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

ISPOR Patient Centered
Special Interest Group

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

ISPOR www.ispor.org

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, it’s components, possible implementation, and next steps
  - Forum Speakers
  - Forum Audience

SECTION

1

What’s in a Definition? Patient Engagement in Research

Rachel L Harrington, BA

Why Stop to Define Patient Engagement ?

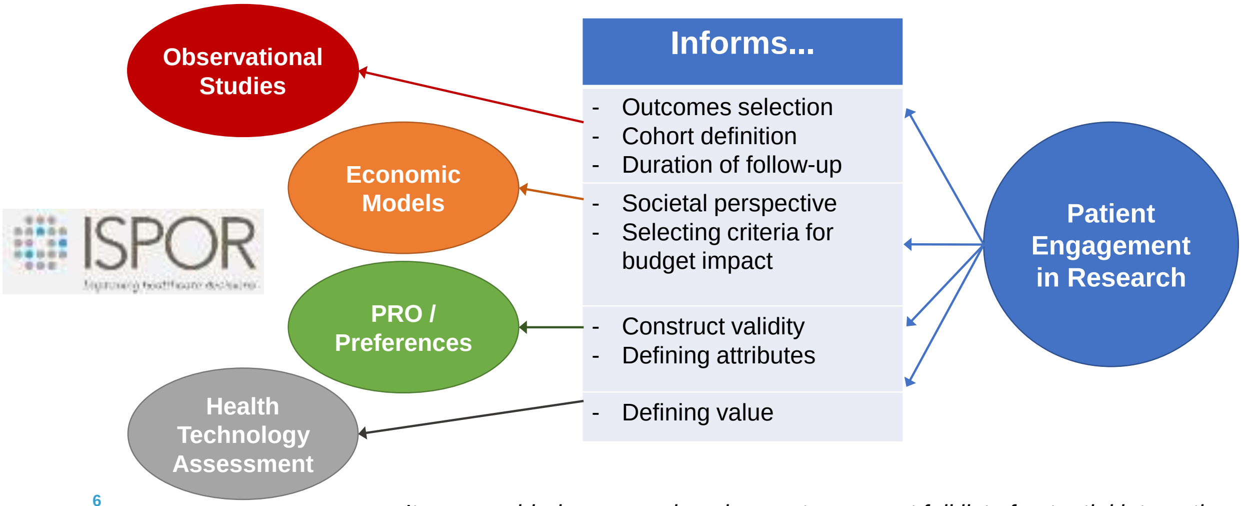
- Foundational Assumption:

  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[1-4]

1] Hoos A, et al., 2015 [2] Concannon TW, et al. 2012 [3] Carman KL, et al.2013 [4] FasterCures, 2018

Why does this matter to ISPOR?

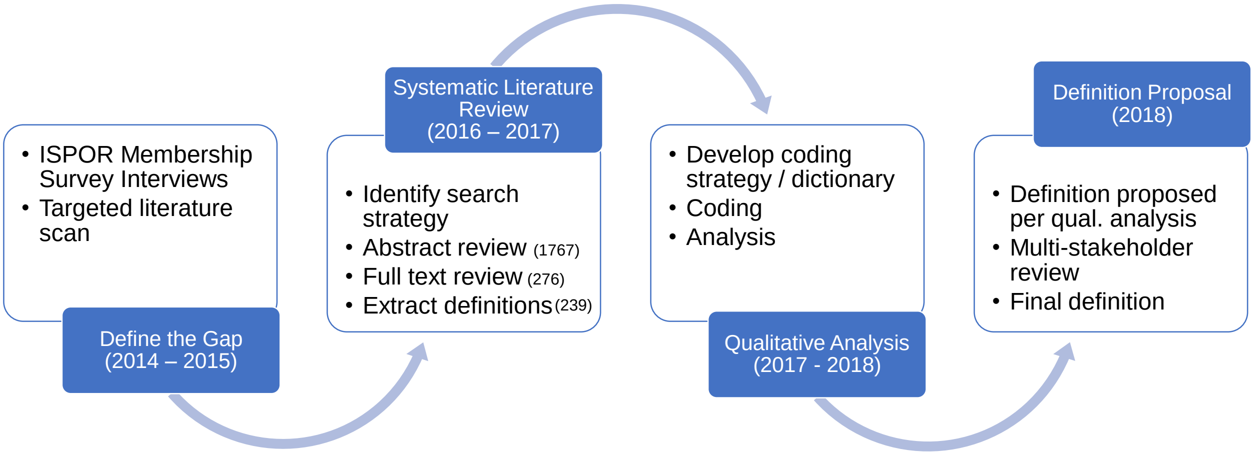


Informs...

- Outcomes selection
- Cohort definition
- Duration of follow-up
- Societal perspective
- Selecting criteria for budget impact
- Construct validity
- Defining attributes
- Defining value

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

Getting to a Definition



Define the Gap
(2014 – 2015)

- ISPOR Membership Survey Interviews
- Targeted literature scan

Systematic Literature Review
(2016 – 2017)

- Identify search strategy
- Abstract review (1767)
- Full text review (276)
- Extract definitions (239)

Qualitative Analysis
(2017 - 2018)

- Develop coding strategy / dictionary
- Coding
- Analysis

Definition Proposal
(2018)

- Definition proposed per qual. analysis
- Multi-stakeholder review
- Final definition

Deliverables Throughout: Workshops, VOS article, Forums, Posters

Getting to a Definition

<u>Strengths</u>

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

<u>Challenges</u>

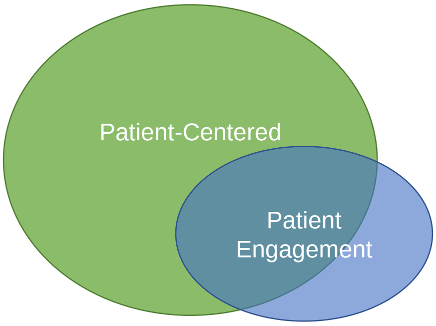
- Timing/duration of project
- Turnover among research team (volunteer effort, changes in leadership)

Perspectives Represented by Manuscript Authors

Primary Perspective	N (%)
Patient / Patient Advocate	4 (29%)
Academia	8 (57%)
Industry	2 (14%)

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



Conceptual representation

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
- ISPOR Scientific & Health Policy Initiatives
  - Clarissa Cooblall, Theresa Tesoro
- ISPOR Patient Representatives Roundtables
- Data Management Support
  - Aida Kuzacan, O’Mareen Spence, Shannon Marrow, Hager Elgendi

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SECTION

2

Industry Perspective

Sarah Donelson, MA

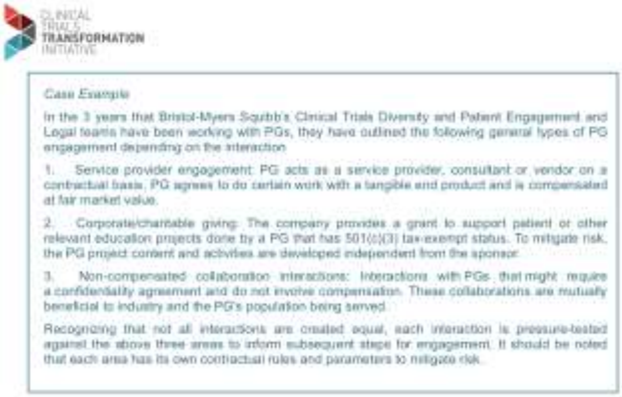
The views and opinions expressed in the following slides are mine and should not be attributed to any affiliated organizationute.

Clinical Trials: Original Research

The Rules of Engagement: CTTI Recommendations for Successful Collaborations Between Sponsors and Patient Groups Around Clinical Trials

Diane Bloom, PhD, MPH[1], Joel Beetsch, PhD[2],
Matthew Harker, MPH, MBA[3], Sharon Hesterlee, PhD[4],
Paulo Moreira[5], Bray Patrick-Lake, MFS[6], Wendy Selig, MSJ[7],
Jeffrey Sherman, MD, FACP[8], Sophia K. Smith, PhD[9],
James E. Valentine, JD, MHS[10], and Jamie N. Roberts, MPH, MA[6]

"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**. "



CLINICAL TRIALS TRANSFORMATION INITIATIVE

Case Example

In the 3 years that Bristol-Myers Squibb's Clinical Trials Diversity and Patient Engagement and Legal teams have been working with PGs, they have outlined the following general types of PG engagement depending on the interaction.

1. Service provider engagement: PG acts as a service provider, consultant or vendor on a contractual basis. PG agrees to do certain work with a tangible end product and is compensated at fair market value.

2. Corporate/charitable giving: The company provides a grant to support patient or other relevant education projects done by a PG that has 501(c)(3) tax-exempt status. To mitigate risk, the PG project content and activities are developed independent from the sponsor.

3. Non-compensated collaboration interactions: Interactions with PGs that might require a confidentiality agreement and do not involve compensation. These collaborations are mutually beneficial to industry and the PG's population being served.

Recognizing that not all interactions are created equal, each interaction is pressure-tested against the above three areas to inform subsequent steps for engagement. It should be noted that each area has its own contractual rules and parameters to mitigate risk.

- Service provider/vendor
- Charitable giving
- Confidentiality agreements

The Rules of Engagement: CTTI Recommendations for Successful Collaborations Between Sponsors and Patient Groups Around Clinical Trials
Diane Bloom, PhD, MPH, Joel Beetsch, PhD, Matthew Harker, MPH, MBA, Sharon Hesterlee, PhD, Paulo Moreira, Bray Patrick-Lake, MFS, Wendy Selig, MSJ, Jeffrey Sherman, MD, FACP, Sophia K. Smith, PhD, James E. Valentine, JD, MHS, Jamie N. Roberts, MPH, MA
First Published July 27, 2017. http://journals.sagepub.com/doi/suppl/10.1177/2168479017720247

NOV 14, 2016 - WASHINGTON, DC

DIA Releases Results of Patient Engagement in Drug Development Study

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

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Capturing the Value of Patient Engagement: 2016 Study of Patient-Centric Initiatives in Drug Development

DIA

Tufts Center for the Study of Drug Development
TUFTS UNIVERSITY

Patient-Centric Initiatives† (PCI) by Category

Innovative Partnerships
- **Patient group support and involvement**
- **Patient advisory boards and focus groups***
- Professional panels
- Community conversations
- Medicine co-development partnerships with patient groups
- Patient group landscape analysis tool (disease area specific)

Protocol Design
- **Adaptive trial designs and adaptive licensing**
- **Open design and crowdsourcing**
- Patient involvement in study feasibility and design
- Protocol feasibility review committees
- Real world, practice-based clinical trials

Technology Advancements
- **Apps for clinical data collection/analytics**
- **Digital medicine****
- **Direct-to-patient clinical trials/telemedicine**
- **E-Consent**
- **Gaming**
- **Social Media/Online Engagement**
- Human factor testing/simulation
- Centralized/integrated HER & clinical records

Study Volunteer Ease
- **Home nursing networks and logistics assistance**
- **Patient counseling and education**
- Patient trial community during trials and after trials
- Lay summary clinical trial results
- End of study surveys

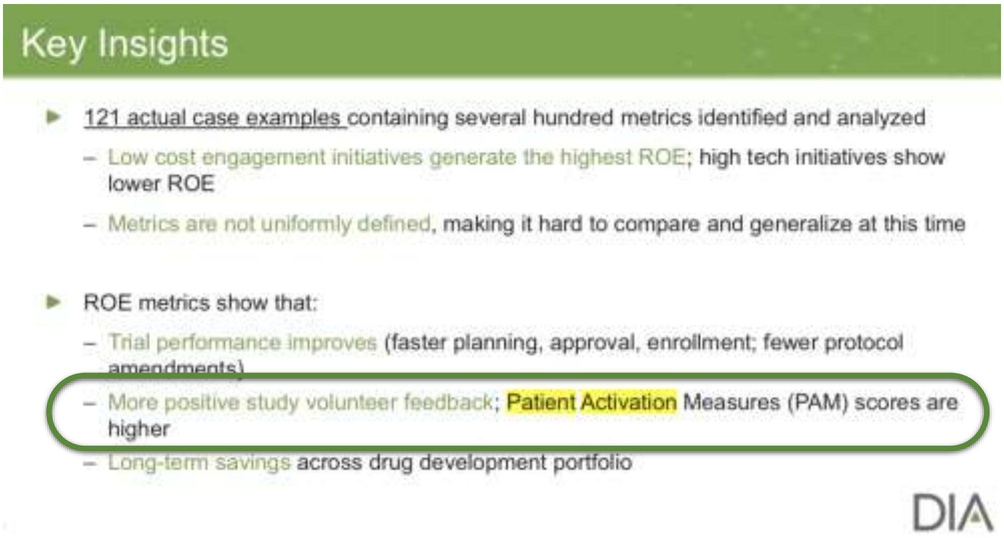
Text in **RED** indicates
identified

† Only those PCIs repo
case studies

*Includes groups such
(National Institute for
Research)

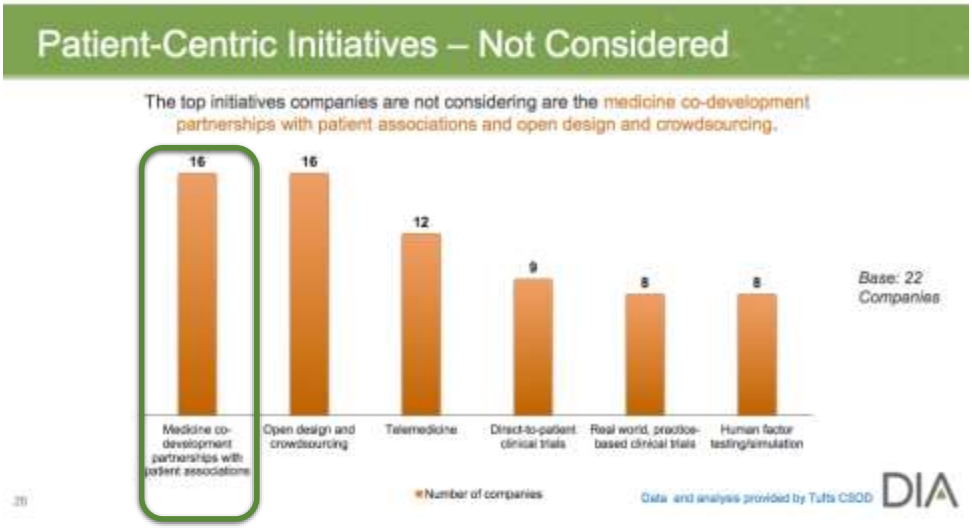
** Medicine that can be
using technology.

https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement



Key Insights

- 121 actual case examples containing several hundred metrics identified and analyzed
  - Low cost engagement initiatives generate the highest ROE; high tech initiatives show lower ROE
  - Metrics are not uniformly defined, making it hard to compare and generalize at this time
- ROE metrics show that:
  - Trial performance improves (faster planning, approval, enrollment; fewer protocol amendments)
  - More positive study volunteer feedback; Patient Activation Measures (PAM) scores are higher
  - Long-term savings across drug development portfolio

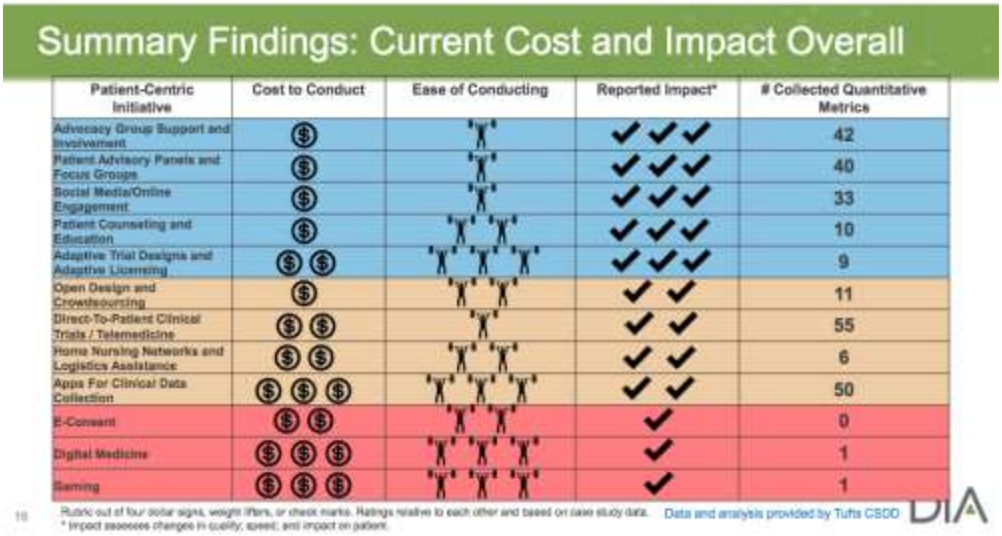


Patient-Centric Initiatives – Not Considered

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

Data and analysis provided by Tufts CSDD DIA

https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement



Summary Findings: Current Cost and Impact Overall

Patient-Centric Initiative	Cost to Conduct	Ease of Conducting	Reported Impact*	# Collected Quantitative Metrics
Advocacy Group Support and Involvement	$	🏋	✓✓✓	42
Patient Advisory Panels and Focus Groups	$	🏋	✓✓✓	40
Social Media/Online Engagement	$	🏋	✓✓✓	33
Patient Counseling and Education	$	🏋🏋	✓✓✓	10
Adaptive Trial Designs and Adaptive Licensing	$$	🏋🏋🏋	✓✓✓	9
Open Design and Crowdsourcing	$	🏋🏋	✓✓	11
Direct-To-Patient Clinical Trials / Telemedicine	$$	🏋	✓✓	55
Home Nursing Networks and Logistics Assistance	$$	🏋🏋	✓✓	6
Apps For Clinical Data Collection	$$$	🏋🏋🏋	✓✓	50
E-Consent	$$	🏋🏋	✓	0
Digital Medicine	$$$	🏋🏋🏋	✓	1
Gaming	$$$	🏋🏋🏋	✓	1

Rubric out of four dollar signs, weight lifters, or check marks. Ratings relative to each other and based on case study data.
* Impact assesses changes in quality, speed, and impact on patient.

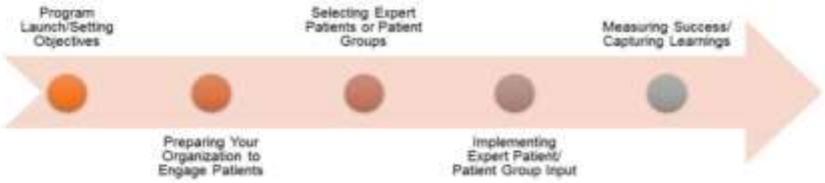
Data and analysis provided by Tufts CSDD DIA

https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement

Considerations Guide & Practical "How to"

Developed a 'Considerations Guide'

- Designed to facilitate the development of a customized patient-centric initiative
- Collects and directs users to various resources currently available



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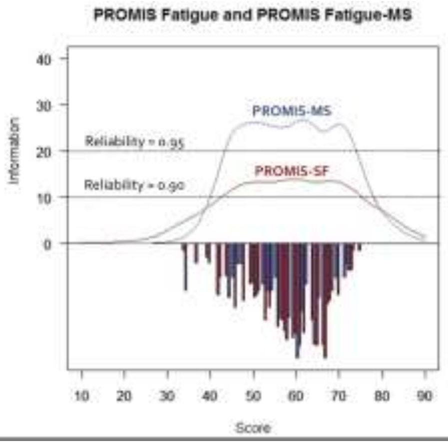

- Including patients changed measure
- More information captured

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. https://c-path.org/wp-content/uploads/2018/05/2018_Session5_WhyReinventWheelFINAL.pdf

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."

Patient Engagement in the Lifecycle of Medical Products

COLOR KEY
- Green: denotes aspects of patient engagement in place, with efforts begun
- Yellow: denotes aspects that are not now in practice but should be implemented in the medical product life cycle for effective and meaningful patient engagement.

DIA

https://www.diaglobal.org/en/resources/areas-of-interest/patient-engagement

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SECTION

3

Meaningful Patient Engagement

Eleanor Perfetto, PhD

NHC Terminology

- **Patient centered:**

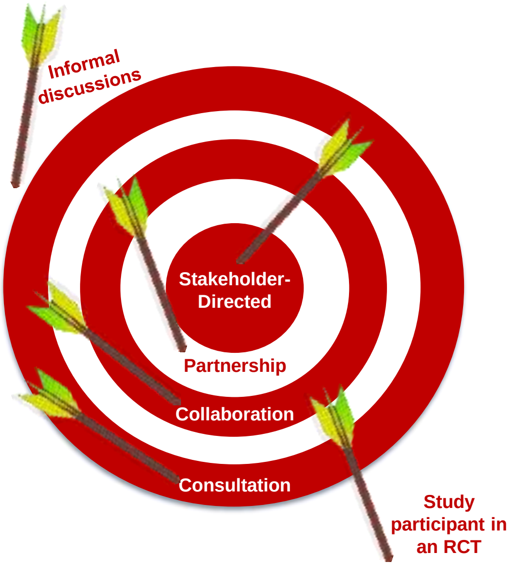
  Any process, program or decision focused on patients in which patients play an active role as ***meaningfully engaged participants***, and the central focus is on optimizing use of patient-provided information.

- 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**

Adapted from Forsythe, et al. JGIM, 2015
Perfetto, ISPOR 2015 Annual Meeting

Don’t fall into the trap…

Not all patient-REPORTED outcomes
are patient CENTERED.

Not all patent-CENTERED outcomes
are patient REPORTED.

Patient Engagement in Drug Development

- Understand disease/condition from patient's perspective; the patient journey; preferences for outcomes, etc
- Patient registries
- Identify unmet need
- Patient/ community/ researcher training on effective partnership
- Assess current treatment effectiveness/ sub-populations

- Develop a research question based on patient interests
- Patients prioritize research questions
- Patients provide feedback on potential indications

- Gather or develop study tools (PROs, ClinROs, PerfOs, ObsROs)
- Patients identify potential barriers for study recruitment/ participation
- Plan for who, when, and how patients will be engaged throughout
- Patients' feedback on study endpoints

- Patients help with recruitment and provide feedback on experiences as participants
- Patients serve on data safety monitoring board (DSMB)

- Patients serve on advisory committees, contribute to benefit/risk discussions, and as Patient Representatives

- Patients provide input on Risk Evaluation and Mitigation Strategies (REMS)
- Patients provide feedback on Phase IV studies
- Patients understand how to report adverse events

- Patients provide context for economic information under FDAMA 114
- Patients provide feedback on patient counseling information, MedGuides, Package Inserts, Instructions for Use

Perfetto EM, Oehrlein E, Anyanwu C, Burcu, et al. 2015. "Stakeholder Perspectives on Patient-Focused Drug Development: Insights from FDA, Patients, Industry, and Payers." URL: http://www.pharmacy.umaryland.edu/media/SOP/wwwpharmacyumarylandedu/centers/cersievents/pfdd/mcersi-pfdd-proceedings-rubric.pdf . Accessed May 15, 2018.

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.

SECTION

4

Why is clarity so crucial?

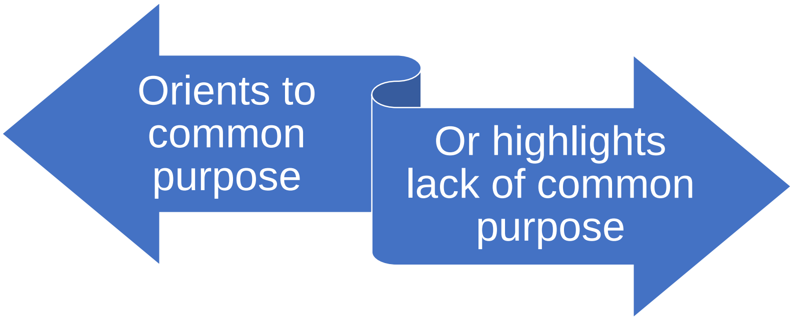
Suz Schrandt, JD

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]

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If you would like to submit a new topic, please send an email to: 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?