Involving patients in research: what have we done and how did we do it? Lessons learned from the Patient-Centered Outcomes Research Institute (PCORI).

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Oslo
3 November 2014
Overview

- Overview of PCORI and patient-centeredness

- Patient engagement in research:
  - The Patient and Family Engagement Rubric

- Patient engagement in building a data research network:
  - PCORnet

- Building the evidence for patient engagement in research:
  - Model for evaluating engagement in research
  - WE-ENACT (Ways of Engaging - ENgagement ACtivity Tool) and Net-ENACT (PCORnet ENgagement ACtivity Tool)
Engagement!

- You can engage us better down here!
Why PCORI?

• Research has not answered many questions patients face
  ▪ People want to know which treatment is right for them
  ▪ Patients need information they can understand and use
PCORI’s Mission

PCORI helps people make informed health care decisions, and improves health care delivery and outcomes, by producing and promoting high integrity, evidence-based information that comes from research guided by patients, caregivers and the broader health care community.
Who Are PCORI’s Stakeholders?
PCORI Engages Patients and Other Stakeholders at Every Step

- **Planning, Conduct and Dissemination**
- **Evaluation**
- **Merit Review**
- **Topic Selection and Research Prioritization**
Patient engagement in the design and conduct of research offers a greater likelihood of:

- influencing research to be patient centered, useful, and relevant
- establishing trust and a sense of legitimacy in its findings
- successful use and uptake of research results by the patient community.
Why Engage Patients in Research?

Patients contribute:

- “Lived experience” of condition or disease
- Research questions that are important to patients
- Outcomes important to patients such as quality of life
- Real world input on risk tolerance of interventions
- Power of data (new currency)
- Focus on patient-centeredness, ethics, safety, and urgency
- Connections to other patients like them, which facilitates recruitment and dissemination of research findings
Patient-Centeredness vs. Patient Engagement

- **Patient-Centeredness** is a component of what PCOR is looking for in research applications
  - Does the project aim to answer questions or examine outcomes that matter to patients within the context of patient preferences?
  - Research questions and outcomes should reflect what is important to patients and caregivers

- **Patient engagement** is about having patients as partners in research as opposed to merely subjects
  - Active engagement between scientists, patients, and stakeholders
  - Community, patient, and caregiver involvement already in existence or a well-thought out plan
What does patient engagement look like in the planning, conduct and dissemination of research?

The Patient and Family Engagement Rubric
Why develop a rubric?

- The rubric is a response to frequent questions from the patient and research communities asking what we mean by “engagement in research.”

What is the rubric?

- The rubric is a framework that provides a variety of options for incorporating engagement, where relevant, into the research process.

How will the rubric be used?

- The rubric will be used as a guide for applicants, merit reviewers, awardees and Engagement Officers.
Rubric: Patient and Family Engagement in Patient-Centered Outcomes Research (PCOR)

- Planning the Study
- Conducting the Study
- Disseminating the Study Results
- PCOR Engagement Principles
Formulating Research Questions and Study Design

Patient partners participate in:

- Identifying the topic and developing the research question to be studied.
- Creating the intervention to be studied (if applicable) and identifying comparators.
- Identifying the goals or outcomes of the interventions to be studied.
- Defining essential characteristics of study participants.
- Other study design and preparation.

Patient partners are involved in:

- Identifying the topic and developing the research question to be studied.
- Creating the intervention to be studied (if applicable) and identifying comparators.
- Identifying the goals or outcomes of the interventions to be studied.
- Defining essential characteristics of study participants.
- Other study design and preparation.

Examples:

- Epilepsy study: the patients and parents of patients with epilepsy pose the question: which anti-epileptic drugs best preserve sufficient cognition to go to work or school and function normally, while still preventing seizures adequately?
- Asthma study: the patients and parents help create the paper asthma tracker tool being compared to the e-asthma tracker tool.
- Cancer study: patient partners determine that all women with breast cancer would be eligible versus only women who had completed active treatment. How can you demonstrate this in your proposal?

- Provide letters of support from patient partners that clearly describe the origin of the study topic, the role of the patient partners in defining the question, outcomes, comparators, and goals/outcomes, etc.
- Include the patient partners in all relevant sections of the application, such as the biosketches, the budget, and the dissemination and implementation assessment.
- Avoid relying entirely on patient partners who have dual roles on the project, e.g., relying on stakeholders or researchers who also happen to be patients. Including one or more patient partners who have no other role on the project is important.
Patient Engagement in Developing the Research Question
Elements of the Rubric

- Planning the Study
- Conducting the Study
- Disseminating the Study Results
- PCOR Engagement Principles
### Rubric: Conducting the Study

#### Conducting the Study

<table>
<thead>
<tr>
<th>Participating in and monitoring the conduct of the project</th>
<th>Patient partners participate in the recruitment of and data collection from the study participants, when appropriate.</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patient partners participate in all potential evaluation activities of patients.</td>
<td>The research team, including patient partners, participates in all potential evaluation activities of patients.</td>
</tr>
<tr>
<td>Patient partners participate in and monitor the conduct of the research project.</td>
<td>Patient partners participate in and monitor the conduct of the research project.</td>
</tr>
<tr>
<td>The research team includes patient partners in all potential evaluation activities.</td>
<td>Patient partners are truly involved in the research and if they think that their involvement is contributing to the research.</td>
</tr>
</tbody>
</table>

- Include in your application a plan for “check-ins” with patient partners to monitor their perceptions of the extent to which a) they are meaningfully involved in the study and b) their participation is contributing to the study.
- Also include a plan for “check-ins” with the other research team members to monitor their perceptions of the extent to which a) patient partners are meaningfully involved in the study and b) their participation is contributing to the study.

- Clearly articulate in the application the roles of the patient partners in interacting with study participants (e.g., recruiting participants, conducting interviews, leading focus groups, etc.).
- Provide letters of support from patient partners that clearly describe the role of the patient partners in interacting with study participants, when appropriate.
- The PI regularly asks the patient partners if they feel that they are truly involved in the research and if they think that their involvement is contributing to the research.
- Patient advocates assist with recruitment through their patient networks.
- Patient partners are trained to go out into the community to recruit study participants and to conduct interviews with them.
- Patient partners advise researchers to substitute the term, “emotional well-being,” for the term, “mental health,” to enhance the recruitment of study participants.
- Include in your application a plan for “check-ins” with the other research team members to monitor their perceptions of the extent to which a) patient partners are meaningfully involved in the study and b) their participation is contributing to the study.
Elements of the Rubric

Planning the Study

Conducting the Study

Disseminating the Study Results

PCOR Engagement Principles
### Rubric: Disseminating the Study Results

**Helping to plan the dissemination of the study’s results.**

Patient partners are involved in plans for disseminating the study’s findings to patient, stakeholder, and research audiences so that the findings are communicated in understandable, usable ways.

<table>
<thead>
<tr>
<th>Examples</th>
</tr>
</thead>
<tbody>
<tr>
<td>Chronic pain study: patient partners co-author manuscripts, present at scientific and lay conferences, and share study findings through their networks.</td>
</tr>
<tr>
<td>Cardiac study: a Patient Dissemination Board is helping to craft the dissemination plan and advise the research team on how to best share study findings.</td>
</tr>
</tbody>
</table>

How can you demonstrate this in your proposal?

- Provide letters of support from patient partners that clearly describe the role of the patient partners in planning the dissemination of the study’s results.
- In the application, clearly identify the role of patient partners in planning the dissemination of the study’s findings.
Rubric: Disseminating the Study Results

Examples

- Chronic pain study: patient partners co-author manuscripts, present at scientific and lay conferences, and share study findings through their networks.

- Cardiac study: a Patient Dissemination Board is helping to craft the dissemination plan and advise the research team on how to best share study findings.
Elements of the Rubric

1. Planning the Study
2. Conducting the Study
3. Disseminating the Study Results
4. PCOR Engagement Principles
Rubric: PCOR Engagement Principles

<table>
<thead>
<tr>
<th>Reciprocal Relationships</th>
</tr>
</thead>
<tbody>
<tr>
<td>The roles and decision-making authority of all research partners, including patient partners, are clearly stated.</td>
</tr>
</tbody>
</table>

Examples:

- Many applications state that patient partners are co-investigators, and that decisions about the study are made by consensus among all the research project partners.
- Many applications describe patient partners as key personnel, and their biosketches illustrate how the skills and experiences of the patient partners prepare them to function effectively in this role.
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.
Rubric: PCOR Engagement Principles

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.

Examples:
- Compensation for patient partners is included in the budget at market rates for consultants.
- In a study focused on a Latina population, several members of the research team are Hispanic and fluent in Spanish.
- In a project with a patient partner with a disability, the research team selects sites for team meetings that are accessible.

Partnership
Rubric: PCOR Engagement Principles

Trust, Transparency, 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.
What does patient engagement look like in building a data research network?
The **goal** of PCORI’s National Patient-Centered Clinical Research Network Program is to **improve the nation’s capacity to conduct clinical research** more efficiently, by creating a large, highly representative, national patient-centered clinical research network with a focus on conducting CER – both randomized and observational.

The **vision** is to support a learning US healthcare system, which would allow for **large-scale research** to be conducted with **enhanced accuracy and efficiency** within real-world care delivery systems.
11 Clinical Data Research Networks and 18 Patient Powered Research Networks
Patient and Family Engagement in Data Network Development (PCORnet)

Enrollment and diversity
- Increasing size of the network
- Increasing the diversity of the network
- Retention of network members

Governance
- The development of the network governance structure, roles and responsibilities
- Development of procedures, bylaws and policies for the network

Data collection
- The development of data collection tools
- Identification of Patient Reported Outcomes (PROs) for inclusion in database

Data sharing, privacy and consent
- The development of consent processes and policies
- Development of data sharing agreements
- Development of privacy policies
Patients are Actively Shaping the Network

- Governance of the Network
- Cohort characterization
- Identifying important considerations for consent
- Determining plans for returning results to patients
- Identifying opportunities to inform patients about research in the care continuum

It's time for patients to have a voice in national healthcare conversations.

You bring a valuable perspective to the national conversation surrounding healthcare delivery and research.

Share that perspective and help shape future programs by joining OCHIN's Patient Engagement Panel, and contribute your thoughts on:
- Research questions
- Changes to healthcare processes and their impact on patients
- Project communication to the general public
- Patient recruitment for studies
- Health technology

Details:
- You will receive gift cards for time spent during MEP meetings.
- The Patient Engagement Panel meets monthly for one hour and focuses on primary care research and the patient perspective.

Interested?
Email or call Nate Warren, OCHIN Patient Engagement Coordinator, for more information about getting involved today: warrenn@ochin.org or 503.343.2568.
Patient Powered Research Networks represent a number of conditions…

<table>
<thead>
<tr>
<th>Organization</th>
<th>Condition</th>
<th>Pop</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Accelerated Cure Project for Multiple Sclerosis</strong></td>
<td>Multiple Sclerosis</td>
<td>20,000</td>
</tr>
<tr>
<td><strong>American Sleep Apnea Association</strong></td>
<td>Sleep Apnea</td>
<td>50,000</td>
</tr>
<tr>
<td><strong>Cincinnati Children's Hospital Medical Center</strong></td>
<td>Pediatric Crohn's Disease and Ulcerative Colitis</td>
<td>15,000</td>
</tr>
<tr>
<td><strong>COPD Foundation</strong></td>
<td>Chronic Obstructive Pulmonary Disease</td>
<td>50,000</td>
</tr>
<tr>
<td><strong>Crohn’s and Colitis Foundation of America</strong></td>
<td>Inflammatory Bowel Disease (Crohn’s disease and ulcerative colitis)</td>
<td>30,000</td>
</tr>
<tr>
<td><strong>Global Healthy Living Foundation</strong></td>
<td>Arthritis (rheumatoid arthritis, spondyloarthritis), musculoskeletal disorders (osteoarthritis), and inflammatory conditions (psoriasis)</td>
<td>50,000</td>
</tr>
<tr>
<td><strong>Massachusetts General Hospital</strong></td>
<td>Major Depressive Disorder and Bipolar Disorder</td>
<td>50,000</td>
</tr>
<tr>
<td><strong>University of California, San Francisco</strong></td>
<td>Cardiovascular health</td>
<td>100,000</td>
</tr>
<tr>
<td><strong>University of South Florida</strong></td>
<td>Hereditary Breast &amp; Ovarian Cancer</td>
<td>17,000</td>
</tr>
</tbody>
</table>
including rare diseases

<table>
<thead>
<tr>
<th>Organization</th>
<th>Condition</th>
<th>Population Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>ALD Connect, Inc</td>
<td>Adrenoleukodystrophy</td>
<td>3,000</td>
</tr>
<tr>
<td>Arbor Research Collaborative for Health</td>
<td>Primary Nephrotic Syndrome, Focal Segmental Glomerulosclerosis, Minimal Change Disease, and Membranous Nephropathy Multiple Sclerosis</td>
<td>1,250</td>
</tr>
<tr>
<td>Duke University</td>
<td>Juvenile Rheumatic Disease</td>
<td>9,000</td>
</tr>
<tr>
<td>Epilepsy Foundation</td>
<td>Aicardi Syndrome, Lennox-Gastaut Syndrome, Phelan-McDermid Syndrome, Hypothalamic Hamartoma, Dravet Syndrome, Tuberous Sclerosis</td>
<td>1,500</td>
</tr>
<tr>
<td>Genetic Alliance, Inc</td>
<td>Alström syndrome , Dyskeratosis congenital, Gaucher disease, Hepatitis, Inflammatory breast cancer, Joubert syndrome, Klinefelter syndrome &amp; associated conditions, Psoriasis, Metachromatic leukodystrophy, Pseudoxanthoma elasticum,</td>
<td>50- 50,000</td>
</tr>
<tr>
<td>Immune Deficiency Foundation</td>
<td>Primary Immunodeficiency Diseases</td>
<td>1,250</td>
</tr>
<tr>
<td>Parent Project Muscular Dystrophy</td>
<td>Duchenne and Becker muscular dystrophy</td>
<td>4,000</td>
</tr>
<tr>
<td>Phelan-McDermid Syndrome Fndn</td>
<td>Phelan-McDermid Syndrome</td>
<td>737</td>
</tr>
<tr>
<td>University of Pennsylvania</td>
<td>Vasculitis</td>
<td>500</td>
</tr>
</tbody>
</table>
How do we build the evidence for patient engagement in research?
Model for Evaluating Engagement in Research

Patient-Centered CER

Studies that Matter to Patients
- Changes to research questions, processes, & design
- Study participants’ experiences in the research
  - Recruitment
  - Retention
  - Study Completion

Study Quality

Useful Information

To whom & how results are disseminated
- Trust in Information
- Understanding Information

Uptake of Information

Engagement in Research:
- Who is involved
- When engagement occurs
- Type of engagement
- Engaged partners perceived influence
- Experiences of engaged partners
- Principles of engagement

Patient-Centered Outcomes Research Institute
PCORI has three strategic goals

- Substantially increase the quantity, quality, and timeliness of useful, trustworthy information available to support health decisions.

- Speed the implementation and use of patient-centered outcomes research evidence.

- Speed the implementation and use of patient-centered outcomes research evidence.
We asked merit reviewers to rate their agreement with the following statement: *The patient/stakeholder reviewers provided valuable input during the discussion* (Strongly Disagree, Somewhat Disagree, Neither Agree nor Disagree, Somewhat Agree, and Strongly Agree)

<table>
<thead>
<tr>
<th>Survey</th>
<th>Sample Size</th>
<th>Survey Completion Rate</th>
<th>Percent Who Agree Somewhat or Strongly</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cycle II</td>
<td>205</td>
<td>94%</td>
<td>89%</td>
</tr>
<tr>
<td>Cycle III</td>
<td>167</td>
<td>97%</td>
<td>85%</td>
</tr>
<tr>
<td>August 2013</td>
<td>286</td>
<td>91%</td>
<td>93%</td>
</tr>
<tr>
<td>Winter 2014</td>
<td>209</td>
<td>83%</td>
<td>94%</td>
</tr>
</tbody>
</table>

*Surveys of merit reviewers are conducted immediately following each in-person merit review and are live in the field for 2 weeks.*
Percent of merit reviewers who agree with the statement: “The patient/stakeholder reviewers provided valuable input during the discussion”
Ways of Engaging- ENgagement ACTivity (WE-ENACT) Inventory

- Self-report tool for researchers, patients, and stakeholders
- Completed at baseline and on an annual basis

Domains:
- Who is engaged
- Partnership characteristics
- Level of engagement
- When in research process are they engaged
- Perceived level of influence of partners
- Effects of engagement on research questions, study design, study implementation, and dissemination of results
- Challenges, facilitators
- Lessons learned for engagement
- PCOR principles (respect, co-learning, etc.)
Find Us Online

www.pcori.org
Thank You

Jean Slutsky
Chief Engagement and Dissemination Officer and
Program Director for Communication and Dissemination Research

Patient-Centered Outcomes Research Institute