Advisory Panel on Patient Engagement Meeting Summary

April 2015

Overview

On January 13 and 14, 2015, the PCORI Advisory Panel on Patient Engagement (PE) held its sixth meeting in Arlington, Virginia.

The 22-member panel includes patients, caregivers, patient advocates, industry, researchers, clinicians, and policy makers. PCORI staff also joined the meeting, and some panel members joined by teleconference.

During the course of the two-day meeting, PCORI staff and guests presented perspectives on key patient engagement topics, including patient representation and the role of other stakeholders in PCORI-funded research. These presentations were followed by discussion by the group as a whole, as well as in breakout groups. In addition, new data on patient engagement in research was presented, including how patient involvement has led to different research questions and revised research plans to more nearly reflect concerns of patients who have the diseases or conditions under investigation. Committee members also received updates on the Ambassador Program and Pipeline to Proposal Award program and how these relate to patient engagement. Compensation policies previously discussed were reviewed and approved, and the group started an initial discussion of how the patient perspective should be integrated into policies on privacy, data and safety monitoring, and institutional review boards.

Related Information

- About this Advisory Panel
- Meeting Details and Materials
- Advisory Panel on Patient Engagement October 2014 Meeting
- PCORI Engagement Program

The Patient-Centered Outcomes Research Institute (PCORI) is an independent organization created to help people make informed healthcare decisions.

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Perspectives on Meaningful Patient Representation in Research

Several presenters discussed the roles and characteristics of patient representatives and of patient, caregiver, and consumer advocacy organizations in patient-centered outcomes research. After a series of presentations looking at the topic from several different perspectives, the panel then broke into small groups to develop key considerations and report back to the committee as a whole.

Authentic Patient-Centered Roles and Capabilities

Panel member Perry Cohen, PhD, prepared slides about the Authentic Patient-Centered Roles and Capabilities. PCORI's stated goal of "research done differently" means patients' interests come first in the design and implementation of research projects. This means patients must be involved in healthcare improvement efforts, including research design and implementation. Perry pointed out that patients who have actually experienced illness have different perspectives than other stakeholders in the research process, and patients with terminal or chronic illness have different preferences, interests, and tolerances for research than other stakeholders in a research project.

Perry identified several qualities that patient representatives need to speak effectively about research interests: training (provided by researchers or by PCORI), linkage to community (through patient organizations, social media, or other means), trust, and authorization. PCORI is currently drafting a training curriculum that can help facilitate the involvement of patient partners in research. Perry also recommended the creation of "institutes" to define and maintain patients' interests for different patient populations and the inclusion of patient representatives in all policy decisions about research and treatment of illness.

Who Is the Authentic Patient in a Patient-Centered Research Network?

Jaye Bea Smalley, MPA, Engagement Officer, PCORI and Rebekah Angove, PhD, Engagement Director, LACDRN gave a brief overview of PCORnet, The National Patient-Centered Clinical Research Network, a national network of 29 health data networks conducting clinical effectiveness research (CER), and discussed the issue of defining an authentic patient representative.

PCORnet's Patient and Consumer Engagement Task Force (PCE TF) is currently developing the patient engagement policy for PCORnet. As the PCE TF embarked on developing this policy, issues have come up around who is truly in a position to represent the views of patients. At times, we see patient representatives that have additional roles in the network.

Patients and other representatives are involved with governance of the networks, developing policies for data use and data sharing, setting research agendas, and co-developing privacy and consent policies. Networks need many different types of talent and expertise onboard, and patients may play multiple roles, but the most important consideration is making sure that the patients' voice is represented by patients with meaningful lived experience who truly represent patients' interests.

To help illustrate the questions and conflicts that might arise, the presenters offered several case studies informed by real-life situations that have come up in the PCORnet network. These case studies help identify key considerations in describing and selecting authentic patient representatives, including whether employees of the institution can act as patient representatives.

Committee member discussion following the presentation brought up the following points:

- It's important to consider if the patient representative or patient advocate reflects the patient population in that geographic area. If both patient reps have PhDs, they may not reflect the demographics of the typical patient in that area.
- Clinicians who have an interest in the role of patients, people employed by the system, and people with research or technology experience can contribute to research planning and implementation, but they should be in a different category from the patient representative. Patient representatives should bring the patients' voice to the table and represent the patient and not the institution.
- Conflicts of interest should be adequately exposed and not result in unfair advantage or financial gain.

FDA's Patient Advisory Group

Kimberly McCleary, Director of Strategic Initiatives, *FasterCures,* presented information about how the Food and Drug Administration involves patients and consumers.

The most typical way patient perspective gets included in the FDA process is through representation on the advisory committees that review the evidence and make recommendations on whether the drug is safe and effective for the intended patient population. Those committees usually have 12 to 20 representatives, including one patient and one consumer who represent those points of view.

In many healthcare circles, people use the words "patient" and "consumer" interchangeably, but patients and consumers can have different points of view, with consumers perhaps emphasizing safety over speed and patients tending to want to move medications to the market faster.

There is now an effort at the agency to include patient representatives earlier in the process and not wait until all the evidence is in. FDA is also grappling with issues of conflict of interest and representativeness.

The Center for Drug Evaluation and Research (CDER) has a current initiative called "patient-centered drug development," which will bring together patients on a disease-by-disease basis outside of the discussion of a particular product. Many are interested in empowering the FDA with legislative tools to continue this work on a broader basis. One of the issues in these conversations is how best to involve the public, from patients with lived experience with disease or illness, to members of the healthy public.

Small Group Discussion

After the initial presentations, the panel broke into three groups (patients and caregivers, researchers and investigators, and other stakeholders) to discuss key considerations for patient representation in research.

Patient representatives: qualifications and responsibilities

- Patients should know enough about the condition to speak on behalf of those who have the condition. The person should be empowered, prepared, and representational. Having just one patient involved limits the representativeness. It may take multiple perspectives to represent a broader base of patients.
- Need to be clear about roles and responsibilities and make sure the representatives' expertise fits with those roles and responsibilities. Dual roles can lead to conflict of interest for a patient representative.

Distinction between patients and patient advocacy groups

- Organizations claim to speak for groups of patients, but they should specify who those patients are that they represent. Organizations can also reach out into communities to find patient representatives. Patient organizations may be more helpful in the dissemination phase than in the formulation of the research agenda.
- Dual roles have value—people who wear multiple hats can offer perspective on different levels. Someone who can talk about research methodology and also has lived experience is helpful, but they are not a substitute for meaningful patient engagement and should not be considered the patient representative.

Researchers'/networks' obligations when seeking patient representatives

- Researchers may need tools and training to most effectively interact with patient representatives.
- Networks should dig deep for patients. There are enough patients out there—even with rare diseases—that you don't have to keep going to the same people. PCORI could help spread this message.

Suggestions for PCORI

- PCORI should provide training to help patients learn how to relate their personal experience to different contexts. Training can help patients share their experience and life journey more effectively.
- PCORI is further along than others in incorporating patient representation in research. Other funders may benefit from PCORI success stories—and troubleshooting stories too. (The evaluation group is working to extract these stories and share them.)
- Instead of developing yet another set of recommendations or a new document on dual roles and finding authentic patient representatives, can the points about patient representatives be added as hyperlinks in the already existing rubric?
- PCORI should consider ensuring that patients and caregivers are involved in research planning and implementation in a meaningful way. Developing clear guidelines will make it easier for other organizations to follow.

- Researchers have commented on how important it was to get the patient perspective, which helped them figure out the best way to carry out their research. The more opportunities they have to do this, the more they will learn from that.

The Role of Other Stakeholders in PCORI

Susan Hildebrandt, MA (Director, Stakeholder Engagement) and Greg Martin (Deputy Director) spoke about their efforts to reach out to stakeholders other than patients (i.e., clinicians, hospitals and health systems, training institutions, policy makers, industry representatives, payers, and purchasers). In order to determine how best to engage these stakeholders, they met with representatives from different groups beginning in 2012 at a host of venues to seek their feedback. Resulting outreach efforts have included one-on-one meetings, regional workshops, issue-oriented roundtables, public presentations, award notification, webinars, and requests to join Advisory Panels and serve as merit reviewers. PCORI staff also have made regular visits to the Hill to educate congressional staffers about PCORI's efforts. PCORI also reaches out to non-patient stakeholders in the states. One way this is done is through the Medicaid Medical Directors Network, which is housed within the National Association of Medicaid Directors.

- Overall, PCORI Stakeholder Engagement team staff has had contact with about 6,500 different non-patient stakeholders from 1/1/2013 to 9/30/2014.
- Nearly 2,800 have been researchers, but representatives from all other groups also have been involved.
- Traditionally, payers and purchasers have always been the more difficult to connect with and attract to PCORI events.

Staff from the Stakeholder Engagement team is currently putting together an education plan for the 114th Congress so that Senators and Representatives have clear information on PCORI's funded research and priorities.

Ways of Engaging—Engagement Activity Tool (WE-ENACT)

We-ENACT is a self-report tool that PIs, patients, and other stakeholder partners complete at the baseline of the project, then at annual intervals. Laura Forsythe, PhD, MPH, Senior Program Officer for Research Integration and Evaluation and Kristen Konopka, MPH, Senior Program Associate for Stakeholder Engagement reported on preliminary results and discussed the implications and opportunities to improve.

The data presented included results from 58 PIs or designees and 75 patient or stakeholder partners on 29 projects. Partner data lags behind researchers because researchers were asked to identify partners so we could invite them to participate. (This step will not be necessary in the future because PCORI is now collecting partner data and can contact partners directly.) Data collection is ongoing, so additional data will soon be available from researchers, patients, and stakeholders from more projects. About a third of the stakeholders were patients/consumers/caregivers, and another 27 percent were advocacy organizations. Clinicians made up 19 percent of the stakeholders.

Researchers were asked what types of stakeholders they involved in their projects. Patients, caregivers, advocacy organizations, clinicians, and hospitals were all well represented, but payers and purchasers were included in very few projects (8 percent involved payers, 4 percent involved purchasers). Results included:

- 84 percent of researchers said they had an advisory group for their project; 74 percent had a patient/stakeholder on the research team. Sixty percent had a patient/stakeholder co-investigator.
- 49 of the 58 researchers surveyed said they had stakeholders involved in the planning of the study.
- About 75 percent of the researchers and 50 percent of stakeholders indicated that stakeholders had at least a moderate amount of influence on identifying research questions. Three percent of stakeholders said they didn't know how they influenced the research questions.
- 35 percent of researchers and 50 percent of stakeholders expressed satisfaction with the level of training and support for research engagement.

Lack of time and lack of knowledge about engagement were the biggest challenges for both researchers and stakeholders. Most reported that they were able to either completely or partially resolve this lack of researcher and stakeholder knowledge. Time issues were more difficult to resolve.

Learning from Applicants and Reviewers about Engagement Resources

Sana N. Vieuz, MPH, Program Associate, Research Integration and Evaluation presented results from the closed-end questions in the Applicant and Reviewer surveys conducted by PCORI after the merit review process. The applicant survey findings showed that the rubric and engagement plans were helpful, but there was room for improvement. Most applicants established stakeholder partnerships before applying but not very long before. Applicants requested more resources to help put together proposals.

Preliminary feedback shows that the patient engagement rubric has increased the quality of applications for PCORI funding and is improving the quality of research questions. Other organizations have asked PCORI to share the patient engagement rubric, and plans are underway for that. PEAP members are welcome to share the data.

Ambassador Program—Update

Aingyea Kellom, MPA, Program Associate, Patient Engagement, and Suzanne Schrandt, JD, Deputy Director, Patient Engagement gave an update on the Ambassadors program, which provides training to patient partners and connects them to patient-centered outcomes research (PCOR) opportunities both inside and outside of PCORI. To date, 99 individuals have completed the training, including 82 individuals and 17 organizational representatives. In the beginning, most of the people in the training were researchers, but now more consumers, advocacy groups, and patients are joining in. The program would like to increase participation from the western portion of the country and from minority populations.

- An individual Ambassador has a personal interest in PCOR and an organizational Ambassador represents a health and healthcare entity that is interested in PCOR.
- All Ambassadors are asked to commit to the PCOR principles of reciprocal relationships, partnership, co-learning, trust, transparency, and honesty.

The program is designed to benefit both PCORI and the Ambassadors. Ambassadors can participate in an active calendar of calls and webinars, PCORnet's Grand Rounds, and PCORI surveys. Social media may offer a way for Ambassadors to connect with each other, share resources, and help disseminate results from PCORI research projects. Finding the right platform and content for social media is a challenge. PCORI is looking at various ideas to increase the value of the program to Ambassadors, including CME or other recognition for participation.

The panel members, some of whom are in the Ambassadors program, brought up some concerns with the current format:

- The online training is difficult for many to complete.
- Many find the social media platform difficult or inconvenient to use and therefore are not kept up to date on the program's offerings.

Pipeline to Proposals (P2P) Awards—Update

Courtney Clyatt, MPH, Senior Program Associate on Patient Engagement gave an update on the Pipeline to Proposals (P2P) program, which expanded in November 2014 after a successful pilot phase. P2P fits in before the research process in the pre-planning phase. The goals of the program are to strengthen relationships between researchers, patients, and stakeholders, and create community partnerships to formulate patient-centered research questions and submit proposals for PCORI funding. The program is currently reviewing up to 298 Letters of Intent (LOIs) for this funding opportunity. Up to $4 million in awards will be given in fiscal year 2015. First-year grants are $15,000. The pilot phase of the project resulted in some promising practices in patient engagement, and program managers are hoping to produce a publication in the near future about some of those lessons learned.

PCORI has produced three videos showing three different P2P programs:

- Katie Wilkes, a melanoma survivor, was funded for a project (Solsurvivors) in which patients and researchers worked together to create a website and other information about melanoma.
- Suzanne Pak from Cornerstone Medical Services worked with Asian Americans to educate about stomach cancer in Washington state.
- Dr. Wen at Loma Linda University redesigned his research into post-sepsis syndrome in response to patient input.

Efforts are being made to reach out to junior researchers and community health organizations to talk about more opportunities for non-traditional researchers.

Suggestions from the panel included:

- Use the videos to introduce the program—they give a good overview of what's possible.
- Offer prospective applicants help with the LOI process. Can Ambassadors or past awardees help with that?

Compensation Framework—Finalization and Approval

The Compensation Committee has been focusing on whether and how PCORI should articulate restrictions or requirements for compensation to patient representatives on research teams. The committee previously developed draft language and sought PEAP endorsement of that language before vetting within PCORI. With no serious objections to the framework, it will be moved to the next level of review.

Privacy/Data and Safety Monitoring Board/Institutional Review Board

Suzanne Schrandt, JD, Deputy Director, Patient Engagement, gave an update on some of the activities in ethics and human subjects work, starting with background on the different entities.

Institutional Review Boards (IRBs) review and approve research projects. Anything funded by PCORI that has human subjects has to have IRB approval. IRBs are particularly interested in the safety of human patients (subjects) involved in the research. PCORI-funded research causes some confusion because patients are involved not only as subjects but as research partners. PCORI is looking at ways to provide guidance to help facilitate understanding of this new type of research. One idea is to produce a white paper on the topic.

Data Safety Monitoring Boards (DSMBs) look at the data coming out of the study as it is conducted. They monitor data for safety and privacy concerns and can actually stop a research project if necessary. In keeping with PCORI's mission to increase patient involvement in research, PCORI is exploring the potential roles for patient partners to serve on DSMBs.

Privacy and confidentiality: Vocal and visible patients involved in the research may feel compelled to share information about themselves. PCORI wants to respect patient autonomy and promote the work of patient partners while respecting and protecting their privacy. Guidelines on what is appropriate personal information for patients to reveal/conceal may be helpful.

PCORnet has recently formed a task force looking at Patient-Centered IRBs and may be reaching out to PEAP members to volunteer on working groups or to contribute input on this area.

Suggestions from the panel included:

- Make available training for patient representatives and encourage boards to avoid jargon and spell out acronyms so patients can fully participate. These changes may help other board members too.
- Centralized IRBs are becoming more popular in drug development studies, and NIH is embracing them so there may be an opportunity for working together.

Wrap-up

PCORI staff thanked all of the panel members for their participation, with a special thank-you to those cycling off the panel, including Saul Weingart, MD, PhD; Stephen Arcona, MA, PhD; Paul Arthur, MS, MOT, ORT/L; Amy Gibson, BSN, RN, MS ; Leana Wen, MD; Melanie A. Nix, MBA; Laurel J. Pracht, BS; and

Lygeia Ricciardi, EdM. The next meeting will welcome six new members to the panel. PCORI staff are putting together a booklet of information to help orient the new members to PCORI, the panel, and their work. A post-event survey will be sent to all panel members, and the spring meeting is scheduled for June 1 and 2, 2015.