



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

### Summary of Webinar #2: Spotlight on Current and Emerging Approaches and Methods to Enhance Patient-Centricity and Inclusive Patient Engagement in Value Assessment

November 12, 2021

In part two of this three part-series, the PhRMA Foundation aimed to provide attendees with a deeper understanding of real-world approaches toward advancing patient-centricity and inclusivity in value assessment. Participants heard from leading health economics researchers representing three of the Foundation's Centers of Excellence, as well as the Innovation and Value Initiative, highlight their latest efforts to move the conversation forward. The presentations were followed by a lively discussion between the researchers and three patient advocates who shared their own experience working to make value assessment more inclusive of diverse perspectives.

#### Value Assessment Researchers

- **Rick Chapman**, PhD – Chief Science Officer, Innovation and Value Initiative (IVI)
- **R. Brett McQueen**, PhD – Director, pValue & Assistant Professor, Department of Clinical Pharmacy, University of Colorado Skaggs School of Pharmacy and Pharmaceutical Science
- **Peter J. Neumann**, ScD – Principal Investigator, CEVA & Director, Center for the Evaluation of Value and Risk in Health at the Institute for Clinical Research and Health Policy Studies at Tufts Medical Center & Professor of Medicine, Tufts University School of Medicine
- **Julia F. Slejko**, PhD – Co-Director, PAVE Center & Associate Professor, University of Maryland School of Pharmacy



#### Patient Advocates

- **Kenneth "Kenny" Mendez**, MBA – CEO and President, Asthma and Allergy Foundation of America (Moderator)
- **Annie Kennedy** – Chief of Policy, Advocacy, & Patient Engagement, EveryLife Foundation for Rare Diseases
- **LaTasha H. Lee**, PhD, MPH – Vice President, Clinical and Social Research and Development, National Minority Quality Forum (NMQF)



## Featured Value Assessment Research Institutions

### Expanding the Use of Patient and Societal Considerations in Value Assessment



IVI's mission is to advance the science and improve the practice of value assessment in healthcare to make it more meaningful to those who receive, provide, and pay for care. IVI has a goal of building a community to create rigorous, innovative, and relevant methods and translational research that facilitates patient-centered value assessment and emphasizes flexibility and usability for the unique decision contexts of individual decision-makers.

### Deriving Patient Experience Value Elements in Rare Disease: The Role of Multi-Criteria Decision Analysis



The University of Colorado's pValue initiative is exploring how multi-criteria decision analysis (MCDA) can improve decision making in health care. By engaging key stakeholders to quantify and weigh decision-making criteria, MCDA provides a tool that can help answer this in ways that are more objective, comprehensive, transparent, and sensitive to differing perspectives of patients, caregivers, and other stakeholders.

### Incorporating Non-Traditional Elements of Value into Cost-Effective Analyses



Headquartered within the Center for the Evaluation of Value and Risk in Health (CEVR) at Tufts Medical Center, CEVA aims to explore the incorporation of non-traditional elements of value into cost-effective analyses. These efforts involve engaging stakeholders – including patients, health insurers, and therapeutic area leaders – to identify important novel and non-standard elements to inform coverage, reimbursement, and access decisions.

### Capturing Value from the Patient Perspective



PAVE (Patient-Driven Values in Healthcare Evaluation) is a unique collaboration between the University of Maryland School of Pharmacy and the National Health Council dedicated to developing and advancing new methods to incorporate the patient perspective into value assessment and value-based decision-making. PAVE takes a bottom-up approach using a systematic process to identify patient-driven value elements that are important in healthcare decision-making:

- Engage patients directly to elicit elements of value
- Prioritize and refine value element priorities specific to specific clinical areas
- Quantify patient-informed value elements for translation to economic evaluation

## Panel Discussion with Patient Advocates and Researchers

### ***The Role of the Patient Organizations in Value Assessment Research***

**Q: What works, what has been successful, and what challenges have you faced in your work as a patient advocate?**

Annie Kennedy:

“Early and ongoing collaboration of patient communities is needed – not just at the beginning and not just at the end. We need a frame shift from early and often to early and *always*. “

“We also really need to be cognizant of the need to balance the data collection needs with the additional burden of participation on the patients...we’ll have to ask ourselves, what’s the need to have versus the nice to have when we’re thinking about these data collection efforts.”

“For patients and caregivers, the goal is to improve lived experience of a disease...this requires we go beyond typical biomarkers and traditional clinical outcome measures.”

LaTasha Lee:

“Patient engagement has not happened early enough and is not sustained. Early, meaningful engagement is really important – and it’s not just the patients sharing their experience but also [the value assessors] sharing their perspectives and goals as a part of this. Oftentimes, the patient community is not aware of the end goal and how that can inform the outcomes of some of these assessments. Education should be a two-way street.”

“Another thing to note; many value assessments, when working with marginalized communities, deal with a lot of data that may not be the most representative of those communities...So, value assessments that utilizing that data could actually be doing a disservice to those communities.”

“It is important to bring in real world evidence as well to capture the full patient lived experience. Place matters. And going to the same group for value assessment is not the best approach because we know that there are different lived experiences in various diseases.”

### ***Health Equity in Value Assessment***

**Q: For LaTasha, what are you seeing on the health equity side in terms of short comings?**

LaTasha Lee:

"It’s important to consider, for whom value is being provided”

“Unfortunately, the conventional value assessment methodology that is described in some of the earlier talks today really are dismissive of [patients from marginalized communities] and their lived experience.”

“Many of the flaws in the cost effectiveness analyses that are currently in use really have pronounced issues when looking at [marginalized communities] who have not received equitable consideration in the research in terms of diagnosis and even treatment paradigms.”

“In trying to understand the value of a treatment in these populations...it’s important to really think about where those patients are to ensure their voices are heard. One of the efforts that I really value is the PCORI

approach, ensuring that patient centered outcomes are reflective of the people, the caregivers and the communities impacted by a disease.”

**Q: For the researchers & modelers, how do you include health equity in Value Assessment models?**

Peter Neumann:

“I think a lot of groups that do value assessment struggle with this to some extent. But there are some initiatives. Extended cost-effectiveness is one, where we’re measuring how results vary based on [a variety of factors]

“Looking at aspects of financial risk protection – the idea that a new treatment might offer better health benefits but also might protect patients financially because it allows people to go back to work, and perhaps even saves them from going into bankruptcy. Another idea are the value dashboards – there may be ways to measure and display aspects of equity that could be displayed alongside traditional cost effectiveness information.”

Brett McQueen:

“We need to have collaboration with dedicated health equity researchers to generate the evidence that flows directly into models. We know that there are disparities. But the evidence is often not available or useful for modeling analyses.”

Rick Chapman:

“We need to acknowledge that this is an area that our field hasn't dealt with very well, I think we are just starting to realize that and get some other methods in place.”

“I think one of the things that would help is if we had a more explicit framework that acknowledges where those gaps are. So, rather than just brushing those aside or passing over them because we don’t have the evidence, we need to point out explicitly that there is something we wanted to look at and include. We just really didn't have the evidence to be able to do it.”

Julia Slejko:

“From a modeling standpoint, it’s a huge challenge.”

“DCEA (distributional cost effectiveness analysis), I think shows a lot of promise for addressing some of these issues”

“That’s why it’s important to learn from patients specifically about their lived experience... to understand what the priorities of the value elements for different types of patients are. It’s been a challenge in our COPD work...and I think emphasizes how important it is to partner with patient organizations who can help you reach some of those diverse groups.”

“It’s also important to think about diversity in payer type. One thing that we learned in our COPD work is that our first phase had recruited a lot of patients from clinics in Baltimore...many of those patients were veterans. So who pays for their health care is very different for them than for some of the patients who we recruited through the other organization we were working with. When it comes to the financial aspects of treatment, it’s important to think about this kind of diversity too.”

## **Patient Advocates Respond**

### Annie Kennedy:

“We need to be thinking about equity more broadly. Value assessment isn’t static and patient perceptions can change over time. In our recent engagement with PAVE...cultural and political context were really important. Some of the patient groups we were engaged with really helped us understand that impact of health insurance vulnerability that many patients and communities were experiencing at the time. “

“Another thing to think about is *healthcare tenacity* – the ability to navigate complex healthcare environments, access a diagnosis, an approved therapy or be able to file an appeal [with an insurer]. These are all equity issues, and I think we need to think about whose getting left out when we don’t think about these issues.”

### LaTasha Lee:

“Garbage in, garbage out. Until we think about some of those issues, these models really will be flawed for particular communities.”

“Look at the changing demographics of this country, by 2050 we're going to be a majority minority country. If we don't begin to address issues of equity – I know it's challenging from a value framework – we're really putting ourselves at a disservice.

## ***How to Get More Patient Groups Involved in Value Assessment Work***

### Annie Kennedy:

“There are hundreds of patient organizations...What we need to be leaning into next is ensuring that all of this work that was presented here today is being pulled down and operationalized into elements that can be incorporated into their registries, so that when you're developing the next methodology for value assessment that data is ready to get pulled in.”

### Rick Chapman:

“There needs to be a lot more dialogue in terms of what types of evidence value assessment organizations are looking for and how they think about levels of evidence. Because I think there's often a disconnect between the data that is collected and what's fit for purpose for value assessment.”

### Julia Slejko:

“Oftentimes when we're getting ready to do our evaluations, we don’t have [input from patient groups] that has been developed early on. And by then, it’s too late to include it.”

“Sometimes the issue of how people are going to access these technologies is not even a concern. So, we have to start even earlier than clinical trials.”

## ***Questions From the Audience***

**Q: How can we move the field forward in developing data to allow for more meaningful methods like MCDA or distributional CEA with respect to health disparities?**

Brett McQueen:

“It’s really important to define health equity and how it actually fits into whatever methods we’re using like MCDA or distributional cost-effectiveness. With MCDA, the way we’re using it, can help us identify the priorities within health equity areas and how we can translate those into value assessment.”

“The exercise of identifying whether you can do a distributional cost effectiveness is actually very informative. If gaps are present, one can then generate the evidence to inform the distributional cost-effectiveness analysis and produce standalone results important to multiple stakeholders.”

Rick Chapman:

“The way we define equity is also one of the barriers, we have right now. Because we know there are many ways to define equity and I don't think we really know yet quite how to deal with that in our methods.”

Peter Neumann:

“What we tend not to do in [cost effectiveness analyses] is look at some of the implementation issues that might accompany the treatments. We know from clinical trials whether the treatments work or not. Whether the treatments work in the real world is a function of many other factors that we typically don't include in the analyses and often don't have good information on. Adherence and access issues, for example. We need to do better on all of those things.”

**Q: What are some alternatives to using the QALY for deciding how much to reward innovation for, say, a new diabetes drug versus the new oncology medicine?**

Peter Neumann:

“There's very interesting work coming out of different places that argue we should risk adjust QALYs and adjust cost-per-QALY thresholds for severity. Perhaps in the future, we have conventional QALYs and augmented QALYs and risk adjusted generalized QALYs [all together] presented to decision makers. I think the best we can do as analysts is try to measure value as best we can, recognize limitations and alternatives, and then give that information to the decision makers.”

Julia Slejko:

“We’re currently looking at some of our patient preference data – how patients make their tradeoffs and what their priorities are in a treatment – to think about how we might be able to use some of that to adjust health state utilities. While that’s not necessarily an alternative to the QALY per se, you can get to a point where you have several outputs that you can display for decision makers.”