WHAT'S ICER MISSING? THE RARE PATIENT VOICE.

Why the rare patient experience needs to be at the forefront of ICER's value assessment methodology when reviewing rare disease treatments.

Patient Advocate Toolkit
ICER PNH Review

ABOUT ICER

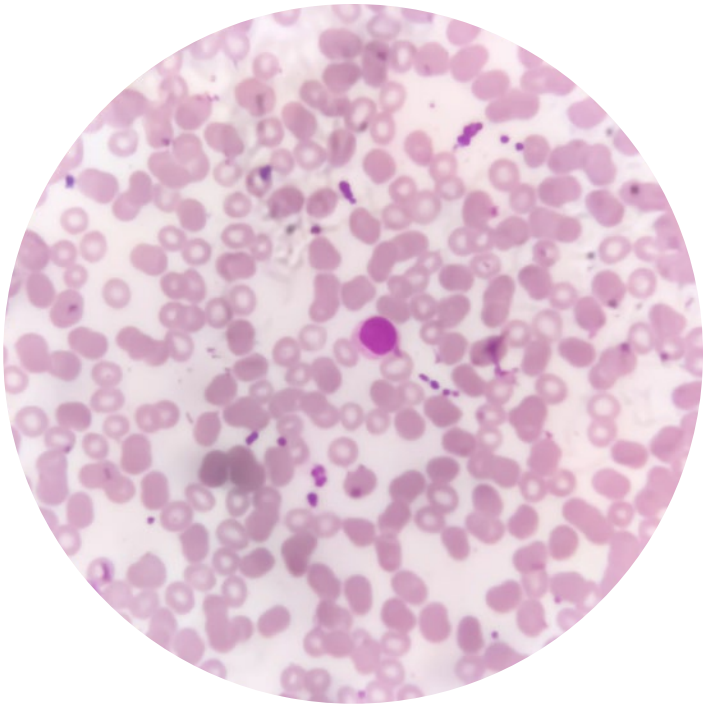
Its Goals and Approach to Treatment Access

- **History:** The Institute for Clinical and Economic Review, or ICER, is a private entity that creates cost analysis reports that can be used by insurance companies to deny patients access to innovative medical treatments. They use the Quality Adjusted Life Years (QALY) metric in their value assessments, which is inherently discriminatory to patients, as it values the life of sick patients as less than those of a healthy person. There is currently federal legislation to ban the use of this metric in all government programs.
- **Goals.** ICER's stated goals are to improve access and affordability while retaining incentives necessary for future innovation; however, their assessments lack a true understanding of the patient experience.
- **Value framework and methodologies.** Value frameworks represent tools that may be used to measure the value of medications and other health technologies to determine potential impacts on the healthcare system. In an effort to focus on the healthcare system as a whole, the patient experience is often sidelined and provides no real weight to the final report outcomes.
- **Rare disease considerations.** ICER's current QALY-based value framework is insufficient for rare disease treatments. When using QALYs in value assessments for rare diseases, it's essential to account for data limitations, patient perspectives, heterogeneity, and ethical considerations.
- **Patient voice.** Rare disease patients and their caregivers often have unique perspectives and priorities when it comes to treatment outcomes. It's crucial to involve patients and gather patient-reported outcomes to ensure the calculations and outcomes reflect their perspectives.

ICER's PNH Review

ICER is currently in the process of reviewing Paroxysmal Nocturnal Hemoglobinuria (PNH), which is a rare blood disease that causes red blood cells to break apart prematurely and can lead to life-threatening complications. PNH is a rare disorder with roughly 500 people diagnosed in the United States each year. It is estimated to affect between 5,000 and 6,000 people in the United States. PNH is not hereditary and can affect people of any race or gender, but it is most commonly diagnosed in people in their thirties and forties.

Whether you or someone you love has PNH or not, we believe rare disease patients supporting each other during ICER reviews is vital to ensure that the unique challenges and needs of this population are considered in assessments of treatment value. Their collective advocacy can contribute to more informed, compassionate and equitable healthcare decisions for individuals with rare diseases.



Here are some key considerations:

1. **Limited patient populations.** Rare diseases often affect a very small number of individuals, making it challenging to generate robust clinical and economic data for these conditions. When rare disease patients support each other during ICER reviews, they can collectively provide a more comprehensive and representative picture of the challenges faced by rare disease populations as a whole.

2. **Advocacy for access.** ICER reviews can influence treatment access and reimbursement decisions. Rare disease patients advocating for their needs and sharing their stories can raise awareness about the importance of these treatments and the impact they have on their quality of life. This advocacy can be instrumental in ensuring continued access to effective therapies.

3. **Ethical considerations.** Patient involvement in ICER reviews can highlight the ethical considerations surrounding access to treatments, especially when cost per QALY assessments may not fully capture the value of these therapies in improving patients lives.

4. **Policy influence.** Rare disease patient advocacy can extend beyond individual ICER reviews and influence broader healthcare policies related to orphan drug development, pricing, and reimbursement. A united patient voice can drive policy changes that benefit the entire rare disease community.

ICER PNH REVIEW TIMELINE

Overview of ICER's assessment of treatments of PNH

The Institute for Clinical and Economic Review (ICER) will assess the comparative clinical effectiveness of two proximal complement inhibitors for the treatment of paroxysmal nocturnal hemoglobinuria (PNH).

The assessment will be publicly discussed during a meeting of the California Technology Assessment Forum (CTAF) on February 16, 2024, where the independent evidence review panel will deliberate and vote on evidence presented in ICER's report.

November 28, 2023

Patients Rising Educational Webinar

Prior to the release of the draft evidence report, Patients Rising will host a webinar to educate the PNH and larger rare disease community on the ICER process for this review of drugs to treat PNH. You will hear from experts on the ICER process and ways you can ensure patients' experience and preferences are included in ICER's decision making process.

November 2023

December 5, 2023 – January 9, 2024

Public Comment Period

ICER's Draft Evidence Report will be released on December 5, 2023. With that begins a one-month comment period where **advocates and patients can submit their comments** to ICER. Patients Rising will release our thoughts on the Draft Evidence Report on Wednesday, December 13th. ICER welcomes comments from all interested stakeholders. They can be made on paper or at the virtual meeting of the expert committee to be held on February 16, 2023. The deadline for submission of written comments is January 9, 2024; requests to speak for five minutes—again, virtually—at the February 16th meeting of the California Technology Assessment Committee, the arm of ICER that is conducting the review, is January 9, 2024. All submissions and comments are public.

February 1, 2024

Evidence Report Released

ICER will release the final evidence report to be used at the public meeting on February 16. Patients Rising will release our thoughts on this report on February 6th.

March 14, 2024

Final Evidence Report

ICER's final evidence report plays a significant role in shaping discussions and decisions related to the use and access to healthcare treatments and technologies. This is why it is important for patients to engage throughout the process, and once the report comes out, to stay engaged on the pros and cons of the final analysis.

March 2024

February 16, 2024

ICER Public Meeting

This full-day virtual event includes ICER's presentation on the value of PNH treatments along with personal testimonies from patients and others. The ICER meeting is held in a virtual public forum. This day-long event includes presentations by experts and public comment opportunities for patients and other stakeholders. At the end of the day their selected panel (which generally do not have any experience or expertise in the disease being reviewed) votes on the key questions outlined in the evidence report. All voting outcomes are recorded and made public.

March 21, 2024

Patients Rising will release our analysis of ICER's final evidence report

Patients can use this analysis along with ICER's final report as a resource to educate payers and other stakeholders about their perspectives and experiences, especially when those perspectives may be missing or underrepresented in the report. By using these tools as a starting point and supplementing with patient stories, data, and engagement efforts, patients can effectively educate payers and decision-makers about the aspects of their condition and treatment that may be missing from the report. This collaborative approach can lead to more patient-centered healthcare decisions.

ICER's full timeline and materials can be found here.

RAISE YOUR VOICE

We encourage the rare disease patient community to unite to advocate their needs and concerns during the ICER review process and beyond. Their collective voice can have significant impact.

Ways to Participate

1. **Get educated.** Register to attend the Patients Rising educational webinar on Wednesday, November 28th!
2. **Make your views known to ICER.** If you are inclined to public speaking, request to speak for five minutes at the virtual meeting on February 16th!
3. **Submit your comments.** Express your views on ICER's draft assessment by 5:00pm EST on January 9, 2024.
4. **Attend the public meeting.** Register here to join the meeting.
5. **Encourage others to participate.** Tell others in your network about the ways they can become involved.
6. **Share this toolkit.** Encourage others to become educated about ICER and why the PNH review is important to all rare disease patients.
7. **Raise your voice on social media.** Educate your social media network about ICER and the implications of the ICER review. Encourage them to like and share your posts.
8. **Engage with payers, policymakers, and employers.** Learn how to discuss ICER's report with those determining access to treatment. Take Patients Rising's Advocacy Masterclass. Join our Patient Senate and help represent your state and disease areas.

SOCIAL MEDIA ACTION

Hashtags

#PNHDisease

#PNHAdvocates

#RareDisease

#ICERWatch

#ICER-PNH

#PatientAccess

Twitter Tags

@PatientsRising

@icer_review

General Messages

ICER is assessing the value and effectiveness of new treatments for #PNHDisease. Calling all #PNHAdvocates and #RareAdvocates to learn more and enage in the process. #RareDisease #ICERWatch #PatientAccess

Share your PNH story with @icer_review. Patient perspectives and insights are key to access to treatment for people with #PNHDisease and all #RareDisease patients.

Core Patient-Driven Messages

By educating yourself and your community about ICER reports and other value assessments, we can make a difference. Through personal and community experiences, urge ICER (and all payers considering access to treatments) to incorporate what patients value in their decisions. Here are some key messages to consider when talking about this challenge:

ICER's QALY-based approach is unsuitable for rare disease

There is currently a "QALY-ban" bill in the House of Representatives, **H.R. 485 – The Protecting Healthcare for All Patients Act**. Patients Rising along with more than 75 patient organizations signed onto a letter in support of this legislation. There is no reason ICER should use this metric when so many patient advocates are against this arbitrary measure.

ICER's approach is impractical for rare diseases

ICER's methodology for small patient populations makes it impossible to capture meaningful data and insights that would lead to informed decisions about access

ICER does not capture true patient value in its analysis

ICER does like to hear from and listen to patient groups but falls short of weighing this information in their final reports. Well accepted methodologies that put patients at the center such as patient reported outcomes should be a part of every ICER assessment.

ICER reports are often done before FDA's approval of a new treatment

ICER reports issued before FDA decisions often lack all the evidence needed to assess the value of a new treatment.

ICER does not consider caregiver burden

ICER reviews do not give adequate consideration of caregiver burden in the assessment. There is economic impact including lost productivity and increased healthcare costs for caregivers that should be included in all value assessments.

ICER's framework increases healthcare inequities

ICER's assessment may inadvertently promote health inequities by undervaluing treatment benefits like side effects and quality of life improvements for patients and their families.

RESOURCES

- ICER Draft Evidence Report for Paroxysmal Nocturnal Hemoglobinuria (PNH)
- ICER requirements to submit a public coment
- Registration for ICER Public Meeting on PNH
- ICER Education Center

Additional Resources from Patients Rising

- HTA Principles for Rare Disease Patients
- The Patient Helpline
- The Patient Learning Center: About PNH & Patient Challenges

About Patients Rising

Patients Rising is a national patient advocacy organization raising patient voices to provide education, resources, and advocacy for people living with chronic and life-threatening illnesses.

PATIENTS RISING

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