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

Summary of Webinar #3: Looking Forward – Opportunities Towards a Collaborative Research Agenda for Patient-Centered Evidence Generation and Broad Impact Beyond Current Models

December 10, 2021

In the final part of this three part-series, the PhRMA Foundation aimed to discuss the future of value assessment research. The webinar featured two panel discussions focused on patient centered outcomes and health equity in value assessment. Following the discussion, the Foundation unveiled the research questions for the 2022 Challenge Awards.

Patient Centered Outcomes Panel

This panel discussed how to address a key challenge in patient centered outcomes research – the lack of data available to measure PCOs, especially those not captured in clinical trials.

- **Jennifer Bright**, MPA, President of the Innovative Value Initiative (IVI) – Moderator
- **Charlene Son Rigby**, Chief Executive Officer of RARE-X
- **Lori Frank**, PhD, Senior Scientist at Rand Corporation and President of ISOQL
- **Edward "Eddie" Lee**, PharmD, Head, US Health Economics and Outcomes Research (HEOR) Strategy of UCB

Health Equity Panel

Panelists discussed how value assessment research can capture and communicate differences in outcomes and preferences that impact diverse and underrepresented populations.

- **Bari Talente,** Executive Vice President, Advocacy and Healthcare Access, National Multiple Sclerosis Society – Moderator
- **Terri M. Booker**, Esquire, Board Member of Sick Cells and Hemoglobin Advocate
- **Eberechukwu Onukwugha**, MS, PhD, Associate Professor of Pharmaceutical Health Services Research at the University of Maryland, Baltimore
- **Stacey Kowal**, MS, Principal, Health Policy & Systems Research of Genentech

Patient Centered Outcomes Challenge Award Questions

1. What are potential solutions for more rapidly prioritizing and closing evidence gaps to measure treatment effects on patient-centered outcomes and impacts (e.g., using RWE and electronic health data) in clinical or economic evaluations? Include case studies and consider the role of key research organizations (e.g., PCORI, AHRQ).

2. What templates or techniques can be employed to reliably demonstrate important gaps in meaningful patient-centered outcomes and impacts?

Health Equity Challenge Award Questions

1. What methods or processes can be employed to inform clinical and/or economic evaluations to capture important differences in experiences, preferences, and health outcomes of diverse populations, particularly among groups typically underrepresented in research?

2. What templates or techniques can be employed to convey these differences in how results are communicated when adequate data are and are not available?

Patient Centered Outcomes Panel

Q: Specifically, from a rare disease perspective, what's at stake when we get it wrong? What are we leaving on the table by not having adequate measures of patient impacts? What are your organizations doing"?

Charlene Son Rigby:

"Fewer than 5% of rare diseases have an approved therapy and these therapies typically have an orphan designation…there's growth in this area, but there's clearly a huge amount of white space and opportunities for precision therapies for the thousands of rare diseases."

"A significant challenge is the that rare diseases typically have little or no natural history or data collection to characterize the disease, the progression over time, and the impact on patients."

"At Rare-X we have launched a cross disease, patient level data collection platform…Our goal is to capture robust patient data on patient's symptoms and to do this in a broad way."

"Quality of life is an important aspect of the data we're collecting and we're looking to do this on a disease domain level, so we can capture quality of life impacts related to disease specific areas. We're also investigating ways to extend this to capture impacts beyond quality of life scales."

Q: What's changed about methods and researcher's endeavors to engage patients in different ways to elicit patient important impacts. What you see some of the challenges and opportunities in front of us?

Lori Frank:

"One of the biggest changes is an acknowledgement that beyond capture of the patient voice, but active patient engagement has different dimensions to offer to improving measurement"

"Challenges I've encountered in the field are that these issues are relatively new, and they haven't been brought into health technology assessment"

"Active engagement [with patients] means broadening concept elicitation, so who's at the table during concept elicitation and what role are they playing?"

Q: What's top of mind for you in terms of patient engagement and better capture of patient important impacts? How have you seen that change over time and what are the things you are emphasizing as a sponsor of this type of research?

Edward "Eddie" Lee:

"We're really focusing on how to enhance our ability to translate the clinical benefits of a particular asset or drug. Whether it be outcomes that are physician or patient reported, into financial impact from multiple perspectives."

"There's a fundamental shift in disease states, where the outcome is primarily measured by physician or lab assessments, to whether you're achieving an outcome or not based on a patient reported outcome."

"We wanted to be consistent with how we approach [value assessment], ensuring that whatever decision that we made…that we continue to keep the patient at the center."

Q: How have you dealt with prioritization questions? Are there certain aspects of the patient experience that are the most important to capture and improve the consistency of that data?

Lori Frank:

"We don't know how to prioritize and I think that's really key in terms of the methods. It's listening to the right voices and the right stakeholders. Patient centeredness doesn't mean patient only outcomes, to the exclusion of other outcomes."

"There's disease by method interactions that need to be considered."

Charlene Son Rigby

"We need to form that baseline so that we at least have some data to start from."

"There may be different priorities depending on the type of symptomology for that specific disorder."

Q: What have you found in your efforts to implement research and your journey to bring a diverse patient community together? Where have made progress and where do we still have to work?

Lori Frank:

"The first question to address is feasibility. To what extent is outreach widely to those with lived experience feasible, and what are the methods that should be pursued?"

"Who's at the table [for a disease]? Race, ethnicity, gender, age, what is the ideal representation, so that our methods are rigorous for inclusion?"

"Source data is really key. There needs to be attention to collecting data from the right set of patients."

Charlene Son Rigby:

"Representativeness is a huge challenge for us, especially from a rare disease perspective. Numbers of patients are small and dispersed geographically."

"Online methods are improving the abilities to identify patients and gather data from them. But it's not the full answer. There are some patient communities who are not comfortable providing medical information online so we need to figure out the right ways to engage those communities and bring them into the dialogue."

Edward "Eddie" Lee:

"It's not only about capturing the right patient centered outcomes but obtaining the relevance of these patient centered outcomes from the right patient groups. So, from an industry perspective we focus a lot on who we need to enroll into our clinical programs."

Q: How do we normalize the collection and use of patient and caregiver important data? What is the biggest new horizon for the use of this kind of data? Do you think there is still a problem with forgetting the importance of collaboration?

Lori Frank:

"The idea of multi-stakeholder consensus being normalized is part of the process. This is the way it's going to be done, because there's recognition of the cost of not doing it that way"

Charlene Son Rigby:

"I think we here that attitudes are changing, but attitudes to practice…is still a work in progress."

"It can't always be an immediate feedback loop. Information is feeding into a larger project, where there will be an output. But it might be further down the road and understanding that is important."

Edward "Eddie" Lee:

"From a manufacturer sponsor side…we want to be very transparent as to why [patient participation and feedback] is important."

"We always ask ourselves; can we ensure that there is clear value to the patient spending their time to contribute."

Q: Where do we need to forge new partnerships with patient organizations?

Charlene Son Rigby:

"Robust data sources that are going beyond the very basics in terms of real world evidence…Bringing together data providers that are actually coming from different resources."

Lori Frank:

"There needs to be some external incentives to really add some fuel to [connect stakeholder communities]"

Edward "Eddie" Lee:

"We want patients to know that [their input] is not going into a black box….it's advancing the science. And the way we do that may vary greatly by disease state or how patients like to work with industry."

Q: What are the things that make you most hopeful about this area, and what we can see in the future?

Edward "Eddie" Lee:

"I think there's an awareness that this is kind of the way of the future and is something that has to be addressed…as something that needs to be systematically incorporated into the development process."

Lori Frank:

"I'm hopeful because there is increasing recognition of the ethical impure imperative for representation, that it is an element that needs to be part of the work."

Charlene Son Rigby:

"I've really been seeing that there's an acceleration in therapy development, in rare and other sectors, and initiatives like PCORI and the Patient Focused drug development form…are really seeing demonstrated success."

Health Equity Panel

Q: Can you describe how you think we're starting to slowly make progress in terms of equity?

Stacey Kowal

"We're trying to generate three pieces of information: 1.) Across the US, what is my capacity to benefit, what is my health, what does my quality of life look like based on my race and ethnicity, where I live, my sex, my age? 2.) How do you actually collect equity relevant information and equity relevant subgroups? 3.) How do we value health equity?"

'If we don't capture heterogeneity we're not really going to understand who's losing when we make decisions."

<u>Terri M. Booker:</u>

"It starts with listening to patients…and not jumping to conclusions"

"We have to talk about racism. We have to deal with the biases in the medical system. Those are the things we have to discuss in order to be able to digest what's going on."

Q: What's getting in the way with measuring equity? What are the tools and resources that you really need to able to engage in this work, and come up with measurable outcomes?

<u>Eberechukwu Onukwugha</u>

"Others have alluded to investments in big data…what does that mean for methods, for algorithmic biases and then beyond that?"

"We need a recommitment to improving the measures that we already have…this could start immediately with the measures we already have dispersed throughout different data sets."

'We have a lot of tools already available to direct us as to how to collect [equity] measures. We now need to invest the dollars that are needed to fund more systematic collection of these measures."

Q: If we really looked at health equity components, how do we think that data might be telling us something different? Or would it be the same?

<u>Stacey Kowal</u>

"You need to think about what drives outcomes, whether its Anderson model or country health rankings models. The more information we can put in about other things that we know drive health outcomes, the more likely we are to get correct conclusions."

"Each dataset has its own limitations, and there are different ways to enrich or link them. Even something as simple as geography helps us to do a little bit better than simply saying here's comorbidities, race and ethnicity, that are self reported."

<u>Eberechukwu Onukwugha</u>

"We want to move away from anything that suggests we're blaming the victim. If we look at race and ethnic disparities in isolation, we run the risk of ignoring the role of systems and policies."

"If you think about socially assigned race compared to self-reported race, I think that opens up additional points for investigation to dig deeper into the patient's experience and invites more nuanced conversations about the implications for engagement with the healthcare system."

<u>Terri M. Booker:</u>

"The last session, they were talking about humanizing their experience. I know we have to do the data, but I mean that's the part of making things realistic. This is a person and they're going through a human experience and how can we help make it a better experience, while remembering that things happen. [A patient] lost health insurance, couldn't afford some of the pills you asked me to buy, I didn't know that I could get a coupon that will help me."

"Giving information to patients, making sure that they're aware of their options… giving resources without necessarily asking "do you need this?", then you start getting the information back."

Q: What are some other ways that we can incorporate some of this health equity data and the conversations we've been having into HTA?

Stacey Kowal:

"For equity, it's not just for population X vs population Y…everything has an opportunity cost."

"Why don't we take methods that are out there, and say they might not be perfect, but let's discuss them."

"I think we start with the methods other countries are using. Why shouldn't we be trying them, and then also using it as a roadmap"

Eberechukwu Onukwugha:

"When we recruit and engage with communities about clinical trials, are we open to having fundamental conversations about treatment and medical decision making? Do we truly understand the patient's perspectives? Do they consider [treatment] to be beneficial? Do they see it as toxic? When treatment requires a commitment over time, do patients see it as something that they need to be investing in and wanting to receive in their bodies? Do they see it as something that's life prolonging?"

"Commitment to understanding the community perspectives on treatment and clinical trials will go a long way towards engaging the community as partners."

Q: What makes you hopeful and what is one concrete step that you would like to see happen to move us forward?

Stacey Kowal:

"I'm hopeful because discussions like this are a lot more commonplace…We're finally valuing the importance of addressing health disparities."

"The most important next research step within the field of HEOR, is having a roadmap, kind of where we are today, what are the terms, what are the problems and where do we think we should go?

Terri M. Booker:

"I'm hopeful, because we are having this conversation…and I'm hopeful that those who are creating the assessments are able to connect with the patients."

"I'm really hoping for a shift in American culture, shift to prioritizing health…If you're not healthy you can't do anything."

Eberechukwu Onukwugha:

"I am hopeful when I think about our pipeline of researchers and trainees. We need an army of researchers who are committed to applying the best science to support and improve [these measures] so that we can maximize our impact. Rather than try to re-invent the wheel, we should leverage existing organizations, like ISPOR and ISPE, and other aggregators of scientists and policy makers to keep these conversations going."