup analyses is presented in clear and consistent terms across all ICER reports, ICER will start using a formal credibility assessment tool ([ICEMAN for RCT](#): Instrument for Assessing the Credibility of Effect Modification Analyses) to evaluate and present information on the credibility of subgroup findings.³
4. As stated in the current Value Assessment Framework, ICER will consider issuing separate evidence ratings for an intervention if there is robust, high-quality evidence that supports substantial differences in the comparative clinical effectiveness of the intervention across subpopulations.

Long-Term Cost Effectiveness

Perspective in Economic Models

1. In each assessment, ICER will continue to report cost-effectiveness results from both the health care system perspective as well as a modified societal perspective. ICER will implement new methods to ensure that cost-effectiveness analyses done according to a modified societal perspective have “non-zero” inputs for impacts on productivity for the patient and caregivers, even when direct data are lacking.

Dynamic Pricing Scenario

1. ICER will conduct a dynamic pricing scenario for small molecule and biological products that are predominantly targeted to Medicare-eligible populations. This scenario will generate cost-effectiveness findings assuming that new drugs and some comparator drugs will be subject to CMS price negotiation at a future time point and will have a reduction in price at that time to the relevant ceiling price as described in the Inflation Reduction Act.

Quantifying Additional Dimensions of Value

1. After ongoing consideration of the potential to perform quantitative analyses of additional dimensions of value, such as those reviewed by an ISPOR Task Force,⁴ we believe that methodological issues related to double counting and the inability to measure related opportunity costs present a strong argument to keep these dimensions as qualitative considerations at this time.
2. To support tangible consideration of severity as a potential modifier of the value of health gains, we will regularly calculate QALY and evLYG shortfall measures to accompany primary cost-effectiveness analysis results and will include these findings in material presented during public deliberation by appraisal committees on the long-term value for money of treatments.
3. After piloting the Health Improvement Distribution Index (HIDI), ICER will continue to calculate this measure and include it in reports and as a part of the public deliberation related to the potential benefit of some interventions to have a positive impact on health equity. As noted in our white paper on HTA and methods related to health equity, we will be careful to frame the HIDI as just one element in considering the potential impact on health equity.

Health Benefit Price Benchmarks

1. ICER will continue to frame our Health Benefit Price Benchmarks (HBPB) based on analyses using the QALY at the \$100,000 threshold and the evLYG at the \$150,000 threshold. However, we will emphasize that policymakers who prefer or who may be mandated to consider only measures of health gain other than the quality-adjusted life year (QALY) can find results at every threshold based solely on the equal value of life-years gained (evLYG).

ICER remains committed to an opportunity cost perspective on determining appropriate cost-effectiveness thresholds for decision-making. Within this paradigm, academic work suggests a top threshold at approximately \$104,000 per QALY based on direct health losses within the health system perspective. The calculations underlying this estimate do not include consideration of the significant negative effects of self-rationing caused by increasing insurance premiums and health care costs for those who retain insurance coverage. If additional elements of value are quantified as benefits of new interventions, there is also the risk of unmeasured equal or greater losses within these same elements among those individuals who drop insurance coverage. We also note that consideration of health equity would suggest that individuals with lower incomes experience a disproportionate share of the harms from the opportunity costs imposed by increasing health insurance premiums. Working from these insights, although we are not proposing to change our effective threshold range for price benchmarks at the current time, we will pursue further discussion with academic experts and stakeholders to consider whether the Health Benefit Price Benchmark Price range should be shifted to \$50,000 to \$100,000 per QALY or evLYG in order to better reflect the true opportunity costs experienced by many Americans.

Other Changes

1. ICER's Reference Case will be revised to reflect any of the proposed and adopted revisions.
2. ICER will continue to seek opportunities to use real-world evidence within our assessments.

Potential Other Benefits and Contextual Considerations

1. ICER will change the terms used to describe these elements to “Benefits Beyond Health” and “Special Ethical Priorities.” The structure of the ICER report will continue to highlight these elements in a separate section.
2. ICER will change the voting categories and framework to address overlap and misinterpretation in certain areas and to seek to create a more actionable set of voting results on key elements. The voting structure will be changed to a “Strongly Disagree” to “Strongly Agree” five-point Likert scale on four elements, as shown below. Votes in the public meeting will be presented as average scores across all voting appraisal committee members:

Please vote from 1-5, from “Strongly Disagree” to “Strongly Agree” on the following statements related to the value of this treatment in the context of this condition.

- Patients: There is significant unmet need despite currently available treatments.
- Caregivers: The treatment is likely to produce significant improvement in caregivers’ quality of life and/or ability to pursue their own education, work, and family life.
- Health equity 1: The condition is of heightened relevance for disadvantaged communities.
- Health equity 2: The treatment offers a significant opportunity to improve access to effective treatment by means of its mechanism of action or method of delivery.

Topic Selection

1. Strengthen approach for elevating health disparities that may be related to emerging drugs considered for ICER topic selection.

Patient Engagement Program

1. Amplify the new “Share Your Story” online form to increase accessibility and ease of individual patient and caregiver testimonials at the initial phase of ICER’s process.
2. Formalize small-group patient and caregiver discussions after the scoping phase, ensuring inclusion of diverse patient community voices, to enhance understanding of the lived experience.
3. Compensate patient representatives fairly for their time, expertise, and contributions to the small-group patient discussions and public meetings.

4. Enhance accessibility and inclusivity of public meetings that allow for remote attendance and closed captions on virtual meetings.
5. Convene a Patient Council to advise and strengthen ICER's current Patient Engagement Program.

1. Introduction

This document describes proposed updates to the ICER value assessment framework. These proposals build on several years of experience with the current framework, which applied to reviews launched in July 2017 and later. The current framework was most recently updated in 2019 and published in January 2020.

In the sections that follow, we describe proposed changes to the current value assessment framework. Elements of the framework that will remain unchanged are generally not discussed in detail, and full descriptions of these elements can be found in the 2020-2023 Value Assessment Framework² and its adaptations posted to [ICER's website](#).

The proposals in this document will be subject to a public comment period from June 5 through June 30, 2023. After reviewing all public comments, ICER will reflect further and make any final changes before releasing our Final 2023 Value Assessment Framework on or around September 25, 2023.

1.1. Overarching Purpose and Principles of the ICER Value Assessment Framework

For more than 17 years, ICER has been active in developing methods for evidence assessment. The first effort to formalize ICER's methods was undertaken between 2014-2015 with input from a multi-stakeholder advisory group on ways to define with greater detail the conceptual and methodological underpinnings of ICER reports – a “value assessment framework.” This framework was updated in 2017 and in 2020, and special adaptations were also created for assessments of treatments for rare/ultra-rare disorders and for high-impact single or short-term therapies such as cell and gene therapies.

The purpose of the ICER value assessment framework is to form the backbone of rigorous, transparent evidence reports that, within a broader mechanism of stakeholder and public engagement, will help the United States evolve toward a health care system that provides fair pricing, fair access, and a sustainable platform for future innovation.

1.2. The Population Perspective and Intended Uses of the ICER Value Framework

The ICER value framework describes the conceptual framework and set of associated methods that guide the development of ICER evidence reports. ICER reports are intended to support deliberation on medical policies related to health services (e.g., tests or treatments) and delivery system

interventions (e.g., preventive programs, changes to the organization of medical personnel). To inform these kinds of medical policies the ICER value framework takes a “population” level perspective as opposed to trying to serve as a shared decision-making tool to be used by individual patients and their clinicians. Taking a population perspective implies that the ICER value framework seeks to analyze evidence in a way that supports population-level decisions and policies, such as broad guidelines on appropriate care, pricing, insurance coverage determinations, and payment mechanisms. A value framework intended to support decisions about the care of individual patients requires a structure that invites weighting of benefits, harms, and costs from the individual patient’s perspective. There is an important need for better evidence-based shared decision-making tools for individual patients and clinicians, but this is not the primary intended purpose of the ICER value framework or of ICER reports.

Even with its population-level focus, however, the ICER value framework seeks to encompass and reflect the experiences and values of patients. Representing the diversity of patient outcomes and values in a population-level framework is difficult because there will always be an inherent tension between average findings in clinical studies and the uniqueness of every patient. There will also always be diversity in the way that patients view the balance of risks and benefits of different treatment options. The ICER value framework does not solve these tensions, but neither does it obscure them. Population-level decisions and policies have always been made by life science companies, insurers, and clinical organizations looking at evidence in the same general way. One important goal of the ICER value framework is to provide an evidence report that does a better job of analyzing the strengths and limitations of the available evidence, including what is or is not known about the variation in response to different treatments among patients with different personal and clinical characteristics. The ICER value framework also creates an explicit place and role for consideration of elements of value that are important to individual patients but that fall outside traditional clinical measures.

2. Comparative Clinical Effectiveness

2.1 Clinical Trial Diversity

Proposed Changes

1. ICER's reports will include a new subsection called "Clinical Trial Diversity." This section will present information on the demographic diversity of participants in the clinical trials that inform ICER's assessments.
2. ICER will provide an overall diversity rating for the following demographic characteristics: race/ethnicity, sex, and age, specifically, adults aged 65 and older. To do this objectively and consistently across all ICER assessments, ICER has developed a framework for evaluating clinical trial diversity based on the potential best practices described in our white paper on Advancing Health Technology Assessment Methods that Support Health Equity.¹ Specifically, as shown in Table 1 below, the ICER-developed framework assigns a score that ranges from 0 to 3 to each demographic category based on the estimated participation-to-prevalence ratios. Then, using the cumulative score and pre-defined cut points, a rating of "good," "fair," or "poor" will be used to communicate the demographic diversity of the participants in a clinical trial (see Table 2).
3. In recognition of the potential barriers for multinational clinical trials to reflect the racial and ethnic diversity of the disease population in the US, the racial and ethnic diversity rating will focus only on the subgroup of patients recruited exclusively in the US if these data are available; if these data are not published and not provided to ICER, ICER will focus on the diversity of the entire trial population. Trials conducted exclusively in other countries will not be rated on race and ethnicity, as they are unlikely to be representative of the racial and ethnic diversity of the US population.

Table 2.1. Representation Score

PDRR (Participant to Disease Prevalent Ratio)	Representation Score
0	0
>0 and Less than 0.5	1
0.5 to 0.8	2
≥0.8	3

Table 2.2. Rating Categories

Demographic Characteristics	Demographic Categories Included in Rating	Maximum Score	Rating Categories (Total Score)
Race and Ethnicity*	Asian Black or African American White Hispanic or Latino	12	Good (11-12) Fair (7-10) Poor (≤6)
Sex	Male Female	6	Good (6) Fair (5) Poor (≤4)
Age	Older adults (≥65 years)	3	Good (3) Fair (2) Poor (≤1)

* American Indian or Alaskan Native & Native Hawaiian or Other Pacific Islander are not factored into the overall racial and diversity rating. However, information on enrollment and PDRR estimates are reported when reliable prevalence estimates are available.

Discussion

The lack of diversity in clinical trial populations is an ongoing problem, and many stakeholders agree that driving change on a broader level requires investment, transparency, accountability, and partnerships across key stakeholders. As an HTA body, ICER has considered our role in promoting equity in clinical trials of new drugs. As a result, ICER is proposing to capture the demographic diversity of clinical trials included in our reviews in a dedicated subsection of our report. In addition, ICER will provide an overall diversity rating for the following demographic characteristics: race/ethnicity, sex, and age (older adults). We believe rating clinical trials on diversity has the potential to stimulate the conversation on clinical trial diversity and enhance transparency and accountability. Furthermore, it encourages and recognizes the investment and efforts of drug developers that have appropriately included diverse participants in their drug development program and reassures patients that the approved drugs were tested on trial participants like them.

As noted above, in order to do this objectively and consistently across all ICER assessments, we have developed a framework for evaluating clinical trial diversity based on the potential best practices described in our white paper on Advancing Health Technology Assessment Methods that Support Health Equity.¹ This framework relies on evaluating clinical trial diversity quantitatively by comparing clinical trial participants to disease-specific prevalence estimates and using the thresholds defined in Tables 2.1. and 2.2. above to judge representation. ICER will review the following reliable sources for disease-specific prevalence estimates: the Centers for Disease Control and Prevention website and the Global Burden of Disease database, a comprehensive epidemiologic dataset by country supported by the World Health Organization. In addition, a literature search will be conducted to obtain peer-reviewed journal articles that estimate the prevalence of US disease by sex, age, race, and ethnicity. ICER recognizes that there may be a lack of reliable disease-specific prevalence estimates for some conditions, particularly rare diseases. When there are no reliable disease-specific prevalence estimates, consideration would be given to comparing clinical trial

participants to population estimates (US census demographic breakdown) and interpreting the finding accordingly.

The advantage of using the ICER-developed clinical trial diversity framework is that it provides diversity ratings on different demographic characteristics of interest that all stakeholders can easily interpret. However, one potential challenge we anticipated is the current trend of global trials. We recognize the potential barriers for clinical trials conducted in other countries to reflect the racial and ethnic diversity of the disease population in the US. As such, for multinational clinical trials, we propose focusing the racial and ethnic diversity rating only on the subpopulation of patients enrolled in the US. Information on the racial and ethnic diversity of the overall patient population will only be provided for context as needed. ICER knows 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.

2.2 Subpopulation Analyses

Proposed Changes

1. To ensure that our reviews focus on evaluating the most relevant subpopulations, ICER will include an *a priori* list of the subpopulation of interest and the scientific rationale for evaluating these subpopulations in the scoping document and research protocol. ICER will rely on targeted literature reviews and interviews with patient and clinical experts conducted during scoping to identify the most relevant subpopulations.
2. ICER will consider race, sex, and age as presumptive subpopulations for every review. During topic scoping, we will evaluate the current evidence base and consult with clinical experts, patients, patient groups, and other stakeholders to investigate the relevance of subpopulations defined by these characteristics for the topic under consideration. Information gathered during scoping may lead us to conclude that further consideration of subpopulations defined by these characteristics is not warranted or that additional information is needed to proceed. In such cases, our scoping document and research protocol will describe our rationale for not including these subpopulations.

ICER will continue to include the subsection “Heterogeneity and Subgroups” in all of our reports to present the primary discussion of subgroup effects or highlight the other sections of the evidence review that discuss subgroup effects. In addition, to ensure the information on the credibility of subgroup analyses is presented in clear and consistent terms across all ICER reports, ICER will start using a formal credibility assessment tool ([ICEMAN for RCT](#): Instrument for Assessing the Credibility of Effect Modification Analyses) to evaluate and present information on the credibility of subgroup findings. The

development of this tool is full described [here](#).³

- 3. As stated in the current VAF, ICER will consider issuing different evidence ratings for an intervention if there is robust, high-quality evidence that supports substantial differences in the evidence ratings of the intervention across different populations or subgroups.**

Discussion

ICER's reviews are not intended to guide individual shared decision-making between clinicians and patients and are not able to focus on the sorts of individual patient characteristics, values, and preferences that a skilled clinician would assess in making recommendations for a specific patient. However, when evaluating the clinical effectiveness of an intervention, focusing on the average treatment effects alone may obscure the distinct needs, disease burden, or important treatment variations that may be present in certain subpopulations if proper consideration is not given to subgroup analysis. On the other hand, analyzing and interpreting results by subpopulations is often not straightforward due to methodological and statistical issues that create uncertainty about the validity and reproducibility of the findings. As such, there is a need for ICER to develop a consistent framework for knowing when and how to highlight heterogeneity of intervention effects in terms that all stakeholders can easily interpret or substantial differences in the evidence for a specific subpopulation to warrant a separate judgment for that group.

From the inception of the evaluation, ICER examines the contextual landscape of the topic under review, including what is known about sources of heterogeneity of intervention effect and concern about or known subpopulation differences for the disease area. ICER's current approach is to list the subpopulations of interest in the scoping document and research protocol without providing the rationale for including these subpopulations. Instead, ICER proposes that along with the list of subpopulations of interest, the scientific rationale for wanting to evaluate these subgroups will be described in the scoping document and research protocol. We believe this will help to ensure that our reviews focus on evaluating the most relevant subpopulations. In addition, the rationale for not including a presumptive subpopulation (race, sex, age) will also be stated. The decision about which subpopulations to evaluate will be based on careful consideration of the likelihood of a subgroup effect. Early investigations of a 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.

In all reports, the subsection “Heterogeneity and Subgroups” will be included to present information on the subgroup effects detected. In cases where subgroup effects are detected, ICER will evaluate the credibility of the subgroup claim using the Instrument for assessing the Credibility of Effect Modification Analyses ([ICEMAN for RCT](#)).³ The Instrument considers the following five key questions to evaluate the overall credibility of a subgroup claim:

1. Was the direction of the effect modification correctly hypothesized *a priori*?
2. Was the effect modification supported by prior evidence?
3. Does a test for interaction suggest that chance is an unlikely explanation of the apparent effect modification?
4. Did the authors test only a small number of effect modifiers or consider the number in their statistical analysis?
5. If the effect modifier is a continuous variable, were arbitrary cut points avoided?

Based on responses to these questions, ICER will judge the overall credibility of the subgroup effect claim as “very low credibility”, “low credibility”, “moderate credibility”, or “high credibility.”

ICER’s evidence rating of the clinical effectiveness of an intervention reflects a judgment of the magnitude of the difference between the intervention and its comparator (net health benefit) and the level of certainty in the best point estimate of net health benefit. As stated in the current VAF, ICER will consider issuing different evidence ratings for a single intervention if robust, high-quality evidence supports substantial differences in the evidence ratings of the intervention across different populations or subgroups. Considerations will be given to the relevance and evidence gaps before issuing a separate overall judgment/evidence rating.

Finally, in addition to the Heterogeneity and Subgroups section, ICER will consider other sections of the report where subpopulation effects should be highlighted. For example, in cases where there are knowable effect modifiers or substantial differences in baseline risk that lead to groups that will achieve larger or smaller absolute benefits from therapy, ICER will most likely highlight these differences in our discussion of the clinical evidence. In addition, in situations with low-quality evidence that suggests differences in the magnitude of net benefit, ICER may highlight the need for additional research on those subpopulations.

3. Long-Term Cost Effectiveness

3.1 Perspective in Economic Models

Proposed Changes

1. **In each assessment, ICER will continue to report cost-effectiveness results from both the health care system perspective as well as a modified societal perspective. ICER will implement new methods to ensure that cost-effectiveness analyses done according to a modified societal perspective have “non-zero” inputs for impacts on productivity for the patient and caregivers, even when direct data are lacking.**

Discussion

ICER will continue to rely on the health care system perspective as the foundation for sensitivity and scenario analyses. If evidence is available to suggest either patient productivity time related differences and/or carer-time differences with the intervention of interest, then such evidence will be used in a modified societal perspective analysis. In such instances where evidence on patient time or carer time is available, ICER will continue to use the current VAF criteria to support decisions around when to promote the modified societal perspective to be co-equal in terms of its policy relevance and its inclusion in ICER’s Health Benefit Price Benchmarks.

There have been ICER reviews for which data unavailability has made it impossible to conduct an analysis from a modified societal perspective without the need to make unfounded assumptions or leave important elements as “zero.” For example, it is not uncommon for there to be no data on the effects of new drugs on non-health sector costs such as patient and carer productivity. We recognize that these are only a subset of non-health impacts that could be considered in the societal perspective, but we have found them to be among the most influential in discussions about the broader value of new treatments.

When direct data are lacking, ICER is therefore proposing to implement a method to capture the potential impacts of an intervention on patient productivity (formal and informal labor, household production, and leisure time) and carer time (time spent caring for patients) using an indirect approach. We believe that an indirect approach is appropriate and provides a reasonable balance between informing the potential impact of the treatment on broader outcomes and encouraging more research to be conducted to inform this perspective.

To inform estimates for the indirect approach, ICER will use the published relationship between patient utility scores and patient time use data⁵ to derive the anticipated impacts of the treatment on time spent in each activity due to the disease and its management for the patient. The indirect

approach values productivity time spent in a given health state, which is in contrast to the most typical approach of valuing productivity time lost, creating an opportunity to capture productivity time gained during periods of life extension. In these circumstances, and in line with the published literature⁵, ICER will include patient productivity time gained and patient consumption costs during periods of life extension. Since no parallel relationship between patient utility scores and carer time use data exists for the US setting, ICER will assume that carer time spent is proportional to 75% of patient formal labor time. This estimate is based on the modeled relationship between carer time required⁶ and patient time lost⁷ according to patient utility scores in the United Kingdom setting.

We will use the human capital approach⁸ to value all time gained according to the marginal pre-tax wage rate plus fringe benefits⁹ for formal and informal labor time and household production (\$41.86/hour [\$28.88/hour + \$12.98/hour]) and the post-tax (13.3%) wage rate without fringe for leisure time (\$25.04/hour).¹⁰ The same wage rates will be assumed for all patients and informal carers regardless of age, sex, and condition.

Table 3.1 Valuation of Productivity Time for Patient and Carer

Patient Productivity and Carer Time	Wage Rate*	Fringe Benefits*	Tax Rate*	References and Notes
Formal Labor Time	\$28.88	\$12.98	NA (pre-tax)	BLS data; accessed December 15 2022 ⁹
Informal Labor Time				
Household Production				
Leisure Time	\$25.04	NA	13.3%	2019 Federal income tax ¹⁰
Carer Time	\$28.88	\$12.98	NA (pre-tax)	75% of patient formal labor time (Mukuria 2017; Rowen 2016) ^{6,7}

*As of December 2022

3.2 Dynamic Pricing Scenario

Proposed Change

- 1. ICER will conduct a dynamic pricing scenario for small molecule and biological products that are predominantly targeted to Medicare-eligible populations. This scenario will generate cost-effectiveness findings assuming that new drugs and some comparator drugs will be subject to CMS price negotiation at a future time point and will have a reduction in price at that time to the relevant ceiling price as described in the Inflation Reduction Act.**

Based on the policies outlined by the Inflation Reduction Act, we believe it is reasonable to assume that for many drugs prescribed in the Medicare population, the net price increases will not be above inflation after launch. In addition, many drugs are likely to fall under the provisions for Medicare price negotiation after nine years post-approval for small molecule products and after 13 years post-approval for biological products. For example, for many small molecule drugs, the price after nine years may be assumed to drop to approximately 75% of the launch net price. The price of any active comparator may also become subject to Medicare negotiation as it hits nine or 13 years after approval, requiring attention to the possibility of mandatory price decreases at a future time point for multiple drugs in an ICER review.

Discussion

Some members of the academic health economics community have advocated for consideration of price increases or price decreases for drugs over the time horizon of cost-effectiveness analyses. Current academic practice, however, mirrored in the approach taken by international HTA agencies, has largely favored not factoring in these potential price changes due to the considerable uncertainties regarding price increases following launch and price decreases following the advent of generic competition. In addition, there is an ethical argument that opportunity costs at launch should not be obscured by the possibility of greater sharing in societal value by the health system many years downstream. ICER has published an academic paper¹¹ examining the contrasting effects of assumptions regarding price increases and price decreases during the market lifespan of biologic drugs.

But the recent passage of The Inflation Reduction Act (IRA) has changed the landscape in important ways. The IRA includes two components that are consistent with further exploration of changes in pricing over time, i.e., “dynamic pricing.” First, by assigning financial penalties for net price increases to Medicare above inflation, it seems reasonable to believe that US net drug price increases above inflation will become the exception rather than the norm. Second, the opportunity for the Centers for Medicare & Medicaid Services (CMS) to negotiate Medicare drug prices after nine years post-approval for some small molecule products and after 13 years post-approval for some biological products gives more certainty to the duration of time prior to drugs commonly used

in the Medicare population experiencing reductions in price. Because of these and other considerations, ICER proposes to add to reviews of drugs that are predominantly targeted to Medicare-eligible populations. This scenario will generate cost-effectiveness findings assuming the CMS relevant ceiling price at the earliest time possible. For example, following the outlines of the price discounts that will be used as the ceiling for Medicare price negotiation, for a small molecule, the price nine years after launch would be assumed to drop to 75% of the launch net price. For a biological agent, the price after 13 years post-approval would be assumed to drop to 65% of the launch net price. The price of any active comparator will also allow for projected price changes due to either loss of exclusivity or as described for the intervention of interest based on CMS negotiation.

ICER acknowledges multiple possible approaches and no recommended standards for how to include dynamic pricing within cost-effectiveness analyses. ICER is most likely to accommodate dynamic pricing within one modeled cohort that begins at the approval or launch of the intervention of interest and tracks this cohort over an appropriate time horizon (e.g., most commonly, a patient's lifetime). ICER notes that such an approach would not allow for changes in the cost-effectiveness findings for one-time cell or gene therapies. Due to additional uncertainty around the opportunity for competition to lower prices for one-time cell or gene therapies, ICER believes that drug price dynamics are more applicable for chronic therapies that are more likely to experience loss of exclusivity or Medicare price negotiation. ICER will track any further progress within the field on drug pricing dynamics to consider future changes beyond the proposed dynamic pricing scenario.

3.3 Quantifying Additional Dimensions of Value

Proposed Changes

- 1. No changes are proposed through which additional dimensions of value would receive a quantified weighting in the reference case incremental cost-effectiveness findings.**

After ongoing consideration of the potential to perform quantitative analyses of additional dimensions of value, such as those reviewed by an [ISPOR Task Force](#),⁴ we believe that methodological issues related to double counting and the inability to measure related opportunity costs present a strong argument to keep these dimensions as qualitative considerations at this time. Although the incremental cost-effectiveness findings will not be modified, unmet need or severity of disease measures will accompany the findings and will support deliberations on the long-term value for money of treatments.

- 2. To support tangible consideration of severity as another potential modifier of the value of health gains, we will regularly calculate QALY and evLYG shortfall measures to accompany primary cost-effectiveness analysis results and will include these findings in material**

presented during public deliberation by appraisal committees on the long-term value for money of treatments.

- 3. After piloting the Health Improvement Distribution Index (HIDI), ICER will include it in regularly as one measure of the opportunity for potential health gains to accrue disproportionately to a racial or ethnic subpopulation that has faced historical barriers to care resulting in disparities in health outcomes. The HIDI measure will be presented in public deliberation as part of the material relevant for judgments of long-term value for money.**

Discussion

Although academic research is emerging on methods that facilitate weighting cost-effectiveness findings based on broad concepts such as risk and equity, more research is needed to understand the implications and unintended consequences of modifying health gains quantitatively. The largest concern in weighting health gains is that there is limited research on the opportunity cost implications, including what additional elements of value may be “lost” in displaced services (or individuals), and whether the overall opportunity cost threshold should be adjusted accordingly. ICER will continue to monitor advances in methods as well as monitor changes made in the health technology assessment ecosystem prior to updating our approach to weighing the cost-effectiveness findings.

In the 2020 VAF, QALY shortfall measures were proposed to facilitate value deliberations. The QALY shortfall is intended to serve as a quantitative estimate of the quality of life lost and the life years lost that a patient experiences when living with a specific condition. For the 2023 update, we propose using evLYG shortfall measures and highlighting benchmarks of absolute and proportional shortfalls that would constitute a severe unmet need based on the present standard of care. The approach to calculating evLYG shortfall measures is similar to the approach for calculating QALY shortfall measures; however, a constant utility score reflective of the US population is modeled across all ages rather than using age-adjusted utility estimates when calculating the general population quality-adjusted life expectancy estimates, allowing an analysis that does not underweight the impact of conditions and treatments on patients with disabilities or chronic conditions. Benchmarks used by other HTA bodies could be highlighted, but will not be used to modify the cost-effectiveness findings. Even without modifying the cost-effectiveness findings, such evidence on shortfalls and corresponding benchmarks can facilitate appraisal committee deliberation and subsequent decision making, by highlighting the severe unmet need that exists for some conditions.

The HIDI is calculated as the disease prevalence in the subpopulation of interest divided by the disease prevalence in the overall population. A HIDI above one suggests that more health may be gained on the relative scale in the subpopulation of interest when compared to the population as a

whole. Importantly, the HIDI will certainly not substitute the deliberative process that should integrate multiple important equity criteria in policy decisions, but it can serve as one example of a quantitative measure of the relative prevalence of the condition across key subpopulations. Additional evidence, qualitative or quantitative, on other health equity considerations (e.g., access, uptake, etc.) will remain important for deliberation.

3.4 Health Benefit Price Benchmarks

Proposed Changes

- 1. ICER will continue to frame our Health Benefit Price Benchmarks (HBPB) based on analyses using the QALY at the \$100,000 threshold and the evLYG at the \$150,000 threshold. However, we will emphasize that policymakers who prefer or who may be mandated to consider only measures of health gain other than the QALY can find results at every threshold based solely on the evLYG.**

The starting point for ICER’s HBPB range uses the health care system perspective threshold-based prices from the highest and lowest annualized price within the \$100,000 to \$150,000 per QALY and per evLYG. The most common ICER HBPB range includes the treatment’s price that meets the \$100,000 per QALY gained on the low end and meets the \$150,000 per evLYG on the high end of our range. In highly unusual situations such as pandemics, in which there is an exceptionally large magnitude and urgency regarding the use of new health care interventions, ICER may consider using a lower cost-effectiveness threshold to provide additional accommodation between pricing to value and affordability.

Policymakers who prefer or who may be mandated to choose one measure of health gain can choose either the QALY or the evLYG. For those seeking either lower or higher thresholds for a particular measure of health gain, ICER will continue to provide threshold-based prices from \$50,000 to \$200,000 per QALY and per evLYG within our reports. The Perspectives discussion ([Section 3.1](#)) describes when ICER will promote the modified societal perspective to be co-equal in terms of its policy relevance and its inclusion in ICER’s Health Benefit Price Benchmarks. Finally, ICER may identify additional analyses within an assessment that are of relevance to policymaking such as [shared-savings analyses](#).¹² In such situations, ICER will highlight these analyses in the Draft Report and provide justification for their planned inclusion within ICER’s HBPB range in Final Reports.

- 1.a. ICER remains committed to an opportunity cost perspective on determining appropriate cost-effectiveness thresholds for decision-making that suggests a top threshold at approximately \$104,000 per QALY.¹³**

ICER is aware that academics and HTA agencies continue to conduct new research regarding appropriate cost-effectiveness thresholds for decision-making. ICER continues to believe that an opportunity-cost paradigm is most appropriate as a guide to decision-making in the US, and notes that important academic work in “willingness to pay” methods also suggest an operational threshold of approximately \$100,000 per QALY if a single threshold is desired. The calculations underlying opportunity-cost estimate do not include consideration of the significant negative effects of self-rationing caused by increasing insurance premiums and health care costs for those who retain insurance coverage. If additional elements of value are quantified as benefits of new interventions, there is also the risk of unmeasured equal or greater losses within these same elements among those individuals who drop insurance coverage. We also note that consideration of health equity would suggest that individuals with lower incomes experience a disproportionate share of the harms from the opportunity costs imposed by increasing health insurance premiums. Working from these insights, although we are not proposing to change our effective threshold range for price benchmarks at the current time, we will pursue further discussion with academic experts and stakeholders to consider whether the Health Benefit Price Benchmark Price range should be shifted to \$50,000 to \$100,000 per QALY or evLYG in order to better reflect the true opportunity costs experienced by many Americans. Inclusion of additional quantified elements of value without being able to measure their magnitude as part of the opportunity cost would create an even greater rationale for lowering the effective cost-effectiveness range.

1.b. For the Health Benefit Price Benchmarks of high-impact single or short-term therapies (SSTs),¹² or of other treatments with relevant and substantial potential cost-offsets, ICER will continue to consider the results of two scenario analyses:

- i. A 50/50 shared savings model in which 50% of the lifetime health system cost offsets from a new treatment are “assigned” to the health system instead of being assigned entirely to the new treatment; and**
- ii. A cost-offset cap model in which the health system cost offsets generated by a new treatment are capped at \$150,000 per year but are otherwise assigned entirely to the new treatment.**

ICER will be most likely to apply the results of one of these scenarios in framing our Health Benefit Price Benchmark when a) a large percentage of the traditional value-based price comes from cost offsets of comparator (e.g. standard of care) therapy; and b) the comparator therapy price is believed unlikely to meet common cost-effectiveness thresholds.

Discussion

ICER’s approach to HBPBs is not changing, but rather being codified into our Value Assessment Framework. As noted, earlier, ICER’s starting point for estimating our HBPB uses the health care

system perspective threshold-based prices from the highest and lowest annualized price within the \$100,000 to \$150,000 per QALY and per evLY.

On rare occasions, ICER will judge that the modified societal perspective findings should be included in some way in framing the HBPB, either as a “co-equal” HBPB or as the perspective used to generate the higher price of the HBPB range. In making this judgment, we will apply the following general guidance:

- An HBPB based upon findings from both the health care sector and modified societal perspectives will be presented when the impact of treatment on indirect costs such as patient and caregiver productivity, education, disability, or nursing home costs is judged to be substantial, and these costs are considered large in relation to health care costs associated with treatment of the condition. This will most often occur in cases where the incremental cost-effectiveness ratio changes by greater than 20%, greater than \$200,000 per evLYG or QALY, and/or when the result crosses thresholds of \$100,000-\$150,000 per evLYG or QALY. Due to additional uncertainty, in situations where the modified societal perspective is estimated based on ICER’s proposed missing data approach, the findings from this perspective will not be considered for inclusion into the HBPB.
- ICER notes that although studies have explored the willingness-to-pay thresholds for cost-effectiveness from both the health care system and societal perspectives, limited research has been conducted to estimate the opportunity-cost threshold from the societal perspective. If research expanded upon the Vanness et al. study¹³ to include broader societal domains within an opportunity cost paradigm, it would show a decreasing threshold from the original estimate of \$104,000 per QALY with each added broader societal element. Other HTA groups and academics¹⁴ share the view that with added dimensions of value comes the need to consider lowering the cost-effectiveness threshold for decision-making. Given the lack of definitive research and ICER’s acknowledgement that not all components of value can be quantified through cost-effectiveness, ICER will continue to apply the current threshold range of \$100,000 to \$150,000 per QALY and per evLYG to the determination of the HBPB when the modified societal perspective is used, even though lower thresholds likely apply when taking the societal perspective.
- ICER may identify additional analyses within an assessment that are of relevance to policymaking such as the shared-savings analyses. In the adaptation to ICER’s Value Assessment Framework for single- and short-term therapies,¹² ICER provides criteria for considering the shared-savings analyses as the basis for our HBPB.

3.5 Other Changes

Proposed Changes

1. ICER's Reference Case will be revised to reflect any of the proposed and adopted revisions.
2. ICER will continue to seek opportunities to use real-world evidence within our assessments. Manufacturers of products and other stakeholders now have the ability to update previous ICER findings with real world evidence (RWE) through the ICER Analytics platform. ICER will therefore not seek out further pilots of RWE to update prior assessments. ICER remains open to using RWE in all of our reviews, both in consideration of comparative clinical effectiveness and in the design of economic models.

Discussion

ICER acknowledges that the [Reference Case](#)¹⁵ is a more detailed guide to ICER's standardized approaches to cost-effectiveness. And, that detailed changes to the Reference Case are not being updated until after finalizing the changes to ICER's Value Assessment Framework proposed within this document. ICER's Health Economics team will be making updates to the 2020 Reference Case to ensure it is up-to-date with current practice and based on feedback on the proposed changes outlined in this document.

In consideration of ICER's mission and vision, ICER's impact is best achieved through assessment of interventions for clinical effectiveness and value around the time of US launch. ICER welcomes feedback on how best to use observational RWE toward achieving fair prices at launch as well as addressing other goals around fair access and future innovation.

4. Potential Other Benefits or Disadvantages and Contextual Considerations

4.1 List of Voting Questions and Voting Format

Proposed Changes

1. ICER will change the terms used to describe these elements to “Benefits Beyond Health” and “Special Ethical Priorities.” The structure of the ICER report will continue to highlight these elements in a separate section.
2. ICER will change the voting categories and framework to address overlap and misinterpretation in certain areas and to seek to create a more actionable set of voting results on key elements. The voting structure will be changed to a “Strongly Disagree” to “Strongly Agree” five-point Likert scale on four elements, as shown below. Votes in the public meeting will be presented as average scores across all voting appraisal committee members:

Please vote from 1-5, from “Strongly Disagree” to “Strongly Agree” on the following statements related to the value of this treatment in the context of this condition.

- Patients: There is significant unmet need despite currently available treatments.
- Caregivers: The treatment is likely to produce significant improvement in caregivers’ quality of life and/or ability to pursue their own education, work, and family life.
- Health equity 1: The condition is of heightened relevance for disadvantaged communities.
- Health equity 2: The treatment offers a significant opportunity to improve access to effective treatment by means of its mechanism of action or method of delivery.

Discussion

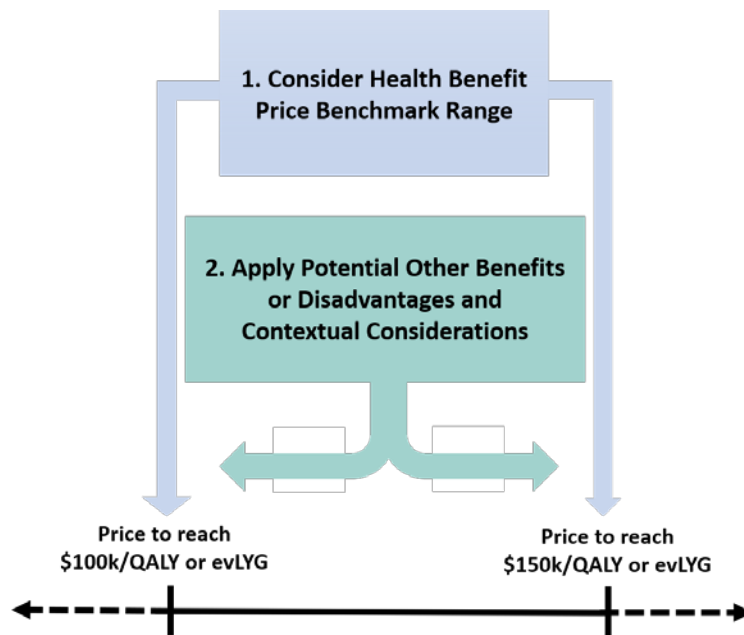
The inclusion of explicit domains of value labeled “potential other benefits or disadvantages” and “contextual considerations” in the 2020-2023 version of ICER value assessment framework has been a core feature of all iterations of the framework since its inception. These domains fully acknowledge that often what matters 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 from both the patient perspective and that of the health system and society that are either unmeasured in clinical trials or that reflect social or ethical values

that in some way may influence the relative weight decision-makers place on health gains and uncertainty when making an overall judgment of value for money for any specific new treatment option.

Many researchers and policymakers have explored different ways to elicit potential other benefits and contextual considerations and apply them to weight QALYs or adjust cost-effectiveness thresholds. However, any method by which additional domains of risks and benefits are added quantitatively, or health gains themselves are quantitatively adjusted to reflect “values,” involves potential risks. For example, the impact of treatment on patients’ relative productivity in the workplace is often measured as part of the cost-effectiveness modeling from a societal perspective. However, making productivity a core quantitative element in considerations of value will adversely affect the relative value of treatments for people who are elderly or disabled. Therefore, there are no widely accepted protocols for how best to weight factors outside traditional cost-effectiveness analysis, and most health technology assessment groups around the world do not attempt to quantify these domains of value, believing that their relative weight in any overall judgment of value should be left qualitative and subject to a public deliberative process.

Over the past decade, ICER has considered several methodological options that could enhance the transparency and explicit integration of these considerations in deliberation. 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 the reliability of value judgments. ICER has attempted formal MCDA with its independent committees on several occasions in the past and found the technique too complicated and unreliable for regular use. Based on discussions with stakeholders, benchmarking other value frameworks around the world, and the input of public comment in previous cycles of updates to our value assessment framework, ICER reports will continue to use a variation on MCDA that makes other benefits or disadvantages and contextual considerations explicit and part of the formal judgment on value for money without attempting an overly facile quantification. The figure (Figure 4.1) we have used since 2020 to communicate this vision for integration of potential other benefits and contextual considerations into a judgement of value for money is shown below.

Figure 4.1. Conceptual Guide to Application of “Potential Other Benefits or Disadvantages” and “Contextual Considerations” to Judgements of Value



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

We are proposing this year to change our term for potential other benefits or disadvantages to “Benefits Beyond Health.” We are also proposing to change the term for contextual considerations to “Special Ethical Priorities.” The change to Benefits Beyond Health is intended primarily to create a shorter and more clear term that specifies that we seek to examine the impact of the intervention on elements of value “beyond” health. The change away from the term contextual considerations is due largely to feedback from the patient community. We have heard that labeling important elements of value as merely “contextual” feels dismissive. We hope that the new term better connotes the overarching importance of these considerations as well as being more explicit that they involve what many would call social or ethical priorities.

We are also proposing to change the list of specific elements on which our appraisal committees will vote, along with a new voting structure. Feedback from our appraisal committee members and from the patient community has aligned with our own judgment that the list involved elements with differing degrees of overlap, that the terms being used in relation to different types of “severity” of illness created confusion, and that the voting question on health equity was not clear in whether the goal was to capture the opportunity to improve health equity or the reality that in the US health care system most new interventions, especially if expensive, will only accentuate barriers to access for individuals who have fewer economic and social resources.

Some consideration of the severity of the condition is viewed by many academics and stakeholders as an important consideration in judgments of value of treatment, but HTA agencies have conceptualized this idea somewhat differently in different countries. Some have seen that giving

some priority to treatments according to “lifetime burden of illness” or “need” may better represent the ethical instincts of their society or other decision-makers.

Our current methods have asked appraisal committee members to vote separately on severity of illness and lifetime burden of illness without providing any specific conceptual or empirical guidance. We believe that we can gain greater clarity and consistency in consideration of these issues by moving to a question that asks whether there is significant “unmet need” despite currently available treatments. In pilot testing this question with a subgroup of appraisal committee members we found that this framing was the easiest way for them to express their view of how severity should influence the thinking about the value of a new treatment. To inform this vote we will have perspectives from patients and patient groups in the ICER report, and we will also calculate absolute and proportional evLYG shortfalls.

The absolute shortfall is defined as the total absolute amount of future health patients with a condition are expected to lose without the treatment that is being assessed.¹⁶ It can be measured over the entire lifetime of patients with a condition, but more often it is measured from the point at which patients are diagnosed with a condition. By capturing the magnitude of the number of evLYs lost, the absolute evLY shortfall reflects the aspect of severity of illness related to the idea that treatments for people who stand to lose the most absolute numbers of evLYs should merit some increased prioritization. The ethical consequences of using absolute evLY shortfall to prioritize treatments is that conditions that cause early death or that have very serious lifelong effects on quality of life receive the greatest prioritization. Thus, certain kinds of treatments, such as treatments for rapidly fatal conditions of children, or for lifelong disabling conditions, will score highest on the scale of absolute evLY shortfall. The Norwegian health technology assessment program is perhaps the most notable organization currently using measures of absolute shortfall as a component in their appraisal process.¹⁷

Absolute shortfalls are often viewed in contrast to another way to empirically measure a sense of severity of illness, or “need” as the Dutch have called it.¹⁸ This alternative measure is called a proportional shortfall. The proportional evLY shortfall is measured by calculating the proportion of the total evLYs of remaining life expectancy that would be lost due to untreated illness.¹⁹ The proportional evLY shortfall reflects the ethical instinct to prioritize treatments for patients whose illness would rob them of a large percentage of their expected remaining lifetime. As with absolute evLY shortfall, rapidly fatal conditions of childhood will have high proportional evLY shortfalls, but the highest numbers can also often arise from severe conditions among the elderly who may have only a few years left of average life expectancy but would lose much of that to the illness without treatment.

Absolute and proportional shortfalls are therefore empirical measurements that capture different aspects of society’s instincts for prioritization related to the severity or burden of an illness. Because they can be viewed as complementary in some ways, we propose to calculate both

measures for every intervention. We will include these results in our reports and highlight them when asking our independent appraisal committees to vote on unmet need despite current treatment options.

The next voting question is a reformulation of our existing question on caregiver impact. The new language is framed to provide an easier way for appraisal committee members to register their view on whether the treatment will have a significant impact. We believe a “significant impact” on caregivers will register as a more tangible and actionable voting result for decision-makers.

We are also proposing to change the framing of the single vote we have had on health equity. For this we propose introducing two different questions, the first of which asks whether the condition is of particular relevance to a disadvantaged community. The HIDI score will be relevant to the committee’s thinking here, as will information gathered from patients in the ICER report and further testimony from patient groups and clinical experts at the public meeting. The second question related to health equity now asks whether there is an “opportunity” to improve equity through the specific mechanism of improving access due to a treatment’s mechanism of action or delivery. Pilot testing on these questions also suggested that they would be better at separating the different ways that a new treatment could be viewed as having an impact on health equity.

In addition to changing the specific questions asked, we are also changing the voting structure from a Likert scale of “relative priority” to a Likert scale on “agreement.” From discussion with appraisal committee members, we have concluded that the existing voting structure has proven vulnerable to uncertainty about anchoring at the upper and lower bounds, often leading to a floor effect in which all appraisal committee members voted at the higher end of the scale in order not to risk that patients would feel that their condition was viewed as less important than others. Having a voting scale that does not differentiate well among different situations is less effective in influencing decision-makers, so we believe that a change in our voting structure will enhance the integration of these elements into final decisions regarding value for money, both among our appraisal committee members and among health care decision-makers.

With these changes in mind, it is important to note that many key aspects of our approach to integrating key elements beyond the clinical and cost-effectiveness data into our reports and public meetings will remain the same. ICER will continue to provide guidance to our appraisal committees and to health care decision-makers that consideration of Benefits Beyond Health and Special Ethical Priorities 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 benefits and/or priorities, and lower ends of the range reflecting relatively less consequential added value considerations. In ICER public deliberation meetings, independent appraisal committees will continue to take votes on each specific element so they can be highlighted for decision-makers.

Methods to integrate these elements into HTA reports and public deliberation are one of the most active areas of research in applied health economics, and we will continue to monitor this literature, participate and lead research when feasible, and remain ready to seek improvements to our methods in an iterative fashion. In particular, we are monitoring the research and conceptual analyses on modifying health gains quantitatively in relation to some measure of severity. As noted earlier, we do not feel that this research is mature enough to suggest a single weight by which the health gains of some conditions would be upgraded – or downgraded – depending on the level of severity, and we continue to believe that qualitative consideration of this factor in public deliberation is a preferable manner to address if and how to integrate this ethical priority into decision-making.

ICER Processes for Conducting Value Assessments

Proposed Changes

June 5, 2023

A1. Introduction

This section describes proposed updates to ICER’s processes for topic selection and stakeholder engagement related to conducting value assessments of new technologies. Prior to 2023, these sections have been included in ICER’s value assessment framework.² Once these proposals are finalized in September 2023, ICER’s value assessment framework will only include information about the conceptual structure and methods of value assessment. All processes described in this section here will be published on ICER’s website in a separate document. Going forward, these processes will be updated on an ad hoc basis, and will not follow the standard three-year update cycle that the ICER value assessment framework currently follows.

In this section we will describe proposed changes to our current processes and discuss the rationale behind the proposed changes. Not all processes are currently being changed, and thus this is not an exhaustive overview of all processes related to the key areas highlighted in this document.

The proposals in this section will be subject to a public comment period from June 5 through June 30, 2023. After reviewing all public comments, ICER will reflect further and make any final changes before releasing our final overview on or around September 25, 2023.

A2. Topic Selection

Proposed Changes

- 1. Strengthen approach for elevating health disparities that may be related to emerging drugs considered for ICER topic selection.**

Discussion

ICER seeks to evaluate new pharmaceutical treatments and other health care interventions that may significantly improve patient outcomes, raise questions about their comparative effectiveness in relation to other treatment options, or that may have a significant financial impact on patients or the broader health system. ICER’s current framing of topic selection criteria include one related to health equity: “involve(s) underserved populations with the potential to reduce health disparities.”

ICER recently published a white paper on “Advancing Health Technology Assessment Methods that Support Health Equity” recommending that health technology assessment organizations such as ICER to include clear mechanisms for integrating health equity considerations into topic selection.¹ Therefore, we are proposing to incorporate health-equity related issues into our horizon scanning and topic selection processes by elevating any relevant health disparities particular to the disease under consideration. When relevant, information will be presented regarding health disparities across racial, ethnic, or socioeconomic groups. If available, these data will be formally presented during horizon scanning and topic selection meetings to be considered when selecting a topic for review.

A3. Stakeholder Engagement

2.1. Patient Engagement Program

Proposed Changes

Building on the comprehensive framework from 2020, ICER proposes the following enhancements, some of which have already been piloted during the previous year:

1. Amplify the new “Share Your Story” online form to increase accessibility and ease of individual patient and caregiver testimonials at the initial phase of ICER’s process.
2. Formalize small-group patient and caregiver discussions after the scoping phase, ensuring inclusion of diverse patient community voices, to enhance understanding of the lived experience.
3. Compensate patient representatives fairly for their time, expertise, and contributions to the small-group patient discussions and public meetings.
4. Enhance accessibility and inclusivity of public meetings that allow for remote attendance and closed captions on virtual meetings.
5. Convene a Patient Council to advise and strengthen ICER’s current Patient Engagement Program.

Discussion

One goal of ICER’s Patient Engagement Program is to help ensure a diversity of patient voices can contribute to our assessments. Several initiatives have been developed in consultation with patient community leaders to facilitate greater awareness of ICER and opportunities for patient input. Still, the patient community feedback remains that communications remain too high level, technical and seemingly out of reach for many patients who are thus going unheard in the value assessment process.

We recognize this challenge and have been working with a dedicated group of patient leaders to expand communication to these constituents. The proposals in this update reflect ongoing discussions about how to best communicate the role of ICER in the US healthcare system, why patient voices are critical to the assessment process, how patient input is used within ICER’s research and the impact it has on policymaking.

Our proposed updates also aim to create a process for patient input into health technology assessment that will support health equity and reduce barriers to participation.

Share Your Story Form

In response to patient community input, we have simplified the previous Patient and Care Partner Input Questionnaire into the [Share Your Story Form](#). This change was partly to clarify that the form is not intended to be a validated survey tool but rather to help ground the ICER team in a stronger qualitative understanding of the patient lived experience. The five-question form asks about the impact of the disease on daily life, experience with past or current treatments, hopes for a new treatment, access and affordability challenges, and impact on caregivers. The Share Your Story form was introduced in mid-2022 and a broader communication around its availability is planned as part of this VAF update.

Small Group Patient and Caregiver Discussions

The previous VAF included a provision to co-create and field surveys with the patient community to address specific data gaps. In practice, these surveys have been difficult to implement due to resource and timing challenges, which were further exacerbated as patient communities had other priorities during the COVID pandemic. ICER instead added small group patient and caregiver discussions in order to fill this gap and provide an opportunity to probe deeper into patient insights. These discussions last for one hour and typically include 4-5 individual patients or caregivers and a smaller subset of the ICER review team. Our goal is to have between one to three of these group discussions per drug review, allowing us to speak to a greater diversity of patients than we had before. We aim to conduct these group calls after the Revised Scope is published and include a narrative summary of the key patient insights in the Patient Perspectives chapter of our report. These group discussions have proved highly valuable since they were introduced and were thus standardized in early 2022. With this VAF update, ICER will add these patient and caregiver discussions as a core part of our stakeholder engagement process, while also maintaining the flexibility to conduct surveys if the need and capability arises to do so.

Honoraria

With a commitment to help address potential financial barriers that may hinder the inclusion of diverse patient participation in our process, ICER has formalized honoraria payments for select activities requiring more extensive contributions. Individual patients and caregivers who participate in a small-group discussion will be compensated \$100 for one hour of their time. Patient experts who review and provide feedback on our draft evidence reports will be compensated \$500. Patient experts who serve as panelists at our full-day public meeting and participate in our policy roundtable will be compensated \$500. These honoraria amounts are comparable to the honoraria provided to other experts or stakeholders who participate in our reviews in the same capacity.

Enhancements to the Public Meetings

We recognize that logistical barriers also exist in the patient community's ability to participate in our process. ICER now always offers virtual attendance at our public meetings and options for remote public testimony for those who may not be able to join in real time due to other constraints. If ICER returns to in-person meetings, we are committed to offering a hybrid meeting structure that continues to support virtual attendance, while also offering travel grants to accommodate any financial barriers for patients who may be interested in providing their testimony in-person.

In addition, closed captioning is now available for all public meetings and public webinar livestreams and recordings to enhance the inclusivity of our meetings.

ICER Patient Council

In June 2023, ICER plans to formally announce the creation of a Patient Council that will advise on ICER's patient engagement strategy, outreach, and processes for input into our evidence reviews and broader initiatives. The Council is comprised of six patient advocates who represent a diverse range of communities. Council members will meet quarterly to evaluate ICER's current patient engagement program, identify opportunities to strengthen the process and diversity of participation, and advise on ensuring accessibility and inclusivity of ICER's public-facing materials. Most of the members have not previously collaborated with ICER, offering a fresh perspective and evaluation of the ICER process, and how to better communicate the value of patient participation in the ICER process.

Conclusion

All the proposed changes to our patient engagement process reflect our ongoing commitment to listen to the patient community, identify gaps in outreach and effective communication, and update our approach to better facilitate patient engagement. We are hopeful that the updates to our honoraria payments and public meeting travel assistance can begin to address some of the financial barriers that prevent members of the patient community from participating in our process. Furthermore, the updates to the Share Your Story Form, small group patient discussions, and closed captioning on meetings are intended to increase both the accessibility and diversity of participation and input into our process. Lastly, the launch of our Patient Council signals our long-term commitment to partner with the patient community and iterate on our patient engagement strategy more broadly and consistently.

References

1. Agboola F, Whittington MD, Pearson SD. Advancing Health Technology Assessment Methods that Support Health Equity. 2023.
2. Institute for Clinical and Economic Review. 2020-2023 Value Assessment Framework. https://icer.org/wp-content/uploads/2020/11/ICER_2020_2023_VAF_02032022.pdf. Published 2020. Updated January 31, 2020. Accessed.
3. Schandelmaier S, Briel M, Varadhan R, et al. Development of the Instrument to assess the Credibility of Effect Modification Analyses (ICEMAN) in randomized controlled trials and meta-analyses. *Cmaj*. 2020;192(32):E901-E906.
4. Lakdawalla DN, Doshi JA, Garrison LP, Jr., Phelps CE, Basu A, Danzon PM. Defining Elements of Value in Health Care-A Health Economics Approach: An ISPOR Special Task Force Report [3]. *Value Health*. 2018;21(2):131-139.
5. Jiao B, Basu A. Associating Health-Related Quality-of-Life Score with Time Uses to Inform Productivity Measures in Cost-Effectiveness Analysis. *Pharmacoeconomics*. 2023:1-13.
6. Rowen D, Dixon S, Hernández-Alava M, Mukuria C. Estimating informal care inputs associated with EQ-5D for use in economic evaluation. *The European Journal of Health Economics*. 2016;17:733-744.
7. Mukuria C, Rowen D, Hernández-Alava M, Dixon S, Ara R. Predicting productivity losses from health-related quality of life using patient data. *Applied Health Economics and Health Policy*. 2017;15:597-614.
8. Basu A. *Estimating costs and valuations of non-health benefits in cost-effectiveness analysis*. Vol 2017: Oxford University Press New York; 2016.
9. Employer Costs for Employee Compensation - December 2022 [press release]. March 17, 2023 2023.
10. York E. Summary of the Latest Federal Income Tax Data, 2023 Update. *Tax Foundation*. 2023. <https://taxfoundation.org/publications/latest-federal-income-tax-data/>. Accessed May 2023.
11. Kogut SJ, Campbell JD, Pearson SD. The Influence of US Drug Price Dynamics on Cost-Effectiveness Analyses of Biologics. *Value Health*. 2023;26(3):378-383.
12. Institute for Clinical and Economic Review. Adapted Value Assessment Methods for High-Impact “Single and Short-Term Therapies” (SSTs). https://icerorg.wpengine.com/wp-content/uploads/2020/10/ICER_SST_FinalAdaptations_111219-1.pdf. Published 2019. Accessed June 2, 2023.
13. Vanness DJ, Lomas J, Ahn H. A health opportunity cost threshold for cost-effectiveness analysis in the United States. *Annals of internal medicine*. 2021;174(1):25-32.
14. Vallejo-Torres L. The Broader Opportunity Costs in the Broader Cost-Effectiveness Analysis Framework. *Applied Health Economics and Health Policy*. 2023:1-12.
15. Institute for Clinical and Economic Review. ICER’s Reference Case for Economic Evaluations: Principles and Rationale https://icer.org/wp-content/uploads/2020/10/ICER_Reference_Case_013120.pdf. Published 2020. Accessed June 2, 2023.
16. Arrow KJ. *Essays in the theory of risk-bearing*. Vol 121: North-Holland Amsterdam; 1974.
17. Analysis. UBoE. Personal Income per Capita [A792RC0A052NBEA]. *In*. 2018;Vol 2019: Federal Reserve Bank of St. Louis.
18. Commission. MHSCR. Maryland's Total Cost of Care Model: Update for Providers.
19. Commission MHP. Health Care Cost Growth Benchmark. 2019.