



PhRMA Foundation Patient Centered Outcomes Webinar & Workshop Series: Advancing Patient-Centered Outcomes and Impacts in Value Assessment

Summary of Webinar #1: History and Current Landscape of Patient-Centricity and Patient-Centered Outcomes/Impacts in Value Assessment

September 17, 2021

In part one of this three part-series, the PhRMA Foundation aims to expose existing gaps in the current use of patient-centered outcomes (PCOs) in value assessment research, share and exchange approaches to enhancing patient-centricity and patient engagement in value models, and – ultimately – identify future opportunities for collaborative and inclusive research in this field.

Increasing focus on patient-centeredness in value assessment has revealed gaps between what patients report is most important to them and endpoints typically measured in regulatory approval studies. The presence of these gaps threatens the accuracy and viability of value and health technology assessments (V/HTA). Making progress will require identifying and measuring priority outcomes and impacts most meaningful to patients, while balancing the limitations and opportunities posed by pre-market and real-world evidence generation. This strategy requires multi-disciplinary and multi-stakeholder engagement to achieve a patient-centered and inclusive standard in value assessment to inform individual and population decision-making.

Feature Presentation: Elisabeth M. Oehrlein, PhD, MS, Assistant Vice President of Research and Programs at the National Health Council

To kick off the program, Dr. Oehrlein presented on the history of PCOs in health technology assessments and how these outcomes have traditionally been defined and incorporated into economic models of treatment value. While the outcomes used in HTAs were initially exclusively determined by clinicians with limited patient input, in recent decades, efforts such as the FDA's Patient-Reported Outcomes Guidance and the Patient-Centered Outcomes Research Institute (PCORI) have attempted to make value assessment more inclusive of patient input. However, challenges remain concerning the inconsistent collection of PCO which leads to confusion over which outcomes really matter to patients.

Key Value Assessment Terminology

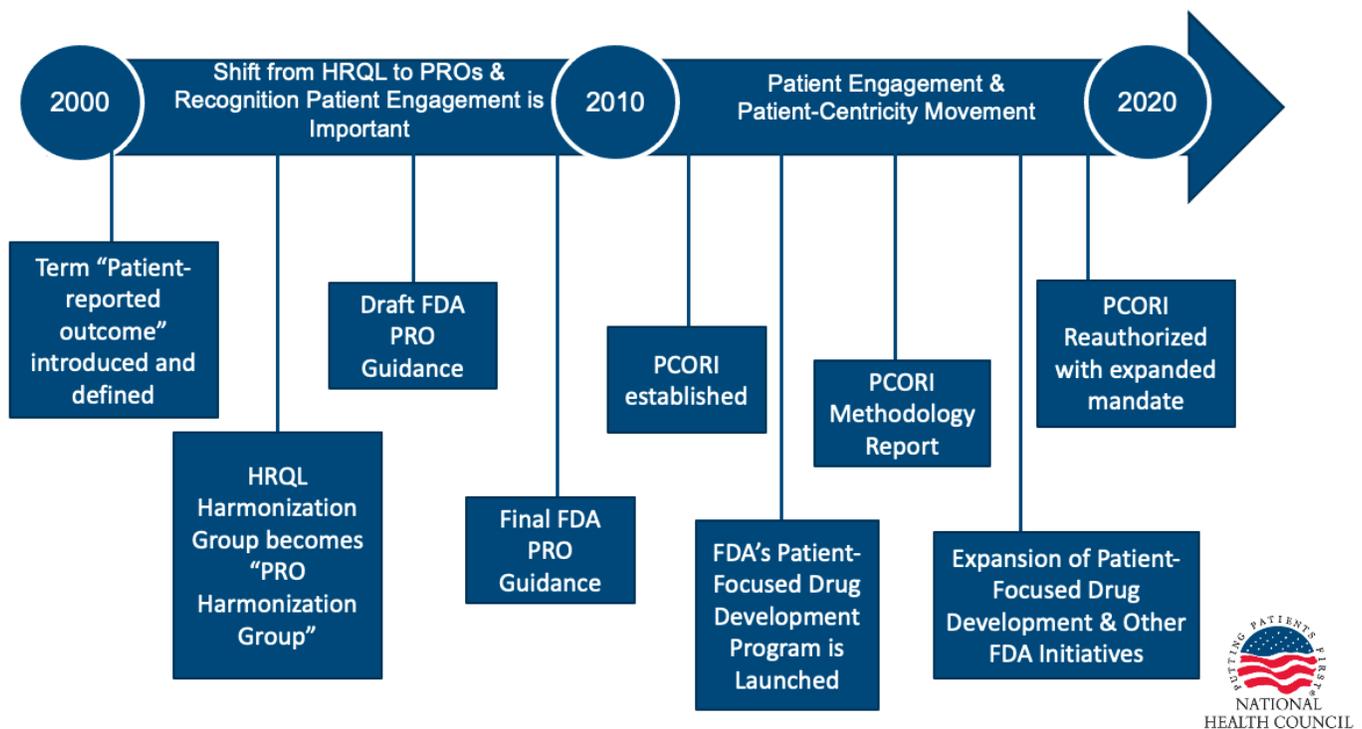
Outcome – Effects of care on the health status of patients and populations.

Patient Centered Outcomes (PCOs) – Outcomes that people who represent the population of interest notice and care about

Health Technology Assessment (HTA) – The multidisciplinary evaluation of health technologies.

Quality-Adjusted Life Years (QALYs) – Common measurement of health outcomes, combining length and quality of life.

The History & Current Landscape of Patient-Centered Outcomes



Dr. Oehrlein also provided an overview of the National Health Council’s work on PCO development as well as some helpful tips for researchers on how to incorporate PCOs, and how these topics relate to quality-adjusted life years (QALYs). Key takeaways from the presentation include:

- **There are a variety of ways PCOs can be captured.** Outcomes can be directly reported by patients themselves or collected by other means – such as from patient registries, medical records or as real-world data.
- **Don’t assume patients were engaged in outcome selection or outcome measure development.** Recent research has found that historically, HTAs did not engage with patients and that many models do not follow FDA guidance on PCO development.¹
- **The onus is on researchers to evaluate if measures are patient centered.** For example, the McGill Pain Questionnaire – a patient-reported instrument to measure pain intensity – has never been evaluated for patient-centeredness despite being used by 92 clinical trials since 2011.
- **Important questions to consider about the use of quality-adjusted life years (QALYs) in HTAs:** Is the QALY the best way to measure a patient’s health status? Is it fair to compare patient populations with the general public? Do health status questions represent what’s important to patients?
- **Inconsistent collection of outcomes that may or may not be patient-centered is a barrier to patient-centered research and health care delivery.** There is currently no uniform way to capture PCOs and incorporate them into economic models. The National Health Council is working to develop Disease-Specific Patient-Centered Core Impact Sets that aim to consider all of the health and non-health impacts patients report as important. These impact sets could then be tailored to specific diseases and used to evaluate treatments and healthcare interventions in a more uniform way.

¹ Ramasamy, Abhilasha et al. "Assessment of Patient-Reported Outcome Instruments to Assess Chronic Low Back Pain." *Pain medicine* (Malden, Mass.) vol. 18,6 (2017): 1098-1110. doi:10.1093/pm/pnw357

Patel, D A et al. "Patient-reported outcome measures in dysphagia: a systematic review of instrument development and validation." *Diseases of the esophagus : official journal of the International Society for Diseases of the Esophagus* vol. 30,5 (2017): 1-23. doi:10.1093/dote/dow028

Close, James et al. "Measuring upper limb function in MS: Which existing patient reported outcomes are fit for purpose?." *eNeurologicalSci* vol. 19 100237. 16 Mar. 2020, doi:10.1016/j.ensci.2020.100237

Panel Discussion: Gaps to Development and Implementation of Patient-Centered Outcomes in Value Assessment from Different Stakeholder Perspectives

Moderated by **Bari Talente**, Executive Vice President, Advocacy & Healthcare Access at the National Multiple Sclerosis Society, audience members heard from a panel of researchers and patient advocates discuss how best to prioritize patient-centered outcomes and ensure inclusive patient engagement in value assessment research.



Charlene Son Rigby – Chief Executive Officer, RARE-X

Q: What challenges and gaps do you see for rare disease patients in contributing to research, and to the development of patient centered outcomes? Do rare disease patients face specific challenges? How are those being addressed in the RARE-X context?

“The collective number of people impacted by rare disease is large – 350 million people worldwide. But, any individual rare disease has very few patients and there’s often very low awareness of that specific disorder...this makes it challenging to have a voice in research and even more challenging to develop patient centered outcomes.”

“If a rare disease group is lucky enough to generate researcher interest in their disorder, they often find that it’s challenging to provide input into the study and then even to access the data on the back end.”

“Why don’t rare disease groups initiate [natural history and data collection] studies on their own? Initiating these types of studies is very costly.”

“Some rare disease patient communities have collected their own data...and that is very exciting, because it really does give power to the patients. To define information that’s asked and include questions that matter to them. But we’ve seen some of these groups have had to start over data collection because they didn’t start with experience on how to structure questions from a survey methodology standpoint.”

“Patient centered outcomes start with patients owning data. So, the RARE-X platform is enabling gathering, structuring, and sharing patient data to support accelerating research, as well as future cures for rare disease patients.”



Ashley Valentine, MRes – Co-Founder and President, Sick Cells

Q: Would you please speak about outcomes and impacts important to patients in your community. How were those addressed or not addressed in value assessment efforts you have contributed to?

“Outcomes that are most important to [those with sickle cell] is first and foremost, access to quality care. Can someone with sickle cell enter the healthcare system and be treated as someone with a disease? Or will they be treated based on what they look like?”

“Data is sometimes misleading. We often hear things like, “people with sickle cell are not compliant, they don’t take their medications.” But if you talk to the people with sickle cell, its that they can’t get someone to write them a prescription.”

“Prior to [1998] we had no treatments, and if you look at research it’s really the impacts of race and being a community that is underserved...we weren’t considered valuable enough to put research dollars into.”

“Going to the hospital, it’s more than just dollars and cents. We’re talking missed workdays, missed school days, the mental health impacts of being hospitalized, and then for the cases of sickle cell, there’s always a racial component to it. So, if people are mistreated in the hospital, or if they’re treated based on skin color, the way they speak, if they don’t speak English.”

“Reducing encounters with a health care system means you’re also reducing the burden and the stress that comes with [a sickle cell] diagnosis.”



Lori Frank, PhD – Senior Scientist, RAND Corporation; President, ISOQOL

Q: Why should patient views be included in value assessment, and what are some of the problems that including patient views could solve?

“I would say that value is everybody’s business.... who decides value and who decides which patients are valued?”

“The patient view is a unique perspective, but so too is the clinician view, and the views of others within the whole healthcare ecosystem.”

“For understanding the value of healthcare intervention, specifically, there are many people who have a lot of different views, a lot of them conflicting. And first is figuring out a method for aggregating or reconciling conflicting views – and as part of that method – determining whose view should be represented is really key.”

“Even within a disease area, there’s a variety of views about what matters and a heterogeneity of experience that needs to be considered. So, aggregating across the right patients is a promising first step.

“Goal attainment scaling can be leveraged to help with this as one potential method. And then, a deliberative method like multi criteria decision analysis could be the next step.”

“We also need to consider when to include stakeholders as research subjects, from whom we collect data and when it makes sense to really partner with the stakeholders to advance valuation methods.”



Louis P. Garrison, Jr., PhD – Professor Emeritus, The Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute, Department of Pharmacy University of Washington

Q: How do we train the next generation of health economists to identify patient-centered outcomes and impacts and apply them when developing an economic model?

“This field is a multi-disciplinary effort. We work with others in the field outcomes researchers, quality of life, researchers, statisticians....and even within our health economics field, there are a number of sub specializations.”

“I think the big market failure we still have in this ecosystem is that we don’t generate high quality information on what happens after products are on the market...we’re not finding how things are performing in the real world.”

“Patient centered outcomes are important that each of these different decision contexts. From the drug development decision to the regulatory decision to the decision that we mostly focused on, the HTA decision – should it be in the benefit package?”

“I think it's important to keep in mind that patient centered outcomes plays a role in each of these different decision contexts and that they're tied together.”



Sean Tunis, MD, MSc – Principal, Rubix Health; Senior Fellow at the Tufts Center for the Evaluation of Value and Risk in Health

Q: We have discussed how outcomes should be collected consistently across studies. I know you have been involved in developing core outcome sets. Would you please speak about barriers to uptake of core outcome sets and suggestions for stakeholders interested in promoting the uptake of core sets of patient-centered outcomes?

“There has to be a process to whittle down from the whole universe of outcomes that matter to patients to a core set that would be measured and reported in all trials in a specific therapeutic area, whether it's sickle cell disease or hemophilia or asthma.”

“I like to use the analogy of Consumer Reports which looks at products like dishwashers. They will give you a table of, let's say, six or seven performance features of the products that consumers think are most important — like how clean they get the dishes or how noisy it is or how often it breaks down. You can think of those as analogous to core outcomes in clinical studies.”

“To be useful, you need to see measures of these core features for all the products, which Consumer Reports will provide. That's exactly the problem when value assessors try to look at outcomes from clinical trials – they're all over the place! And it's a huge barrier to doing meaningful comparisons between treatments and related value assessment.”

“We need to ensure that the core outcomes reported in trials accurately reflect the outcomes that matter most to patients, and then we need to find a way to make sure that researchers consistently use those outcomes in research so that when the evidence is reviewed by value assessors it is possible to compare apples to apples on those critical outcomes.”

“Now, how do we do that? I think that folks who do health technology assessment need to start judging the quality of studies, based on the degree to which they have reported all of the core outcomes that have been identified by patient groups as most important to them.”

Audience Participation: Q&As

Q: Do you think that incorporation of meaningful patient input will increase over the next few years?

Charlene Son Rigby:

- “I do see that the landscape is changing, and we see that researchers are highly interested in trying to figure out how to do this, but it isn't straightforward yet.”
- “I'll give an early stage example from STXBP1 my daughter's disorder. Caregiver surveys.....identified speaking communication as a top burden. But most STXBP1 patients are non-verbal or their minimally verbal and there are no papers or studies that have looked at this in any depth.”
- “While there is a new study, it's based on medical history and medical records and only 40% of the patients have an identified speech issue. So, there's clearly a very big gap in terms of the way that the data is being collected and the patient and family experience.”
- “The data from the caregiver survey was that speech and communication was a top burden of this disorder and it's basically missing for the majority of patients in terms of tracking. So now the [disease] community

has started to look at new communication measures to be able to measure nonverbal communication and are now working with Duke on a new measure.”

Ashley Valentine:

- “More emphasis on talking to caregivers is needed. In sickle cell, caregivers report that the person they’re caring for having double the number of bad days as the individual their caring for themselves report having.”
- “Perhaps the question here is not how do we train patients to work with researchers, it’s how do we train researchers and hospital networks and payers and insurers and the value assessors on how to work with patient organizations.”
- “When producing research, it’s so important to highlight your limitations, because [talking about] limitations....and what you didn’t find [is just as important as talking about] what you did. And I see right now in value assessments that are being published is that the limitations are often underplayed for sickle cell disease. We were not able to measure race or the impact of racism. And anyone who knows sickle cell knows that you cannot talk about sickle cell without talking about the impact of racism.”

Q: How do we incentivize drug developers to incorporate more PCOs into clinical trials so that data is then available for value assessors?

Sean Tunis:

- “If the drug developers come to understand that when the quantitative part of value assessment is done, the studies that report outcomes that matter most to patients consistently are going to be the ones that are weighted most heavily in the judgments of effectiveness and value. Once it is clear that reporting those outcomes will impact the conclusions about cost-effectiveness and value-based prices for drugs, that would be a huge incentive to measure and report those outcomes in clinical studies.”

Lou Garrison:

- “There are other problems in our healthcare system besides the fact that we don’t measure patient centered outcomes. There are other problems related to insurance coverage that people face high out-of-pocket payments. They face financial catastrophe. That has an impact on the patient, but it’s not a health outcome. It has to do with the failure of our insurance system.”

Bari Talente:

- “We’ve heard too often that patients aren’t adherent to treatment, but then we’re not digging into ‘why?’ that’s happening. It can be out of pocket costs or travel to a provider or these other pieces. The outcomes are important and so are the impacts and understanding, so we have to make sure to understand the ‘why?’.”

Q: How do we make sure that we disseminate these findings beyond those of us just in the value assessment community?

Charlene Son Rigby:

- “We need to translate a lot of these...academic or research-type pieces of information into shorter, much more consumable pieces. A lot of the patients are really looking at information on social media so that’s a very important component to the equation as well.”

Ashley Valentine:

- “If we’re thinking about coverage, the limitations and findings of [value assessment] studies need to be translated and given to Medicaid, in all 50 states and DC, and then the local communities that are working with Medicaid so they can make accurate coverage decisions.”