Patient Participation Guide

Updated October 23, 2020

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1. Introduction

This guide was created to help you tell us what we should know about the patient experience. Often, patient experiences are not captured in research or other evidence. That is why we need to know what is important to you and your community. In this guide, you will see the various stages in our process, learn where we need your input most, and receive guidance on the types of input that can be the most influential at each stage.

What is ICER?

ICER, the Institute for Clinical and Economic Review, is a non-profit research organization that evaluates evidence on the value of medical tests, treatments, and delivery system innovations. ICER was founded more than 10 years ago with an ethical goal in mind: to try to provide a fair and objective analysis of evidence as the starting point for bringing all stakeholders—patients, doctors, drug makers, insurers, and others—together to seek better ways to help patients gain sustainable access to high-value care. We want to help create a health care system in which innovation is rewarded fairly, but without overpaying, so that all patients have the opportunity to access a choice of treatments and improve their health at a price they can afford.

What Does ICER Do?

ICER produces several evidence reports every year, each focused on a different topic. Each report includes:

- Information on the lived experience and treatment perspectives that can broaden our understanding of the value of different interventions.
- A review of evidence on how well each drug or other type of intervention works and how the risks and benefits compare with other care options.
- An analysis of how the long- and short-term costs of the intervention line up with the added benefits for patients.

Each report is developed with input from multiple sources, including patient organizations, clinical experts, and manufacturers. When a final draft is completed, each report is the subject of a public meeting, during which an independent expert committee, convened by ICER, discusses the report and votes on the evidence and testimony presented. These three deliberative committees are:

- The California Technology Assessment Forum (CTAF)
- The Midwest Comparative Effectiveness Public Advisory Council (Midwest CEPAC)
- The New England Comparative Effectiveness Public Advisory Council (New England CEPAC)

ICER Listens to Patients

ICER values input from all stakeholders, especially individuals affected directly by a condition and patient organizations. Patients are at the core of ICER's mission to help provide an independent source of analysis of evidence on effectiveness and value to improve the quality of care that people receive. With input from patients, our reports are able to provide a deeper sense of how a condition affects day-to-day life, the financial and insurance challenges people face in accessing their treatment, information about what outcomes or side effects are of most importance to patients, and much more.

We know our reports are better and more useful for everyone when they include the real-world patient perspective. But we also know that patients can be concerned about contributing to a process whose result they cannot necessarily predict. We make sure that all patients and patient organizations know that contributing information, insights or advice to ICER is not an endorsement of our work.

Some examples of how we have incorporated patient perspectives include:

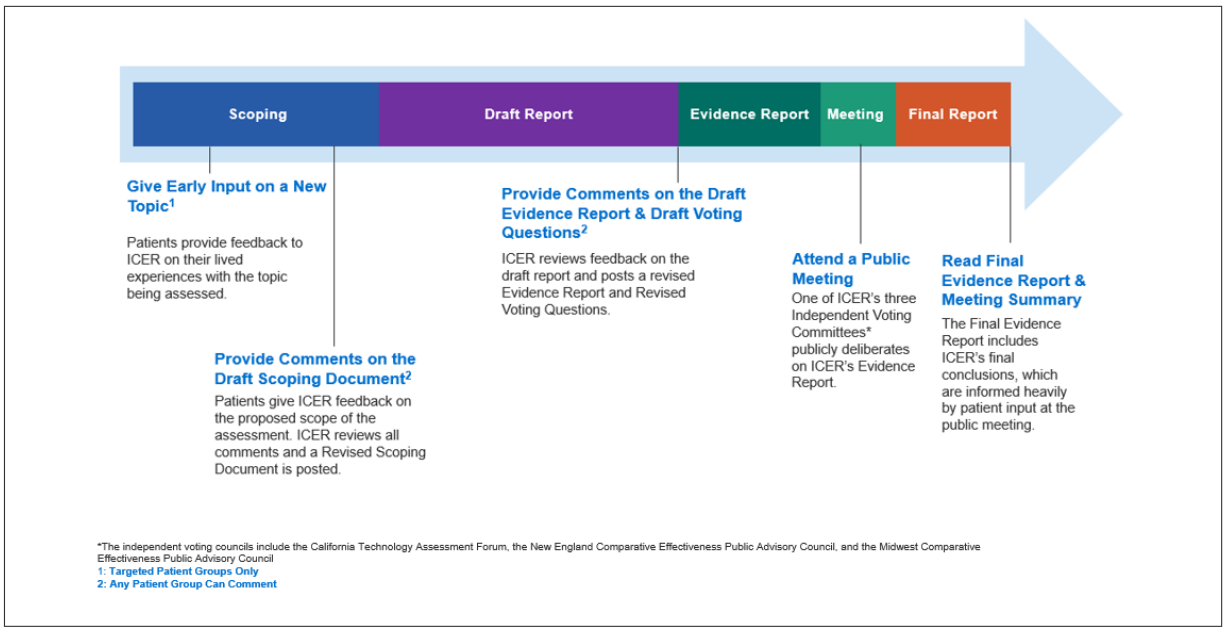
- The sickle cell disease patient community contributed extensive information and results from a patient survey to highlight what patients need in treatments as well as the significant challenges people living with sickle cell disease face due to discrimination. Highlighting these concerns in the ICER Report gave insurers a much better understanding of the lived experience and lead to the creation of exceptions to reduce barriers for opioid pain management for people with sickle cell disease. The report also called for further investment into innovative therapies for a patient community that had been underserved for far too long.
- Patients with psoriasis highlighted how significantly it affected their quality of life at work and school, and pointed to step therapy policies and insurance coverage as a key barrier to appropriate treatment. Our final policy implications reflected these concerns.
- Patient groups we spoke to about our review of abuse-deterrent opioid formulations highlighted the need to balance patients' needs for pain control with the risks of opioid misuse. This concern informed the context of our report.
- For a review of treatments for atopic dermatitis, patients highlighted added burdens of the disease such as sleep disruption that are not well-documented in typical clinical trials.
- We worked with a multiple sclerosis patient group to field a new survey to gather information on aspects of treatments that patients found most important to them. Data on responses from over 18,000 patients was included in our report and was an important complement to clinical data that was narrowly focused on relapse rates.

In a press release, the National Psoriasis Foundation's CEO commented on their work with ICER throughout the report process:

"The National Psoriasis Foundation is very pleased with the final recommendations and in the significant role we have played to provide input which informed the final report. The final report accurately reflects the challenges of living with psoriatic disease and it recommends insurers expand the tools physicians have available to care for patients managing this complex disease over a lifetime," said NPF CEO Randy Beranek.

How You Can Participate in ICER's Process

The overall report development process for a standard review takes approximately eight months between topic selection and the final revision to the report following the public meeting. Class reviews, in which we examine a larger set of treatments, take approximately 10 months. Below is a figure that summarizes key points during standard reviews where all stakeholders, including patient groups, can contribute to report development. Later pages provide additional information on each step.



2. Give Input on a New Topic (Weeks 1-5)

As part of ICER's Patient Engagement Program, ICER provides early notifications to patient groups from therapeutic areas when it has high certainty that it will pursue an assessment pertaining to their focus. Topics are chosen based on several factors, including input from the public. Learn more about how we choose topics, or submit a suggestion for a future review. In this initial outreach, we provide an overview of ICER procedures, answer your questions about ICER, and, if needed, can facilitate connections with other patient leaders who have participated in an ICER review. For major therapeutic classes (i.e., immunomodulators for which ICER has performed a class review, treatments for multiple sclerosis, etc.), ICER may schedule an annual conference call or meeting to discuss the emerging pipeline of new treatments, seek input on key priorities, and explore opportunities to gather new data on outcomes of care that are important to patients and families. This early outreach is intended to give patient organizations a chance to familiarize themselves with ICER and our processes ahead of ICER's final selection of a topic.

Once ICER has selected a new topic for review, we spend five weeks engaging with targeted stakeholders from that therapeutic area prior to announcing our review to the public. During this five-week period, ICER schedules scoping calls with patient groups to get their initial input on how we should approach our review and to learn about the lived experiences of the patient community. At the end of week five, we publicly announce the topic and post the draft scoping document for public comment. This provides the stakeholders with whom we've already spoken, as well as additional stakeholders, an opportunity to provide public input to inform ICER's proposed research agenda.

What Happens During This Step?

- After topic selection, ICER spends five weeks reaching out to targeted stakeholder groups, including patient organizations, to discuss their perspectives on the disease and treatments.
- While ICER is speaking with patient groups and other stakeholders, ICER begins to develop a Draft Scoping Document. The Draft Scope describes what evidence ICER will look for during the review and how it plans to analyze the evidence.
- ICER posts the draft scoping document for a three-week public comment period, which provides all stakeholder an opportunity to provide feedback on our proposed research approach.

What Information is Most Helpful?

At this stage, any information is helpful. We want to learn as much as possible from all stakeholders about the topic. Some examples include:

- Clinical benefits that matter most to patients
- Other non-clinical benefits or disadvantages of new and existing treatment options
- Contextual considerations surrounding the disease and its treatment (i.e., high lifetime burden of illness, uncertainty about long-term risks of treatment, etc.)
- Research publications or patient-generated data to supplement clinical trial data

How Do I Give Input?

- ICER will contact selected patient groups directly to set up scoping calls, and we also accept written feedback, information, insights, and patient testimonials from these groups during the first three weeks of this period.

How Does ICER Use My Input?

- ICER uses the information to help guide the scoping process and inform our initial research plan. More specifically, much of the input we receive is ultimately translated into what we call "PICOTS." PICOTS stands for **P**opulations (people), **I**nterventions (treatments of interest), **C**omparators (other available treatments), **O**utcomes (benefits and harms), **T**iming (length of study), and **S**ettings (where care is given). For example, if we hear from patients with rheumatoid arthritis that the treatment under review provides relief of morning joint stiffness, we will then look for specific data that reflects this "outcome," or clinical benefit.
- In addition, patient input may also provide information about aspects of treatment or the disease that may not be described or captured in available clinical or economic data.
- All submissions are considered throughout the scoping process and are incorporated into the Draft or Revised Scoping Document, depending on when the input was received. Information received towards the end of the Draft Scope Period may not be considered for the Draft Scoping Document, but will be taken into account in the revised version.
- Information submitted during this period provides useful background throughout the report development process.

ICER does not publicly name individual patients who speak with us on these or other calls. Additional details can be found in the disclaimer language below.

You acknowledge that you are not required to provide information to ICER and that you are doing so voluntarily. Please see our Terms of Use and Conditions and our Privacy Policy to review how we use and disclose information submitted to us. While we do not plan to publish information that identifies a particular individual, we intend to use the information submitted as part of our drug reviews. We recognize that submissions sometimes contain specific medical information that might raise concerns about appropriateness of treatment, physical or mental health, and safety. The use of this site, and information submitted to ICER, should not take the place of professional medical

care. ICER does not diagnose health problems or provide treatment advice. The completion of this questionnaire does not trigger any patient-provider relationship. Any information found on this site, or inferred from this survey, should in no way be considered medical advice or a plan for health management. Anyone seeking or needing immediate medical treatment is advised to contact their health care provider or visit an emergency room.

3. Comment on the Draft Scoping Document (Weeks 6-8)

ICER publicly announces the topic at the beginning of this phase and posts a Draft Scoping Document for public comment. The Draft Scoping Document outlines the plan of research, drugs or treatments to review, patient populations to include, and other related information. The document is informed by our own independent research and conversations with clinical experts, manufacturers, policy makers, insurers, and patient groups. Input from patient organizations and individual patients help inform specific components of the Draft Scoping Document, including our PICOTS framework. As we note in the section above, we encourage input on the specifics included in the Draft Scoping Document.

When ICER publicly announces a review, we send an email to major patient groups and all ICER mailing lists to make sure that stakeholders are aware of our review. At this time ICER also publicly posts the timeline for the project on the ICER website. This timeline provides dates for key milestones in the process.

What Happens During This Step?

- The Draft Scoping Document is posted to the website and announced by email.
- The document is open to a three-week public comment period.

What Information is Most Helpful?

- During this stage, we're largely looking for feedback directly related to the content of our Draft Scoping Document, as patient input will help us revise and clarify before we release our Revised Scoping Document. This could mean input on what is included or left out of the Draft Scoping Document that is most important to patients (e.g., patient subgroups, treatments, or additional benefits to examine).
- ICER will also accept responses to the Patient Input Questionnaire. These responses can be sent to ICER any time before the public comment period on the Draft Report ends (see the next chapter for more details).
- Any additional patient-related resources
- Which clinical trial data are most related to patient-centered outcomes

How Do I Give Input?

- Submit public comments on the Draft Scoping Document
    - ICER accepts comments on the Draft Scoping Document for three weeks.
    - The deadline is listed in the posting announcement and on the website.
    - Email comments to publiccomments@icer-review.org.
    - Emailed public comments on the draft scope must be in the following format[1]:
        - Submit as an attached Word document
        - Three page maximum (not including references or appendices)
        - 12-point Times New Roman font
- Submit a response to our patient input questionnaire
    - Individual patients or patient groups are encouraged to submit a response through the Patient Input Questionnaire during the public comment period on the draft scope. Please note that this is not a validated survey tool and we do not aggregate responses into data points. ICER reviews questionnaire responses throughout the assessment to reflect on what patients have told us. As a result, patients may submit responses anytime during the process, though having this information early in our process helps us incorporate patient perspectives into our early thinking.

How Does ICER Use My Input?

- ICER reviews all comments received and considers what changes need to be made to the scope before work on the report begins. For example, ICER might include an additional patient subgroup, comparator, or outcome measure. We refer to components like these as PICOTS in our scoping documents (please see the previous chapter for further information on PICOTS).
- Input received during the scoping phase informs ICER's selection of outcomes measures to include and prioritize in our clinical and economic assessments. Further, public comments and conversations with patient groups inform the second section of our reports, "Patient Perspectives," which precedes the sections describing the clinical and economic evidence.
- Written comments, including those submitted by individual patients, will be posted publicly to the ICER website with the Evidence Report.

[1] If formatting requirements present a burden to any patient, accommodations can be made on a case-by-case basis. Please email publiccomments@icer-review.org or call (617) 528-4013 for more information.

Revised Scoping Document

After public comments close, ICER updates the scoping document based on comments and releases a Revised Scoping Document. This document also includes a brief summary of additional information learned through stakeholder outreach and public comment as well as ICER's rationale behind which major suggestions we did and did not incorporate. The document is posted to the ICER website, along with comments received on the draft version.

4. Comment on the Draft Evidence Report and Voting Questions (Weeks 24-27)

The Draft Evidence Report includes background and context about the disease and its treatment, patient and caregiver perspectives that informed the draft, reviews evidence, and provides preliminary cost-effectiveness analyses. It reflects only ICER's independent analysis of currently available information and does not discuss policy considerations or make policy recommendations. The Draft Evidence Report and Draft Voting Questions are posted on the ICER website, and an announcement is sent to ICER's mailing list.

What Happens During This Step?

- Based on the topic, ICER and patient groups may partner to conduct a formal survey to gather patient preferences for treatment. The results of these surveys may inform our economic model, especially when important information is missing from the clinical evidence, and/or the discussion of patient perspectives.
- Before we publish the Draft Evidence Report, we send it to external reviewers to make sure that the document is accurate and thorough. We typically invite several clinicians to review the clinical and economic evidence, and ask a representative from a patient organization to review the sections describing the patient experience.
- The Draft Evidence Report is posted on the website and announced by email.
- Stakeholders review and provide comment over a four-week period (five weeks for large reviews of entire treatment classes).
- This is also the time to request to make an oral comment at the public meeting.

What Information is Most Helpful?

- Suggestions for additional data, including real world evidence
- Added details for context
- More explanation of additional benefits or disadvantages of a treatment
- Anything else that, from a patient perspective, the report misses. We encourage patients to comment on Section 2, "Patient Perspectives," as well as Section 6, "Potential Other Benefits or Disadvantages and Contextual Considerations."

How Do I Give Input?

- The specific deadline is in the announcement and is posted on ICER's website.
- Email comments to publiccomments@icer-review.org. We will confirm receipt.
- Comments must be in the following format[2]:
    - Submit as an attached Word document
    - Five page maximum (not including references or appendices)
    - 12-point Times New Roman font

How Does ICER Use My Input?

- ICER carefully considers all comments relevant to every section of the report.
- Based on specific feedback we receive on the patient-centered sections, Sections 2 and 6, we may revise to include additional factors or considerations important to patients (i.e., caregiver impact, comorbidities, etc.). In addition, comments related to the financial burden of a disease or treatment may also be helpful, such as specific information about out-of-pocket costs or other medical expenses.
- We also welcome patient input on other sections of the report, including the clinical end economic analyses. We encourage patients to provide as much or as little feedback as they wish. ICER considers all patient input important, no matter how much is provided.
- All changes are reflected in an updated Evidence Report that is published before the public meeting takes place.
- Written comments, including those submitted by individual patients, will be posted publicly to the ICER website with the Evidence Report and will be distributed to members of the relevant voting body prior to the public meeting.
- A document with ICER's response to specific suggestions will also be posted.

Register for Oral Comment at the Public Meeting

This is also the period to request to speak at the public meeting. Patient comments provide additional important context and help inform discussion during the panel vote and policy roundtable. Each meeting includes up to 45 minutes for oral comment before the voting panel takes its votes. Individual comments are limited to five minutes. To request to speak, please follow the below steps:

1) Send request to ICER via email publiccomments@icer-review.org including the name, title, and organization (if applicable) of the proposed speaker.

[2] If formatting requirements present a burden to any patient, accommodations can be made on a case-by-case basis. Please email publiccomments@icer-review.org or call (617) 528-4013 for more information.

2) ICER staff will confirm receipt of the request, and reply with a link to an online conflict of interest form that must be filled out accurately to complete registration.
3) Any reported conflicts of interest will be disclosed at the meeting but will not prevent participation.

Since there may be more requests than can be accommodated during the meeting, and to help provide the opportunity for a broad range of perspectives to be heard, public comment slots will only be confirmed after the deadline for requests has passed. Priority for public comment slots will be given to patients with the condition under study and subject-matter experts from the patient advocacy, clinical, and research communities.

Public commenters may not use a slide presentation or distribute materials to the panel or audience members prior to, or during, the public meeting.

Evidence Report

The Evidence Report includes changes made based on public comments and includes health benefit price benchmarks. This is the price for a treatment that matches with the benefits to patients that have been shown in the evidence. It is posted to ICER's website, and an announcement is emailed to subscribers. Along with the report, a revised set of voting questions, all public comments received, and ICER's response to comments are posted to the website. This version of the report is shared with the voting panel in advance of the public meeting.

5. Attend a Public Meeting (Week 32)

What Happens During This Step?

- Report authors give an overview of report findings.
- Pre-registered speakers give oral public comments.
- The evidence review group votes on the strength of evidence for the topic under review. Report authors, clinical experts, and patient representatives are available to answer questions.
- A policy roundtable, composed of patients, doctors, insurers, and drugmakers convenes to discuss how to apply the evidence to real-world policy and practice.

How Can I Participate?

- **Attend:** Meetings are free and open to the public. We recommend registering in advance to ensure your spot. Registration typically opens when the Draft Evidence Report is posted.
- **Comment:** Register to give an oral public comment using the instructions above.
- **Watch:** A live webcast is offered for all meetings. ICER also posts a recording of the webcast on their website within a week after the meeting.
- **Be a member of the policy roundtable:** One to two patient representatives are invited to participate in the policy roundtable, typically alongside one or two each of clinical experts, payers, and drug manufacturers.

How Does ICER Use My Input?

- ICER uses public meeting discussions to inform the policy implications that are included in the Final Evidence Report and Meeting Summary.
- Public commenters are invited to submit a 750-word summary of their oral comment to be included in the Final Evidence Report and Meeting Summary.

Final Evidence Report and Meeting Summary

The Final Evidence Report and Meeting Summary includes a summary of discussions, public comments, voting results, and policy statements from the roundtable discussion during the meeting. It takes about one to two weeks to publish the Final Evidence Report and Meeting Summary. The report is posted to the ICER website and announced by email.

A short, summarized version of the report, called “Report-At-A-Glance,” is posted alongside the full Final Evidence Report and Meeting Summary.

6. Additional Information

We hope this guide gave you a good overview of our process and how you can get involved. Of course, there is more to our mission than just what's here. Visit our website for more information.

Here are some helpful links:

- To find out more about how we look at the effectiveness and value of drugs, tests, or treatments, visit the How We Do Our Work page.
- To learn how organizations around the world conduct similar assessments, read this Guide to Understanding Health Technology Assessment.

Contact Information: Still have questions, or suggestions for improvements to this guide? Contact us at info@icer-review.org. We look forward to hearing from you!