Defining Patient Centeredness and Engagement in HEOR: Proposed Definition and Stakeholder Response

ISROR Patient Centered Special Interest Group

Forum Presentation | ISPOR 2018 | May 21, 2018 | Baltimore, MD, USA

Speakers

- **Rachel L Harrington, BA**, University of Illinois at Chicago; Chair, ISPOR Patient Centered Special Interest Group

- **Sarah Donelson, MA**, Industry Representative, San Francisco, CA, USA

- **Eleanor M. Perfetto, PhD, RPh, MS**, Senior Vice President, Strategic Initiatives, National Health Council, Washington, DC, USA and Professor, Pharmaceutical Health Services Research, School of Pharmacy, University of Maryland School of Pharmacy, Baltimore, MD, USA

- **Suzanne Schrandt, JD**, Director, Patient Engagement, Arthritis Foundation, Atlanta, GA, USA; Chair, ISPOR Patient Representatives Roundtable – North America
Forum Goals

• To clarify the case for a consensus definition of “patient engagement in research”
• To present a consensus definition of “patient engagement in research”
• To gather stakeholder feedback and responses to the definition, its components, possible implementation, and next steps
  • Forum Speakers
  • Forum Audience

What’s in a Definition?
Patient Engagement in Research

Rachel L Harrington, BA
Why Stop to Define Patient Engagement?

- Foundational Assumption:
  Patients should be engaged in the research activities conducted under the ISPOR umbrella

- ISPOR PC-SIG intended to start a project to define measurement & reporting criteria for “patient engagement in research”

- Quickly identified a lack of consensus on what “patient engagement” meant
  - ISPOR Membership Survey / Interviews
  - Review of the literature

Why does this matter to ISPOR?

- Observational Studies
- Economic Models
- PRO / Preferences
- Health Technology Assessment

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Items provided as examples, does not represent full list of potential interactions

Getting to a Definition

**Strengths**
- Systematic and comprehensive
- Multi-stakeholder, with patients as co-researchers

**Challenges**
- Timing/duration of project
- Turnover among research team (volunteer effort, changes in leadership)

**Perspectives Represented by Manuscript Authors**

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<th>Primary Perspective</th>
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<td>Patient / Patient Advocate</td>
<td>4 (29%)</td>
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<tr>
<td>Academia</td>
<td>8 (57%)</td>
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<tr>
<td>Industry</td>
<td>2 (14%)</td>
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Results of Analysis: Differentiating Patient-Centered and Patient Engagement

- Both terms have distinct themes in literature, but also similarities
- Patient-Centeredness: broader, “older” concept
- Patient Engagement in the research process is, arguably, a necessary but not sufficient requirement for Patient-Centeredness
- Future work: differentiating between related “patient xxxx” terms

For more information, see Poster PHP7, Tuesday 8:30-14:00

Proposed Definition

Patient Engagement in Research is…

“the active, meaningful and collaborative interaction between patients and researchers across all stages of the research process, where research decision-making is guided by patients’ contributions as partners, recognizing their specific experiences and expertise.”
Breaking Down the Details:

- Patients
  - Used inclusively: individual patients, their families and caregivers, as well as patient representatives and advocacy organizations

- Researchers
  - Refers to multiple contributors within the research process:
    - producers of research (those directly conducting the research activity)
    - decision-making consumers of research (e.g., research funders, payer organizations, regulators, etc.)

Breaking Down the Details:

- All Stages
  - Covers the full spectrum of research activities, including planning, conduct, and dissemination.
  - Different patient and researcher representatives may be involved at different stages in the research process.

- Partner
  - The goal of engagement should be partnership
  - Patient contributions given equal weight as those of any other contributor of the research team
Breaking Down the Details:

- **Patients’ Contributions**
  - Includes patient perspectives, preferences, experiences, opinions, and inputs.
  - May be captured formally (e.g., through stated preference methods), or informally (e.g., focus groups, advisory board membership, etc.).
  - Not intended to reflect patient-level clinical information collected as part of a research study, (i.e., the patient as a “data point”).

Next Steps

- In process of getting feedback:
  - Regional Patient Roundtables (~160 representatives, including North America, Europe and Latin America)
  - Full Patient-Centered SIG Membership (~450 members)
- Manuscript submitted to *Value in Health*, Summer 2018
- Update to ISPOR Book of Terms, Fall 2018
- Shifting SIG focus… measurement? webinar series on examples of engagement? Suggestions welcome!

**SIG Open Meeting Tuesday, 22 May, 12:30PM in Room 307**
Acknowledgments and Thanks

• ISPOR Patient-Centered Special Interest Group Leadership Team, past and present
  • Maya Hanna, Elisabeth Oehrlein, Russell Wheeler, Rob Camp, Rainald von Gizycki, Amie Scott, Francis Nguyen, Violeta Astratinei, Asha Hareendran, Donald Patrick, Eleanor Perfetto

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  • Clarissa Cooblall, Theresa Tesoro

• ISPOR Patient Representatives Roundtables

• Data Management Support
  • Aida Kuzacan, O’Mareen Spence, Shannon Marrow, Hager Elgendi

SECTION

Industry Perspective

Sarah Donelson, MA

The views and opinions expressed in the following slides are mine and should not be attributed to any affiliated organization.
The most important elements for effective patient group engagement include establishing meaningful partnerships, demonstrating mutual benefits, and collaborating as partners from the planning stage forward. “

- Service provider/vendor
- Charitable giving
- Confidentiality agreements
Study finds that half of pharmaceutical and biotech companies surveyed are implementing patient-centric drug development initiatives; but measurement and success vary widely

- No specific definition of Patient Engagement
- Fluidity of terms

Patient Engagement in Drug Development Study Results available here:
https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement

Capturing the Value of Patient Engagement:
2016 Study of Patient-Centric Initiatives in Drug Development
https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement
Patient-Centric Initiatives – Not Considered

The top initiatives companies are not considering are the medicine co-development partnerships with patient associations and open design and crowdsourcing.

Base: 22 Companies

Summary Findings: Current Cost and Impact Overall

https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement
Use "as is" PROMIS/FACIT Examples of Avoiding Wheel Re-invention, David Cella, PhD presented at Ninth Annual Patient-Reported Outcome Consortium Workshop, Silver Spring, Maryland, April 26, 2018.

- Including patients changed measure
- More information captured
Meaningful Patient Engagement

Eleanor Perfetto, PhD
NHC Terminology

• **Patient centered:**
  Any process, program or decision focused on patients in which patients play an active role as *meaningfully engaged participants*, and the central focus is on optimizing use of patient-provided information.

• Doing things WITH -- not FOR or TO – patients
• Relies on meaningful engagement

Meaningful Patient Engagement

• A bi-directional relationship between a patient(s) and another stakeholder(s)
• Characteristics:
  - Reciprocal
  - Co-learning
  - Co-development
  - Partnership
  - Honesty
  - Respect
  - Trust
  - Transparency

• Communications are open, honest, and clear
• Goals, participants, methods, desired impacts, & actual impacts are clearly outlined & transparent
Levels of Patient Engagement

- **Stakeholder-Directed**
  - Patient/Patient group led
- **Partnership**
  - Investigator/Co-investigator
- **Collaboration**
  - Advisory committee member
- **Consultation**
  - Consultant
  - Interviews
  - Focus groups
  - Surveys
- **Informal**
  - Unstructured discussions
- **Study participant**

Don’t fall into the trap…

Not all patient-REPORTED outcomes are patient CENTERED.

Not all patient-CENTERED outcomes are patient REPORTED.

Adapted from Forsythe, et al. JGIM, 2015
Peretto, ISPOR 2015 Annual Meeting
Patient Engagement in Drug Development


Case Example #1: Direct Patient Engagement Driving Changes to Target Product Profile and Development Plans

Organization: A Pharmaceutical Company Research & Development Department

Description:

- Company engaged directly with patients to understand what they expect from future products.
- Because of the direct patient engagement, a development team changed its Target Product Profile (TPP) for a lead compound.
- Company teams immersed themselves in patient-experience feedback sessions and interviews of patients with a target disease.

Lessons Learned:

- There is no substitute for understanding diseases through the lens of people living with the condition. Engaging directly with patients caused this team to refine what a new medicine should do and to measure something they were not previously measuring because of what matters to patients. Directly engaging with patients early in the development of medicines can result in solutions that better meet patients’ needs.
Case Example #2: Community-Engaged Treatment Preferences and Priorities for A Specific Rare Disease

Organization(s): A School of Public Health, a Patient Group, and a Contractor

Description:

- Engaged patients, caregivers, and other stakeholders in the development of novel patient-centered instruments.

Lessons Learned:

- Engagement with stakeholders is a vital step to develop meaningful tools that help translate patient voice into the quantifiable and scientifically rigorous language often preferred by policy makers.
- Patients have distinct preferences both for their medical treatments and for how their voices are incorporated into the research process. Listening to and honoring these voices improves the quality of research and its regulatory impact.
- Patients are the experts of their own lives and experiences. Openly recognizing this expertise is the first step in producing genuinely patient-centered research.

Case Example #3: Direct Involvement of Patients in a Prospective Study to Measure Patient Expectations

Organization: Pharmaceutical Company

Description:

- Objective was to understand several aspects in the decision-making process from a patient’s perspective.
- Included the patient’s opinion on all aspects of the study: study design, instrument preparation, and discussions with clinical experts.

Lessons Learned:

- Patients and pharma working together allows transparency for pharma, reducing past barriers and gaining trust from patients.
- This approach supports recruitment and retention of patients.
- The team learned how important it is for patients to have their voice heard and be an advocate for their condition, especially during the design discussions with clinical experts.
- The team learned the importance of adapting to language and patient-preferred terminology, the research is more accessible and understood when recruiting.
Case Example #4: Trial Simulation Workshops to Gather Input from Phase II Investigators, Sites, and Patients

Organization: A Pharmaceutical Company Research and Development Department and Contractor

Description:
- Optimize trial design and implementation of a multinational Phase IIb trial.
- Simulations involved four former Phase IIa participants and caregivers, eight “research naïve” individuals and caregivers.
- Factors influencing willingness to participate included reputation of the investigator/trial site; accessibility; fear of disease; length of trial, lifestyle fit; continuity of trial staff; empathy; physicians in response to other health concerns.

Impact:
- Implementing simulations at academic institutions required additional administrative steps as compared to other settings such as market-research facilities, but was deemed justified given the authenticity of the environment.
- The findings will inform implementation of programs around participant education and caregiver engagement.

Case Example #5: Advocate Feedback on a Clinical Trial Questionnaire

Organization: A Pharmaceutical Company

Description:
- Collaborative process to create a short questionnaire to better understand what symptoms are most important to patients.
- Company drafted initial two-question survey, which was reviewed by national patient advocacy organization dedicated to the disease of interest.
- Feedback from patient organization was critical and led to significant re-wording as well as addition of a third question that was suggested.

Impact:
- Engaging individuals with lived experience and those who represent them in survey design ensures the final survey questions are understandable to the end-user.
- Adding a wellness question provided an opportunity to measure an outcome that was important to patients.
Why is clarity so crucial?

Suz Schrandt, JD

Examples of the “other” interpretations

- Engagement in one’s own care
- Engagement with one’s patient community
- Engagement in a clinical trial—as a study participant
- Engagement in registries and other data-collection efforts
- Engagement in some advocacy and policy activities
Why is this a problem?

How does an established definition help?

Orients to common purpose

Or highlights lack of common purpose
What should come next?

- Broad and targeted dissemination
- Best practice: begin with an agreed-upon definition
- Use the definition as a beginning, middle, and end point.

Sign up as a Review Group Member

- Submit your evaluation of this session using the ISPOR app
- Join ISPOR Special Interest Groups
- Need ISPOR membership number
- For more information, e-mail sigs@ispor.org
Discussion Questions to Consider

• Does this definition resonate from your perspective?
  • Strengths? Weaknesses?
• How could you see implementing this type of definition into your work?
• When working on a research project, have there ever been disagreements about what “counted” as engagement?
  • If so, would having a specific definition have helped?
• How to use the ISPOR “patient engagement in research” definition in securing funding for research proposals?