Patient-Centeredness and Engagement in Clinical Research: Opportunities for Industry

March 10, 2014

About This Document
The Patient-Centeredness and Engagement in Clinical Research: Opportunities for Industry webinar held on February 5, 2014 received a large volume of questions during the event. Due to time constraints, some questions were not addressed.

Below are questions and answers for both outstanding questions and questions answered during the event. Within each section, questions are grouped by respondent, either an individual panelist or an organizational response.

We wish to thank all the webinar participants for their thoughtful questions and comments.
Answers from Janet Woodcock, MD, Director, Center for Drug Evaluation and Research, FDA:

Q. Can we use new PRO instruments like EXACT to potentially achieve a PRO label claim for a drug?
A. Usually they would need to be qualified to be used for a label claim.

Q. Thank you for sharing the 16 conditions you are undertaking at this time. I see chronic kidney disease/transplant is not on the list. Was it considered/“in the running?” What are your thoughts about the appropriateness of this broad condition for PCORI?
A. Because there are so many diseases, FDA could not get to them all. We tried to select a range. I can't comment on PCORI.

Q. Can anyone comment on the FDA’s recent news regarding encouraging pharma to include more patients with multiple chronic conditions in clinical trials and the potential benefit for patients overall?
A. We are trying to encourage trials with more generalizable results.

Q. How did FDA develop the questions it asks during meetings with patients? Were current PROs consulted, for example, or advocacy groups, etc.? And were they validated in some manner to derive desired information?
A. Yes, we went through a process to develop them.

Q. Is there any consideration for simplifying the increasing complexity of pivotal clinical research studies from the point of view of the burden on patients in terms of time, procedures, and difficulty? In addition to PRO measures, does this initiative encompass patient input on other things like cost and availability? Does this initiative encompass pharmacogenetic testing/advancement?
A. Not from FDA.

Answers from Freda Lewis-Hall, MD, Chief Medical Officer, Pfizer Inc.:

Q. Are companies adding patient advocates to their teams directly in order to provide insider views?
A. It’s difficult to speak to all the specific ways biopharmaceutical companies are engaging patients. It may be the case that patients or their advocates are direct members of one or more drug development team, but to date it seems that these groups and individuals are engaged to help at specific stages of the product planning, development, and clinical testing cycles. Pfizer, among other companies, is in close contact with a number of patient advocate groups, and we value their insight on issues such as the value of a proposed medicine, recruitment of clinical trial volunteers,
and planning to ensure access to the new therapy. More engagement, at many more stages of the process, may come in the future if and when FDA provides greater guidance on how this engagement should take place.

**Q.** Can you please provide examples of how industry can improve patient-centeredness, like an example for “external collaborations?” Can you please give some concrete examples from the industry/foundation collaborations?

**A.** The simplest example would be to work with patients and their advocates to understand their preferences in terms of treatment outcomes. This type of information can be hugely helpful in the clinical trial planning process, and can guide much of our early product discussions with the FDA.

We are seeing new models for developing drugs that target small populations in partnership with patient organizations. One patient engagement initiative that’s drawn notice concerns Vertex’s Kalydeco, which proved effective in individuals who carry a G551D mutation in the cystic fibrosis (CF) gene, which accounts for only 4 to 5 percent of CF cases. The early development of this medicine was funded and supported by the CF Foundation in exchange for a royalty on future sales. Kalydeco was launched in 2012 and has set a model for similar partnerships between other foundations/patient groups and drug developers.

**Q.** Who is “leading the industry” in terms of patient engagement? You mentioned Sanofi and Pfizer working with patient engagement activities... any other examples of “what good looks like?”

**A.** It’s too early in the process to declare one company or one approach to be “leading.” There are lessons to be learned from all industry members, and it’s important that we share these lessons with one another in an effort to improve our mutual approaches continually.

**Q.** From an industry perspective, internally it is hard always to engage key stakeholders in these activities because of the pressure on timelines and budgets, etc. Is there any advice regarding how we can “sell” the importance of initiatives internally?

**A.** When it comes to a company’s messaging, there is really no line between internal and external. We have to continue to communicate that greater patient engagement is ultimately good for patients and good for the business. We believe that patient engagement has a lot of positives, especially as we gauge the potential value of new medicines to patient and payer alike. The risks in new product development are so significant that any way we can both reduce risks and improve the odds that a new medicine will be successful is likely to get people internally to sit up and notice.

**Q.** Freda, you mentioned that it’s important to move away from simply asking patients for their insights, e.g., to do such things as monitoring patient functioning and behaviors while at home. What other innovative examples are there for industry to consider?

**A.** There is so much potential for technology to play a role in gathering patient insights. I was excited by the announcement that Google had developed a contact lens to measure blood glucose—what a reliable, unobtrusive way to gather important data that will help physicians understand more precisely how their patients are reacting to both their diet and new treatments. I think these types of innovations—those that are more objectively tied to the patient experience—will prove instrumental in the next wave of clinical innovation.
Answers from Susan Sheridan, MBA, MIM – Director, Patient Engagement, PCORI, and Anne C. Beal, MD, MPH, Deputy Executive Director and Chief Officer for Engagement, PCORI:

Q. The Tier 1 awards to date have been regional, correct? When will other opportunities open?
A. We anticipate that Tier I applicants and any outreach done within Tier I projects will be regionally based (local, state, or regional in scope). Reference the map here. There will be additional Tier I opportunities in other regions of the country in early 2014. Learn more about the Pipeline to Proposal Awards here.

Q. Is PCORI getting involved with investigator-initiated research studies or just larger sponsored studies?
A. PCORI is pursuing two complementary but equally critical approaches to identify and select research topics to fund: the investigator-initiated approach and the patient- and other stakeholder-initiated approach. In the investigator-initiated approach, investigators submit proposals in response to PCORI Funding Announcements (PFAs), broad calls for research proposals in five priority areas. However, we believe research meaningfully informed by input from patients and other stakeholders is more likely to be used for making patient-centered health and healthcare decisions and ultimately improve patient outcomes. Therefore, we require that the proposals we fund under these broad PFAs include patients and other stakeholders in each step of the research process—from proposal development to research design and dissemination of the study results. The second path, the patient- and other stakeholder-initiated approach, is designed to produce “targeted” PFAs through a systematic topic generation and research prioritization process. This process begins with potential research questions solicited directly from patients and other stakeholders through our website, engagement initiatives, and similar efforts undertaken by other healthcare experts and organizations.

Q. Having CER as its focus for both PCORI and managed care, is the payer community part of the advisory panel in selecting the funded studies?
A. One of the ways PCORI seeks to bring voices from across the healthcare community into our work is through PCORI advisory panels. To clarify, the advisory panels assist PCORI by prioritizing research topics for future funding announcements. As of February 2014, PCORI has four multi-stakeholder advisory panels and is in the process of establishing two more in April 2014. The existing advisory panels have representation from across the healthcare community; specifically, representatives from the payer community sit on the Advisory Panel on Assessment of Prevention, Diagnosis, and Treatment Options and the Advisory Panel on Improving Healthcare Systems. In fact, the chair and co-chair of the Advisory Panel on Improving Healthcare Systems both represent the payer community. Learn more about the PCORI Advisory Panels here.

PCORI also relies on multi-stakeholder merit review panels to participate in reviewing and selecting the proposals for funding and some of these multi-stakeholder panels have representation from the payer community. Learn more about the merit review process here.
Q. I see that there are currently no projects within PCORI related to dermatological conditions. Would the initiative welcome studies in this area, particularly for chronic conditions (like psoriasis)? What about recurrent skin complaints? Are these of interest?

A. PCORI supports studies that seek to answer questions important to patients and meaningfully involve patients and others across the healthcare community at all stages of the research process. We believe this includes studies of chronic conditions, and welcome applications that are designed to answer the questions patients have about these conditions and the choices they face.

PCORI does currently fund some projects in the area of dermatological conditions, including a recently announced Pipeline to Proposal Tier I award to the National Psoriasis Foundation to create a new online community for patients, researchers, and other stakeholders to develop ideas collaboratively to direct future psoriatic disease research and, eventually, to select comparative effectiveness questions related to psoriatic disease research that can be further studied by researchers. Learn more about this award, and other PCORI-funded projects here. Two of our recently approved awards for health data networks that will be part of PCORnet, the National Patient-Centered Clinical Research Network, include topics related to dermatological conditions: AR-PoWER and CENA.

Q. What rare and serious orphan disease states have PCORI’s attention for funding? For example, primary immunodeficiency, hereditary angioedema, or myasthenia gravis?

A. PCORI recognizes the importance of funding research that will provide patients and those who care for them the information to make informed healthcare decisions. This includes investing in research that addresses rare diseases. PCORI is actively addressing rare diseases through a number of programs and strategies.

- PCORI hosted a Rare Disease Roundtable of patients and stakeholders on September 11, 2013, to discuss the relevant issues of the rare diseases community that could be addressed by PCORI’s CER agenda and identify optimal strategies for engaging patients and other stakeholders in research on rare diseases. View the meeting agenda and presentations here.
- PCORI is in the process of developing an Advisory Panel on Rare Disease that will advise and provide recommendations to PCORI on the conduct of PCOR in rare disease. The group will also help to facilitate productive collaboration and engagement with the rare disease research community.
- PCORI has funded multiple comparative effectiveness research projects that address rare diseases. For example, a PCORI Pilot Project based at the Medical University of South Carolina involves developing a community partnership approach for advancing burden measurement in rare genetic conditions. Learn more about this award and other PCORI-funded projects here.
- PCORI has also invested in 11 Clinical Data Research Networks and 18 Patient-Powered Research Networks as part of the PCORnet: The National Patient-Centered Clinical Research Network. Over 50 percent of the Patient-Powered Research Networks address rare disease populations.
Q. Will PCORI be involved in the United States in helping to achieve data transparency, much like the current move underway in the European Union?

A. PCORI is committed to sharing all of the data generated from our funded studies and requires that a complete, cleaned, de-identified copy of the final data set used in conducting the final analyses be made available within nine months of the end of the final year of funding. In addition, to facilitate more efficient CER that could significantly increase the amount of information available to healthcare decision makers and the speed at which it is generated, PCORI has invested more than $100 million in the development of PCORnet: The National Patient-Centered Clinical Research Network.

PCORnet will be a large, highly representative, national network for conducting clinical outcomes research. PCORnet will foster a range of observational and experimental CER by establishing a resource of clinical data gathered in “real-time” and in “real-world” settings, such as clinics. Data will be collected and stored in standardized, interoperable formats under rigorous security protocols, and data sharing across the network will be accomplished using a variety of methods that ensure confidentiality by preventing patient identification. Learn more about this network here.

PCORnet was guided by discussions at the National Workshop to Advance the Use of Electronic Data in Patient-Centered Outcomes Research held in 2012. Nearly 100 experts and key decision makers attended the event, including officials from key agencies within the Department of Health and Human Services (HHS), experts on electronic health data, and representatives from patient and provider groups. During the meeting, participants shared their knowledge about current electronic data (eData) healthcare research initiatives and discussed what they viewed as the biggest challenges to advancing the use of electronic data in the conduct of PCOR. One main take-away from the meeting was a broad recognition among participants that, in order for an electronic clinical research network to truly advance PCOR, patients will need to be deeply involved in the creation and governance of the infrastructure. Read the meeting report here.

Q. How can we as pharma professionals and consumers become actively involved in the PCORI process? I’ve reviewed the options available for involvement on the PCORI website, and I’ve submitted my interest, but I’m curious if there will be an upcoming need for additional public involvement. This is an incredibly important initiative that deserves support.

A. PCORI offers a number of opportunities to get involved with our work:

- Contact us with any questions or comments about future PCORI engagement efforts. getinvolved@pcori.org
- Subscribe to our email list for alerts, updates, and opportunities to help guide our work. www.pcori.org/subscribe
- Learn more about past meetings and events.
http://www.pcori.org/events/?type=past

- View upcoming PCORI events, webinars, and opportunities to provide public comment. http://www.pcori.org/events/
- Apply to be a Reviewer of PCORI Funding Applications. http://www.pcori.org/get-involved/reviewers/
- Apply to serve as a PCORI Advisory Panelist. http://www.pcori.org/get-involved/advisory-panels/
- Learn about the Ambassadors program and how you can get involved. http://www.pcori.org/get-involved/pcori-ambassador-program/
- Discover PCORI funding opportunities. www.pcori.org/funding-opportunities

Q. What is PCORI’s viewpoint on the role of patient/HCP education as part of the overarching equation toward improved patient outcomes? Would this be an area of interest for research?
A. As part of the Eugene Washington PCORI Engagement Awards, we are announcing funding directed toward Training and Development Awards, which will cultivate a larger patient-centered research community through activities such as ones that will link interested patients, providers, and researchers to build research partnerships. Learn more about this funding opportunity here.

Q. Patient research partners are knowledgeable not only about disease but also research methods. But patients and caregivers often don’t know what they don’t know and are challenged to provide input that will make a difference for them. Would engagement awards include methods for developing health literacy of a particular patient/caregiver group?
A. As part of the Eugene Washington PCORI Engagement Awards, we are announcing funding directed toward Knowledge Awards, to build knowledge around how consumers of healthcare information receive and make use of PCOR/CER findings. Learn more about this funding opportunity here.

PCORI also funds a number of research projects that address health literacy and communication among patient/caregiver groups, for example, one project funded through the Targeted Funding Announcement on Asthma Treatment Options for African Americans and Hispanics/Latinos addresses Using Information Technology to Improve Access, Communication, and Asthma in African American and Hispanic /Latino Adults. Learn more about this award, and other PCORI-funded projects here.

Q. Do you envision accountable care organizations eventually using these PCOs in quality measures?
A. PCORI is interested in building evidence around innovative models of healthcare delivery and how they affect patients-centered outcomes. In fact, the Advisory Panel on Improving Healthcare Systems considered the topic “Accountable Care Organizations (ACOs) and patient-centered...
outcomes (PCOs): Compared to usual care, what are the effects of ACO care on (PCOs) among patients with chronic conditions?” for a targeted funding announcement. Learn more about this and other prioritized topics here.

Q. Is the current thinking that patients who are interested in engaging in clinical research-related activities, whether with PCORI, FDA, or industry, join some patient advocacy or patient-centered organization rather than trying to be a single entity?
A. Since our creation, PCORI has been committed to funding research that includes meaningful involvement of patients and other stakeholders in all steps of the process. PCORI values the unique perspective and value of engaging both individual patients and patient advocacy groups in the research process.

In response to frequent questions about what we mean by “engagement in research,” PCORI, with contributions from our Patient Engagement Advisory Panel, developed the Patient and Family Engagement Rubric to provide guidance to applicants, merit reviewers, awardees, and PCORI program staff, on meaningful engagement practices. Learn more about how PCORI views Engagement in Research here.

Answers from Multiple Panelists

Q. Is the patient-reported outcome coming from the patient or the physician assessment?
A. PCORI: PCORI is very interested in patient-reported outcomes and recently announced a $5 million initiative to support research that focuses on them. Learn more about this here.
A. FDA: For FDA, it means the outcome reported by patient.

Q. Do you think the diseases and population groups you are studying fairly represent the American population?
A. PCORI: PCORI funding announcements have always noted the importance of including studies of diverse populations with respect to age, gender, race, ethnicity, geography, and clinical status. In Section 3.2 of the revised PFAs, we have developed a more detailed list of “hard-to-reach” or lesser studied populations to guide our research and engagement efforts:

- Racial and ethnic minority groups
- Low-income groups
- Women
- Children (ages 0–17)
- Older adults (age 65 and older)
- Residents of rural areas
- Individuals with special healthcare needs, including individuals with disabilities
- Individuals with multiple chronic diseases
- Individuals with rare diseases
- Individuals whose genetic make-up affects their medical outcomes
- Patients with low health literacy/numeracy and limited English proficiency
• Lesbian, gay, bisexual, and transsexual (LGBT) persons.


Q. With regard to both planning the study and conducting the study, does convenience for the patient ever come up? Is there awareness that study visits don’t have to be done at the investigator site but can be done in the home setting, work, school, etc?

A. PCORI: PCORI outlines the PCOR Engagement Principles in the Patient and Family Engagement Rubric. These include:

Reciprocal Relationships: The roles and decision-making authority of all research partners, including patient partners, are clearly stated.

Co-Learning: The application includes plans to ensure that the patient partners will understand the research process and the researchers will understand patient-centeredness and patient engagement.

Partnership: Time and contributions of patient partners are valued and demonstrated in fair financial compensation, as well as reasonable and thoughtful time commitment requests. When the patient partners represent unique populations, the research team proposes to accommodate their cultural diversity and/or disability.

Trust, Transparency, and Honesty: Major decisions are made inclusively, and information is shared readily with all research partners. Patient partners and research partners express commitment to open and honest communication with one another. The study team commits to communicate the study’s findings back to the study community in a meaningful and usable way.

A. FDA: We are seeing more of this—use of smartphones, for example.

Q. Do patient reviewers and medical professional reviewers receive the same compensation for their efforts?

A. PCORI: All PCORI merit reviewers are compensated equally based on their term of service. Ad hoc reviewers will receive $50 for each application reviewed and will receive a $200 stipend for each day of the in-person panel meeting. In recognition of their long-term commitment to PCORI, Standing Panelists will receive $75 for each application reviewed and a $200 stipend for each day of the in-person panel meeting. Additionally, Standing Panelists who participate in at least four review cycles during their 2-year term will receive an additional one-time $200 stipend.

Q. How is patient-reported outcome research differentiated from real-world patient data utilization, which is strictly observational in nature, and is there a trend for real-world data now to be part of the regulatory approval process for a product?
A. PCORI: To help reduce the burden and avoid unexpected outcomes like those that you mention, PCORI is committed to helping to build a PCOR-ready community and has developed a variety of curricula to enrich capacity building for the non-research partner. Also, PCORI has recently launched a new funding mechanism called the Engagement Awards that include Training and Development Awards to help address some of the shift from experts to non-research partners.

A. FDA: Not for regulatory approval, but we use observational data, e.g., Mini-Sentinel, to address questions that are hard to do trials on.

Q. How are FDA and PCORI positioning themselves to grapple with unexpected outcomes that may unfold as a result of the shift in some of the burden from experts to participants in design, development, and dissemination of studies?
A. FDA: We welcome the additional information and insights.

Q. What role do you believe digital technology plays in enabling patient engagement and patient outcomes?
A. PCORI: PCORI recognizes the promise of telehealth and mHealth technology in improving healthcare delivery and potentially patient-centered outcomes. PCORI currently funds studies investigating the benefits of telehealth, including a study that addresses Using Telehealth to Deliver Developmental, Behavioral, and Mental Health Services in Primary Care Settings for Children in Underserved Areas. Learn more about this award and other PCORI funded projects here.

A. FDA: I think it will be crucial in the future.

Responses included below are paraphrased. For complete answers, please refer the webinar archive.

Q. FDA: What methodology was used to select the diseases targeted by the Patient-Focused Drug Development meetings?
A. Janet Woodcock: The FDA solicited feedback from its reviewers first. A Federal Register notice was sent out that resulted in massive amounts of content, which led to the public meetings and many comments for the docket. The decision criteria were presented on the slide, but to be honest, the final selection of the diseases was somewhat arbitrary. FDA is aware it is not covering all diseases. There are many diseases where this work needs to be done, and the FDA doesn’t think it is the only one that should be doing this work. It would be ideal to develop a paradigm on how to do this so that others can then carry on the work with the multitude of diseases that exist. The FDA has limited resources and won’t be able to get through all diseases where there is need. We would like to take a range of diseases and conditions (consider prevalence, treatment options, severity, etc.) and have these Patient-Focused Drug Development meetings, get people mutually learning how to elicit this information, synthesize it and then utilize it.
**Q. FDA: Will output from the patient-focused meetings be used to develop new PROs?**

**A. Janet Woodcock:** Yes, output from patient-focused meetings can be used as part of the PRO process. Other groups are developing the PROs, and they have patient advocacy groups involved in their processes. However, these meetings will be a valuable resource on the domains that are important to patients and will make sure those domains are included in any outcomes assessments that we may do. This was the absolute intent of the meeting efforts.

**Q. FDA: Can a patient group propose a PRO measure qualification? If so, how?**

**A. Janet Woodcock:** Yes, and the process is all laid out in the Guidance. It’s advised that they find other partners, for example, industry groups and/or academics working in the disease area, and then follow the process in the Guidance on how to engage the FDA.

**Q. PCORI: Is PCORI working with FDA to develop drug development tools for validation under the recent FDA guidance?**

**A. Anne C. Beal:** PCORI is focused on comparative clinical effectiveness and doesn’t work in the area of drug development. That said, some of the work that the FDA is doing around models of patient engagement provides fertile ground for collaboration; PCORI can not only learn from FDA but also share our own experience in patient engagement.

**A. Freda Lewis-Hall:** Joining some of the outcomes research to the questions around unmet needs is essential as a part of “mapping the gap” between what we have available and can best possibly deliver to patients and what we might want to make available and what the characteristics of these advancements would look like on behalf of meeting patients’ needs. The ability to take information from what is provided within PCORI’s work on patient engagement and patient outcomes, and to feed that back into the work that industry does and the FDA assesses, is an important bit in the process work we have for the future.

**Q. PCORI: Could a patient group potentially work through the Pipeline to Proposals pathway to develop a PRO that could then go through the FDA qualification process?**

**A. Susan Sheridan:** Ultimately, the goal of the pipeline is to create partnerships between patients and researchers and others as they go through the pipeline to become “PCOR ready.” That doesn’t necessarily mean submitting an application only to PCORI, but rather to other funding institutions as well. As long as the proposal is around a comparative effectiveness research question that is something we anticipate people will go through the pipeline for and end up in a potentially appropriate funding institution, that’s going to benefit the comparative effectiveness research.

**A. Anne C. Beal:** While I gave an example of the Pipeline to Proposals as one type of engagement award, the proposed project would be part of the wrap-around concept PCORI is talking about. PRO development is not only potentially appropriate in the pathway Sue Sheridan mapped out, but also appropriate as a different type of engagement award. I invite all to come to PCORI.org to learn more about the different kinds of awards. The proposed project would be well aligned.
Q. All: Do patient engagement activities and meetings include families and caregivers of patients (other than the physician)?

A. Susan Sheridan: Absolutely! PCORI focuses on patients, caregivers, and family as really vital members of the research team. A significant amount of PCORI’s portfolio includes caregivers, and its workshops and round tables have a high percentage of caregivers who participate.

A. Freda Lewis-Hall: I second that from the industry perspective. As we convene groups to get critical feedback and information, caregivers are becoming more and more engaged. Particularly over the last several years, we have become more and more aware of the critical nature of the feedback that caregivers themselves can provide. This is an interesting evolution; for instance, parents have been able to give feedback on children’s needs, but now it’s the children and the parents. Also, for individuals for whom self-care is still available, caregivers may still be engaged, even if they aren’t giving 100 percent of the care. They have the opportunity to observe and support and provide important feedback.

Q. All: Are there any U.S. guidelines to stimulate the publication of best practices in engaging patients actively in clinical research?

A. Anne C. Beal: PCORI points to the rubric that Sue Sheridan discussed. It gives the opportunity to think systematically about ways to engage patients in research. PCORI has been deliberate in not talking about best practices but about “promising practices.” The first stage was the creation of the rubric to think systematically about patient engagement, and the second is to develop a mechanism to operationalize the components of the rubric. The third is the evaluation and assessment of different components of the rubric to determine how different practices are associated with different outcomes. PCORI is going to be publishing and sharing work from the rubric—communication around best practices is vitally important. It’s not enough just to gather a compendium of best practices; we must hold ourselves accountable for outcomes. Once the evaluation work has been done, we’ll have the evidence to make statements as to what are best practices.

A. Freda Lewis-Hall: We should accept this question as a challenge for ourselves to think of ways that we may be able to “turbo-charge” the sharing we are doing. The PCORI platform gives us opportunities to share, but it is time for those of us working in this space to get together and drive a broader platform of sharing to further stimulate this work.

Q. PCORI: How does PCORI envision partnering with industry? What types of grant proposals submitted by industry might be funded by PCORI?

A. Anne C. Beal: PCORI is interested in partnering with industry at every stage of the work we are doing and considers industry a stakeholder. The advisory panel and the merit review are examples of where we try to make sure industry is represented. In projects both on the research side and on the engagement awards, PCORI is very clear that opportunities are not limited to nonprofit and academic research institutes. We are very open to hearing from industry on ideas, thoughts, and
proposals that are aligned with our work and are a priority for us. Consider PCORI as a potential partner or source for trying to test new ideas that are related to this kind of work.

A. Susan Sheridan: PCORI has just launched a kick-off event on infrastructure, on the Patient Powered Research Network (PPRN) and the Clinical Data Research Network (CDRN), where partnership with industry is front and center. It’s an important collaboration with industry—Janet Woodcock was the FDA representative, and device manufacturers are a part of this. Last but not least, PCORI has just launched a third committee that works to support collaboration and cooperative agreements and work, and that will be a part of the focus with industry as a potential partner.

Q. PCORI: What routes will PCORI use to share study results with the participating patients?
A. Susan Sheridan: PCORI is looking at a whole model for this. When there are research findings to share, we are committed to providing it back to the study participants. We are working through the Principal Investigators to create feedback loops. We also want several layers for dissemination that could be driven by patient organizations working with the disease, and we’ll request them to share with their partners. These are opportunities to split the paradigm for dissemination, to make sure the message is consistently reaching every avenue that it can. As part of the engagement awards, there will be dissemination awards to facilitate the dissemination of information to all the parties.

Q. PCORI: For rarest diseases, has PCORI considered doing international work such as in Canada or Europe? Is any of the research global, specifically in developing countries?
A. Susan Sheridan: PCORI does consider some international funding, but the criteria are very specific: Any investment that is made internationally must have a direct benefit back to the United States and its U.S. residents. With that said, there is much work globally around patient engagement. PCORI does have the involvement of international rare diseases patients through the Patient Powered Research Network. The data is helping to populate international registries to understand these rare diseases better.

Globally, there is a lot of outreach to PCORI on patient engagement activities. There is interest from WHO, ISQAI, and other interest groups that want to mirror PCORI’s patient engagement activities. We can learn from each other. There is opportunity outside of PCORI as well to help move and transform what we are doing to engage patients.