
2023 Value Assessment Framework

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ICER Speakers



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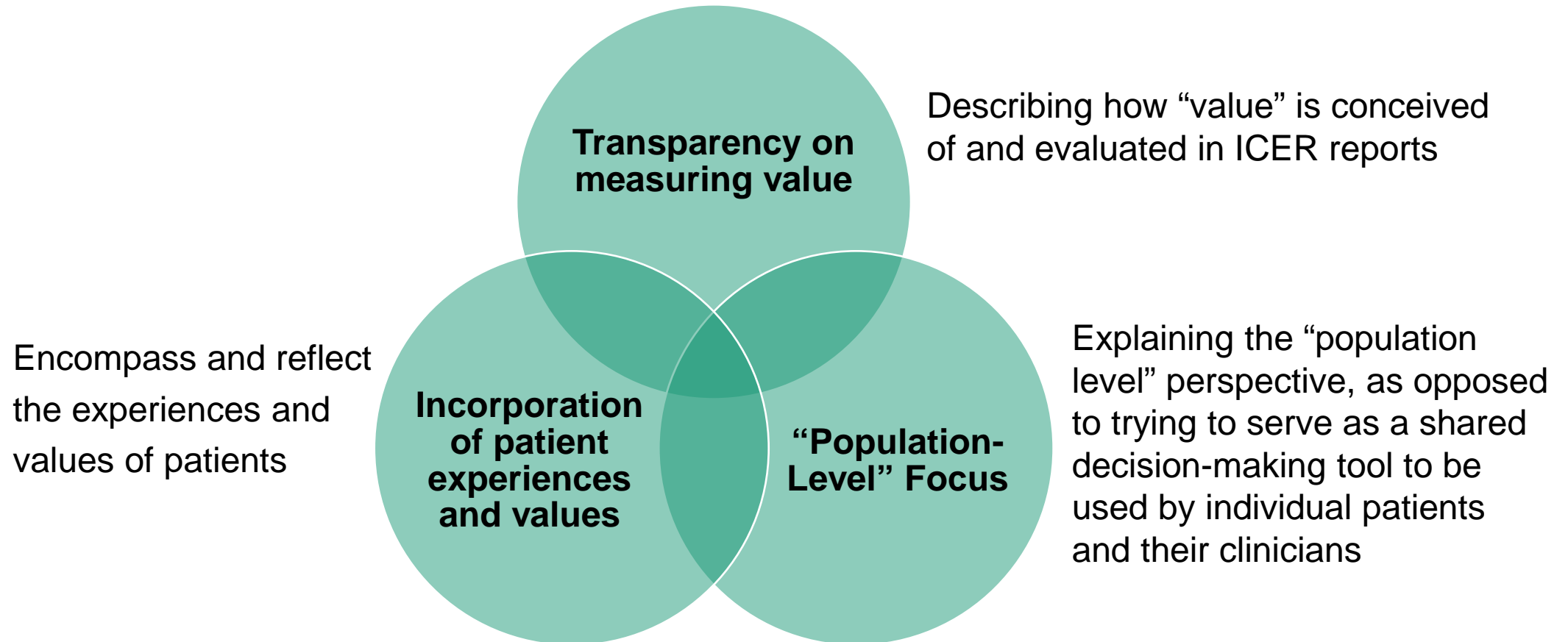


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Agenda for Today



Purpose of the ICER Value Assessment Framework



Experience following the 2020 VAF update:



35 ICER
assessments



56 ICER publications
since 2020



85 new drugs
reviewed



17,000 Final
Evidence Report
downloads



Engaged with over
100 patient groups,
patient reps, and
caregivers



6,000 Report-at-a-
Glance downloads

The 2023 VAF will focus on:

Patient Engagement

- Accessibility and inclusivity
- Share Your Story
- ICER Patient Council

HTA Methods Related to Health Equity

- Analyses of clinical trial diversity and racial/ethnic subpopulations
- Quantitative measures to guide discussion and voting on unmet need

Cost-effectiveness

- “Non-zero” inputs for societal perspective
- Dynamic pricing scenario
- Future methods development on value domains

Focus Areas of 2023 VAF Update

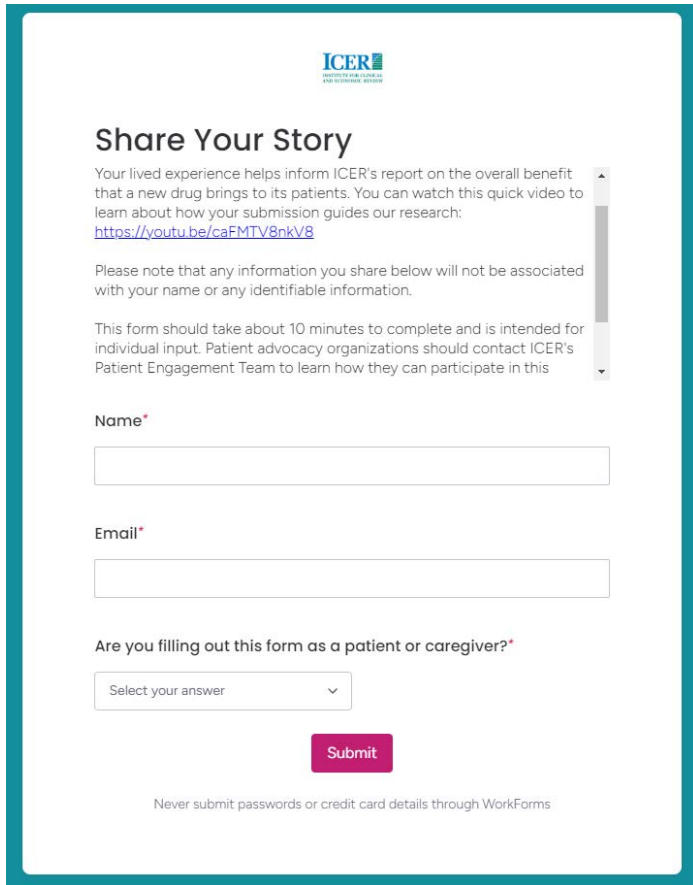
- 1 Patient Engagement
- 2 HTA Methods Related to Health Equity
- 3 Cost-effectiveness

Focus Areas of 2023 VAF Update

1 Patient Engagement

- Share Your Story form
- Small-group patient and caregiver discussions
- Patient compensation
- Accessibility and inclusivity of public meetings
- ICER Patient Council
- Enhanced patient-friendly materials

Share Your Story Form



The screenshot shows the 'Share Your Story' form from ICER. At the top is the ICER logo. Below it is the title 'Share Your Story' followed by a paragraph explaining that the user's experience helps inform ICER's report on the overall benefit of a new drug. A link to a video is provided: <https://youtu.be/caFMTV8nkV8>. A disclaimer states that information shared will not be associated with the user's name or identifiable information. Another note mentions the form should take about 10 minutes and is for individual input, with a contact for patient advocacy organizations. The form includes fields for 'Name*' and 'Email*', a dropdown for 'Are you filling out this form as a patient or caregiver?' with the text 'Select your answer', a 'Submit' button, and a footer note: 'Never submit passwords or credit card details through WorkForms'.

What ICER Wants to Know

- Impact of condition on daily life
- Experience with previous/current treatments
- Experience with accessing/affording care
- Hopes for a new treatment
- Impact on caregivers/family

Small-Group Patient and Caregiver Discussions

- Increase diversity of patient participation during the scoping phase
- 90-minute call with ~5 patient/caregiver participants
- Insights from this call are summarized in our Patient Perspectives chapter

Fair Compensation for Patient Participation

- Formalize honoraria for select activities:
 - Small-group patient and caregiver discussions = \$100
 - Review of draft evidence report = \$500
 - Panelists at public meeting = \$500

Accessibility of Public Meeting

- Virtual attendance and remote testimony
- Closed captioning for all meetings
- If we return to in-person meetings:
 - Still offer hybrid meeting format
 - Travel grants

ICER Patient Council


- Publicly announced in July 2023
- 3 main objectives:
 1. Identify opportunities to improve patient engagement process
 2. Ensure diversity of patient participation
 3. Ensure accessibility and inclusivity of materials
- Council structure and meeting format

Patient-Friendly Resources

How to Share Your Lived Experience with ICER

What is the Scoping Phase?

The scoping phase is the first two months of ICER's research process. During this time, the ICER team is trying to "scope" or figure out what is most important to include in our report about the treatment and patient population we are researching. We do this by speaking to different members of the patient community to better understand the diversity of patient lived experience.



What Happens During this Phase?

- ✓ ICER notifies the patient community about the review
- ✓ ICER offers introductory calls to meet with patient groups
- ✓ ICER offers scoping calls to figure out our research plan
- ✓ ICER publicly announces the review & publishes the Draft Scope
- ✓ ICER asks for public comments on the Draft Scope
- ✓ ICER publishes the Revised Scope

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How to Respond to ICER's Work

Why Does ICER Request Your Input?

Health technology assessment (HTA) is a type of research process that involves all key participants in the health care system. In order for ICER to understand how well a new drug works and how much it could cost, we want to involve the people that this new drug may impact. That's why we invite patients, clinicians, health insurers, and drug makers to provide their perspectives on the new drug.

The patient community is an especially important partner for ICER because they can teach us about the patient lived experience and what matters most to patients when deciding on a new treatment option.



YOUR VOICE MATTERS

Patient community input helps ensure that the ICER report accurately reflects the impact of the condition on patients and caregivers, the benefits and risks of the new drug, as well as any costs related to the condition or treatment. Our hope in highlighting the patient perspective is that decision makers read the ICER report and determine fair pricing and fair coverage for the new drug based on the needs and expectations of the patient community.

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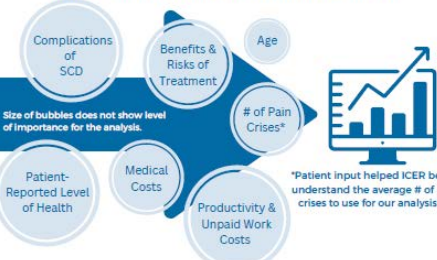
Sickle Cell Disease 2023

How Did ICER Calculate a Fair Price?

Using economic modeling, we calculated the cost effectiveness of lovo-cel and exa-cel based on how well they reduced acute and chronic complications, compared to standard of care. See below for what types of information ICER considered to calculate a fair price range for the two gene therapies.

Population
This analysis included adolescents and adults with severe SCD who were not eligible to get a stem cell transplant.

Factors Included in ICER's Economic Analysis



Size of bubbles does not show level of importance for the analysis.

*Patient input helped ICER better understand the average # of pain crises to use for our analysis.

Fair Price Range for Lovo-Cel and Exa-Cel

\$1.35 - \$2.05 MILLION

A fair price is how much a treatment should cost based on how well it works for patients. Our economic analysis concluded that the fair-price range for lovo-cel and exa-cel is between \$1.35 and \$2.05 million. This price is for a one-time treatment.

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Summary of ICER's Health Equity White Paper

Why Should We Support Health Equity in Health Technology Assessment?

Health technology assessment (HTA) is one way of creating a health system that is fair to everyone. Therefore, it is important to make sure that the process and methods of HTA follow best practices for achieving the goal of health equity.

What is HTA?

HTA is a way to measure how well a treatment works. Knowing how well a treatment works can help people who make decisions about drug pricing and health insurance coverage policies.



What is Health Equity?

Health equity is the idea that all people should have an equal opportunity to better health without any barriers related to race, ethnicity, gender identity, preferred language, where they live, or other important factors.



Objective of White Paper

To give recommendations for HTA that will help societies improve health equity for racial, ethnic, and socially disadvantaged groups.

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Focus Areas of 2023 VAF Update

- 1 Patient Engagement
- 2 HTA Methods Related to Health Equity
- 3 Cost-effectiveness analysis

Focus Areas of 2023 VAF Update

2 HTA Methods Related to Health Equity

- Clinical trial diversity
- Subpopulation analysis
- Patient input on clinical trial design
- Quantitative measures to guide discussion and voting on unmet need

Clinical Trial Diversity

- To promote conversations around equity in clinical trials of new drugs
- ICER-developed rating tool to evaluate Race/Ethnicity, Sex, and Age
 - Benchmark: Prevalence of the disease condition
 - Rating Categories: 'Good', 'Fair', 'Poor'
 - Multinational Trials: Rate US subpopulation

Subpopulation Analysis

- Race, sex, and age will be considered as presumptive subpopulations
- Targeted literature review and consultation with patients, clinical experts, and manufacturers to determine relevant subpopulations
- A formal credibility assessment tool to evaluate and present information on the credibility of subgroup findings

Patient Input on Clinical Trial Design

- To highlight industry-leading practices in incorporating patient perspective into their drug development programs
- Manufacturers are encouraged to describe methods used to collect patient experience and how they identified the outcomes most important to patients

Focus Areas of 2023 VAF Update

2 HTA Methods Related to Health Equity

- Enhancing methods to capture and integrate “benefits beyond health” and “special ethical priorities”

Benefits Beyond Health and Special Ethical Priorities

- Change terminology away from “contextual considerations”
- Acknowledge and incorporate broader elements related to value that may or may not be quantifiable
- Improve capture of impact on health equity
- Improve consistency and transparency of deliberation and voting

Benefits Beyond Health and Special Ethical Priorities

- Move to 1-5 Likert scale voting of agreement with statements regarding the following elements that are important in making judgments regarding the value for money of new health care interventions:
 - *Unmet need with current best treatments (informed by health shortfall)*
 - *Relevance of the condition for disadvantaged communities (informed by HIDI)*
 - Impact on caregivers' lives
 - Mechanisms of action or of delivery that could improve access

**1. Consider Comparative
Clinical Effectiveness and
Cost-effectiveness Range**

**2. Consider Benefits Beyond
Health and
Special Ethical Priorities**

**Price to reach
\$100k/evLYG**

**Price to reach
\$150k/evLYG**

Focus Areas of 2023 VAF Update

- 1 Incorporating the patient voice
- 2 HTA Methods Related to Health Equity
- 3 Cost-effectiveness

Focus Areas of 2023 VAF Update

3 Cost-effectiveness

- “Non-zero” inputs for impact of treatment on productivity
- “Dynamic price” scenario
- Process to evaluate potential application of the Generalized Risk-Adjusted Cost-Effectiveness (GRACE) framework

“Non-zero” inputs for impact of treatment on productivity

- Capturing additional dimensions of impact of treatment from the “societal” perspective often challenged by lack of data
- Most accepted component of societal perspective is productivity, although ethical concerns remain important
- ICER will adopt a relatively new academic approach to crosswalk productivity gains for patients and caregivers from quality of life gains resulting from treatment

“Dynamic price” scenario

- Longstanding debate over whether CEA should try to estimate price increases and/or decreases over the lifespan of a new treatment
- Current adoption of dynamic pricing very limited
- Adoption of IRA has increased consideration of relevance of price decreases due to generic competition and/or negotiation
- Methodological and conceptual challenges remain; ICER will take time to develop approach, potentially involving pilots of dynamic pricing

Evaluating the GRACE framework

- Methods for modifying the measure of health gain (QALY or evLYG) to reflect severity are controversial and vary across academics and HTA groups
- GRACE applies measures of risk-aversion and values of health gains at different levels of severity to modify the QALY
- ICER will work with academics and HTA groups to examine strengths and limitations of GRACE, including conceptual and practical validity, potential impact on discriminatory risk, and “fairness” across patient groups

Reflections on the Value Assessment Framework

- We use the VAF as our “guidebook” for every single ICER assessment
- We want everyone to understand how we approach value assessment to enable participation at all levels
- We value your engagement and support, and we could not do this effectively without your constructive feedback

