PATIENT ACCESS & AFFORDABILITY PROJECT

Empowering Patient Perspective.

Elevating Patient Voices in Ultra Rare Disease: Navigating ICER Reviews

Meet Our Panelists

Rick Chapman, PhD
Chief Science Officer |
Innovation and Value Initiative

Darius Lakdawalla, PhD
Director of Research at Schaeffer Center for Health Policy and Economics at USC
and Co-Founder, Chief Scientific Officer | entityrisk

Who is ICER?

The Institute for Clinical and Economic Review (ICER) is an **unregulated, independent, non-profit organization dedicated to evaluating the clinical and economic value of healthcare interventions in the United States.**

Their assessments aim to inform healthcare decision-makers, including insurers, policymakers, and healthcare providers through their own research protocol and model analysis plan.

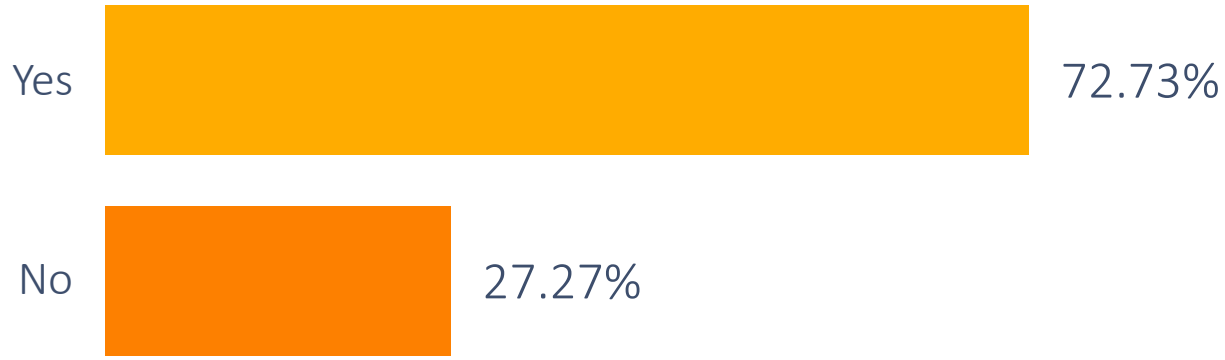
They were founded in 2006 as a research program at Harvard Medical School. However, it was not until 2016 when they received almost $20 million dollars from the John Arnold Foundation to support their assessment work that they started to gain influence.

Challenges for the Ultra-Rare Disease Community.

- ICER Thresholds are Not Appropriate for Rare Disease Drugs
- Impractical for Small Patient Populations
- Does Not Capture the Value for Rare Disease Patients
- ICER framework may cause health equity disparities
- Quality Adjusted Life Years are the Wrong Measure
- Experts' Views Should Guide the Discussion
- Caregivers Deserve Consideration
- Community Views Should Be a Secondary Priority, not an Honorable Mention in the Footnotes

Survey Highlights: ICER Engagement

Did your organization choose to directly engage with ICER?



What were the nature of those comments relating to ICER's methodology?

"We have made extensive comments and/or recommendations to ICER regarding its misuse of assumptions, modeling methods, and clinical and economic data. In almost every case, **our concerns and recommendations were either ignored or acknowledged but not addressed**."

"**Opposing use of the QALY and questioning the focus on disease-specific areas** instead of including the reality that many people live with co-morbid conditions that are affected by treatments."

"**QALYs do not adequately capture the wide variety of benefits that a successful therapy can achieve**, including a person's return to economic productivity, their performance in school, ability to function as a caregiver for others, and so on."

Would you describe your engagement as worthwhile?

Engaging with ICER produced some report language changes.

Created a sense that they were listening.

Established a public record.

Cited some participant submissions.

Do you believe ICER should be part of the future of value assessment development? And in what way?

Needs more collaboration, transparency, and objectivity.

Needs to be “best in class” but free from bias.

Needs to focus on broader value questions in all of health care.

Needs to rethink the role of ”Independent Voting Committees.”

Rick Chapman, PhD

Chief Science Officer

IVI INNOVATION AND VALUE INITIATIVE

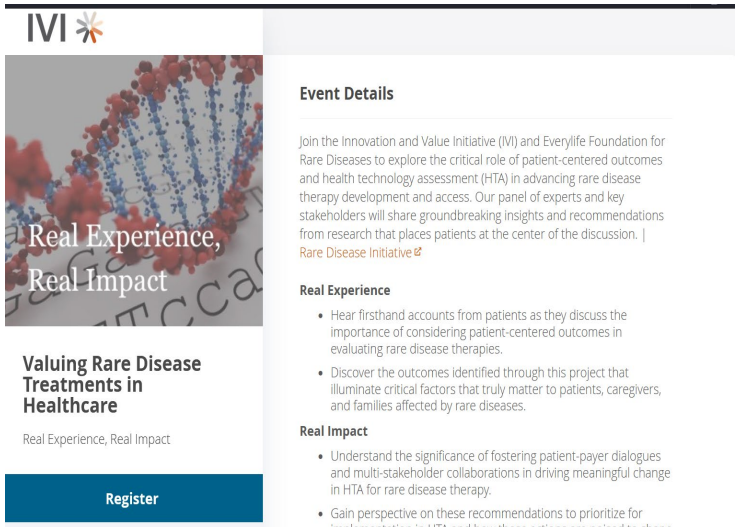
IVI Webinar - Real Experience, Real Impact: Valuing Rare Disease Treatments in Healthcare

Date and Time: Nov. 29, 12:00-1:00 pm (ET)

- Objectives:
- Share key recommendations and patient-centered outcomes identified from this project
- Emphasize importance of considering lived experience and patient-centered outcomes in value assessment of rare disease therapies
- Discuss implementation of recommendations from employer/payer perspectives, including importance of patient-payer conversations
- Hear patient and payer reactions to project findings and share experience/opinions

- Audience: People with rare disease, caregivers, payers, employers, policy makers, pharmaceutical manufacturers, researchers, and those conducting or using HTA

For IVI membership information:
https://thevalueinitiative.org/membership/



Register Now:
https://app.glueup.com/event/valuing-rare-disease-treatments-in-healthcare-92459/

Darius Lakdawalla, PhD

Director of Research at Schaeffer Center for Health Policy and Co-Founder, Chief Science Officer

USC Schaeffer
Leonard D. Schaeffer Center for Health Policy & Economics

entityrisk

Upcoming Ultra-Rare Review:
Paroxysmal Nocturnal Hemoglobinuria (PNH)

Discussion, Q&A

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Thank You!

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