

# Patient Engagement: What Is In A Name?



**ISPOR Student  
Research Showcase**  
Monday, 9 November, 2015

## Patient Engagement In Research Working Group



“Failure to attend to the information and concerns of stakeholders clearly is a kind of flaw in thinking or action that too often and too predictably leads to poor performance, outright failure or even disaster.”

~ J. Bryson, *Public Management Review* (2004)

## Why the focus on patient engagement?



- Patient and consumer involvement in decision-making increasing worldwide
- Change from acute health to chronic health management
- Rate of scientific discovery is increasing
  - More to know
  - More to do
  - More to manage (i.e. care coordination)
- Estimated 17 years for new evidence to reach practice

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### **Moderator:**

**Donald L. Patrick, PhD, MSPH**, Professor, University of Washington, and Director, Seattle Quality of Life Group  
Seattle, WA, USA

### **Speakers:**

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

**Russell Wheeler**, Patient Advocate for Leber's Hereditary Optic Neuropathy, Winchester, UK

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# The Patient Centered Special Interest Group



## Patient Engagement in Research Working Group

Started April 2014

**Goal: To determine how best to involve patients and their representatives in the research process by identifying:**

- ❖ The stages at which patients should be involved
- ❖ The level of their involvement in each stage
- ❖ The challenges that will face the researchers
- ❖ Recommendations

Manuscript for *Value in Health*

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## Patient Engagement In Research Working Group



### Co-Chairs:

**François Houyez**, Director of Treatment Information & Access, Health Policy Advisor  
EURORDIS, Paris, France

**Todd Berner MD**, Medical Director, Head Global Medical Affairs Strategy, Immunology,  
Baxalta, Inc., Bannockburn, IL., USA

### Leadership:

**Laurie Burke, RPh, MPH**, Affiliate Associate Professor, University of Maryland, School of  
Pharmacy, Maryland, USA

**Rob Camp**, Communications Manager, EUPATI, Barcelona, Spain

**Asha Hareendran, PhD, MA**, Senior Research Scientist, Evidera, London, UK

**Rachel Harrington**, Senior Manager, Health Economics and Clinical Outcomes  
Research, Astellas Medical Affairs Americas, Northbrook, IL, USA

**Shrividya Iyer**, PhD, Director, Pfizer, Inc., New York, NY, USA

**Donald L. Patrick, PhD, MSPH**, Director, Seattle Quality of Life Group and Biobehavioral  
Cancer Prevention and Training Program, University of Washington, Seattle, WA, USA



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# Patient Engagement In Research Working Group



## Leadership: (continued)

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

**Bettina Ryll, MD, PhD**, Department of Physiology and Developmental Biology, Evolutionary Biology Centre, Uppsala University, Uppsala, Sweden

**Amie Scott, MPH**, Business Health Research Analyst, SEAS Capital Partners, Plymouth, MI, USA

**Oliver Timmis, BA**, Head of Projects, AKU Society, Cambridge, UK

**Rainald von Gizycki, MD**, Honorary President, Pro Retina Deutschland e.V., Coordinator, Pro Retina Europe, and Member of Board of Trustees, Pro Retina Foundation Fighting Blindness, Bad Nauheim, Germany

**Kim Wever**, Policy Officer Research and International Affairs, VSOP - Dutch Genetic Alliance, Soest, The Netherlands

**Russell Wheeler**, Patient Advocate for Leber's Hereditary Optic Neuropathy, Winchester, UK

**Thomas Willgoss, PhD**, Senior Analyst, Clinical Outcomes Assessment, Abacus International, Bicester, UK



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**Group conducted survey of ISPOR members to determine their knowledge of patient involvement in outcomes research.**

❖ **19th Annual ISPOR International Meeting in Montreal, Canada**

❖ **17th Annual European Congress in Amsterdam, The Netherlands**

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## Presented their findings at:

Workshop at the ISPOR 17th Annual European Congress November 2014 in Amsterdam, The Netherlands

### Patient Engagement In Outcomes Research: Current Status, Questions, Beliefs, And Future Perspectives

Forum at the ISPOR 20th International Meeting, May 2015 in Philadelphia, PA, USA along with ISPOR's other patient groups

### Patient Engagement in Health Economic and Outcomes Research: Current and Future ISPOR Initiatives

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- In conducting the research for the manuscript, the group realized that terms are often used, but rarely defined.
  - It was imperative to arrive at a recommended ISPOR standard term and definition for what is generally meant by "patient engagement" and other "patient" terms to be used in ISPOR communications, documents, etc.
  - ***Objective:*** Identify published definitions for the terms, "patient centeredness", "patient engagement," and related terms; especially in the context of research, drug development, and health care decision making.

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# Acknowledgements

- University of Maryland, School of Pharmacy
  - Maya Hanna
  - Elisabeth Oehrlein
  - Francis Nguyen
- ISPOR Headquarters
  - Clarissa Cooblall
  - Theresa Tesoro

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# Building on Previous Work

**Engaging the Patient: The Need for Standardizing Terminology when Incorporating the Patient Voice in Research and HTA**  
 Elisabeth Oehrlein, BA | Eleanor M Peretto, PhD | Joseph Vandigo, MSA  
 University of Maryland School of Pharmacy, Pharmaceutical Policy Services Research Department

**RESULTS**

24 Unique Organizations Identified

Definition/Process/Framework Found

1

PCORI

"... a major role in the conceptual development of patients, caregivers, clinicians, and other healthcare stakeholders throughout the research process—first input activities through design and needs of research to dissemination of results."

2

NICE

Patient involvement: patients contribute to evidence or decisions that will have consequences for the patient community. Deviation of this principle knowledge and relevant experience as patients

3

EUPATI

Patient involvement: patients contribute to evidence or decisions that will have consequences for the patient community. Deviation of this principle knowledge and relevant experience as patients

4

IQWiG

Patient involvement: patients contribute to evidence or decisions that will have consequences for the patient community. Deviation of this principle knowledge and relevant experience as patients

**RESULTS (Cont.)**

- Experts identified 28 unique organizations.
- The majority of organizations' activities provided only examples of patient engagement activities.
- Only four (16.7%) of the websites supplied a definition, process, or framework for patient engagement.
- The four definitions located had consistent themes, but no definition matched any other.

**CONCLUSIONS**

There is clearly no consensus on patient engagement terms and approaches, even among organizations considered to be the trailblazers. The terms and approaches used in patient engagement in research are in need of standardization. A common definition of "patient input" can facilitate the generation and dissemination of evidence to as broad an audience as possible.

**REFERENCES**

1. PCORI. What do we mean by engagement? <https://www.pcori.org/funding-opportunities/what-is-patient-engagement>. Accessed 11/11/2015, 2015.  
 2. NICE. Community engagement in research. <https://www.nice.org.uk/guidance/communities-engagement/section-1>. Accessed 11/11/2015, 2015.  
 3. IQWiG. Consulting the patient: guidelines. [https://www.iqwig.de/fileadmin/user\\_upload/consulting\\_the\\_patient\\_guidelines\\_2015.pdf](https://www.iqwig.de/fileadmin/user_upload/consulting_the_patient_guidelines_2015.pdf). Accessed 11/11, 2015.  
 4. European Patient's Academy on Therapeutic Innovation. <http://www.patientsacademy.eu/index.php/en/primary-voice-over-the-counter>. Accessed 11/11/2015, 2015.

Oehrlein EM, Peretto EM, Vandigo J. Engaging the Patient: The Need for Standardizing Terminology when Incorporating the Patient Voice in Research and HTA. 12th HTAi Annual Meeting 13-17 June 2015. Oslo, Norway.

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## Building on Previous Work

WORKSHOP

Special Interest Groups

[W21] PATIENT ENGAGEMENT IN OUTCOMES RESEARCH: CURRENT STATUS, QUESTIONS, BELIEFS, AND FUTURE PERSPECTIVES

Wednesday, 12 November 2014  
08:45 – 09:45

ISPOR

ISPOR Patient Centered Special Interest Group:  
Patient Engagement in Research Working Group

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## Search Strategy (1) - Web

### 1. Identify definitions of patient centeredness and engagement among public and private domains in the English language

- a. Candidate organizations recommended by experts (the ISPOR SIG members) as being prominent in the field of patient centeredness and patient engagement (e.g., PCORI)
- b. Organizations considered "umbrella" patient advocacy organizations (an organization of patient advocacy organizations, e.g., NORD, CORD), disease-specific and non-disease-specific
- c. A general search using the Google search engine:
  - **Patient:** *Focused, Focused Drug Development, Focused Outcome Research, Centered/Centric/Centeredness Outcome Research, Centered/Centric/Centeredness Drug Development, Centered/Centric/Centeredness, Engagement, Empowerment,*
  - **Consumer:** *Centered/Centric/Centeredness, Engagement (limit to health care), and*
  - **Person/People:** *Centered/Centric/Centeredness*

### 2. Spreadsheet created with definitions identified (assessed by 3 research assistants for relevance).

- Term searched, definition provided vs used but not defined, organization name, organization type, website address, and country of origin.

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# Search Strategy (2) - Literature

## 1. PubMed and EMBASE were searched for definitions of patient centeredness and patient engagement during the years of XXXX–2015

- a. **PUBMED-** ((((((patient engagement[Title/Abstract] OR consumer engagement[Title/Abstract] OR patient involvement[Title/Abstract] OR patient cent\*[Title/Abstract] OR consumer cent\*[Title/Abstract] OR person cent\*[Title/Abstract] AND definition[Title/Abstract] OR "defined as"[Title/Abstract]))))))))
- b. **EMBASE-** ('patient centered outcomes research' OR 'patient engagement' OR 'patient involvement' OR 'person centered care' AND 'definition' OR 'defined as')

## 2. Inclusion criteria were established *a priori*

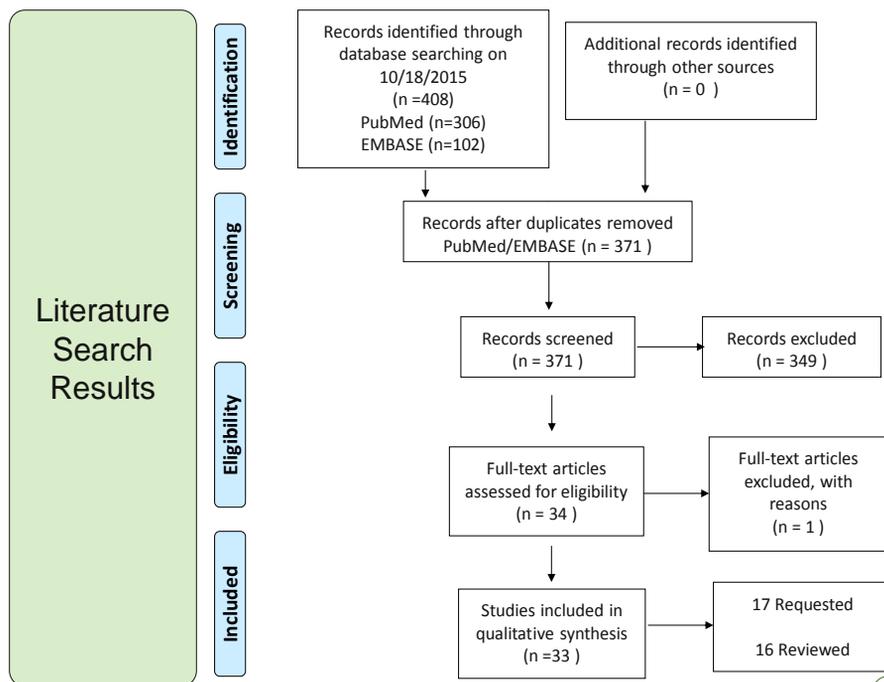
- a. Definition for patient centeredness, patient engagement, or a related pre-specified term,
- b. Definition associated with health care research and/or provision, and
- c. English language

## 3. Abstracts reviewed for relevance by 2 individuals

## 4. Spreadsheet created based on full text review (definitions assessed by 2 research assistants)

- Author(s), title, journal, year of publication, quote/context defining term, definition, data accessed, full citation, and link to article abstract.

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## Draft Results (Nov. 10, 2015)

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- Literature Search
  - 34 articles selected for full-text review
  - 16 reviewed thus far
  - 17 requested
  - 1 in Chinese (but abstract was in English)
- Web search

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## Nota Bene

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- Predominantly low-tier journals
  - The Clinical Teacher (0.00)
  - Studies in Health Technology Informatics (0.00)
  - Journal of Health, Organization and Management (0.00)
- Not immediately available through University Health Sciences Library (requested through interlibrary loan)

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# Draft Literature Search Results (9 definitions/ 16 full-text articles)

	Term	Definition
1	patient centricity	"...a dynamic process through which the <b>patient regulates the flow of information to and from him/her via multiple pathways to exercise choices</b> consistent with his/ her preferences, values, and beliefs. This fundamentally transformative concept affects how health care decisions are made and who has the authority to make them.."
2	patient centered care	"treating the patient as a unique individual."
3	patient centeredness	"a comprehensive model of dimensions of patient-centeredness that should be considered if one wants to implement a patient-centered approach to health care in routine practice."
4	person centeredness	"... (i) To <b>address the person's specific and holistic properties</b> , (ii) to address the person's difficulties in everyday life, (iii) to <b>consider the person as an expert on their own condition</b> and put the emphasis on participation and empowerment, and (iv) to <b>respect the person 'behind' the impairment</b> or the disease."
5	patient-centred outcome measure	"It <b>identifies outcomes that are desired and valued by individuals</b> (patients). It is developed to <b>reflect patient priorities</b> . Measurement is undertaken at appropriate times and points within routine clinical care. The resultant <b>information is used to inform the health-care professional/patient decision-making process, service evaluation, audit and planning.</b> "
6, 7	public involvement	(1) "... <b>emphasizes the involvement of lay people</b> as taxpayers who may or may not have special knowledge of the subject under discussion. (2) "... <b>the spectrum of processes and activities that bring the public into the decision-making process.</b> "
8, 9	patient empowerment	(1) "A social process of <b>recognizing, promoting and enhancing people's abilities to meet their own personal needs</b> , solve their own problems and mobilize the necessary resources in order to feel in control of their own lives." (2) a continuous process through which <b>patients work in partnership with the healthcare system</b> to enable patients to become more responsible for, and involved in, their treatment and healthcare...developing the patient's own skills and <b>promoting a more balanced partnership in decision making.</b> "

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## Example: Dimensions of Patient Centeredness

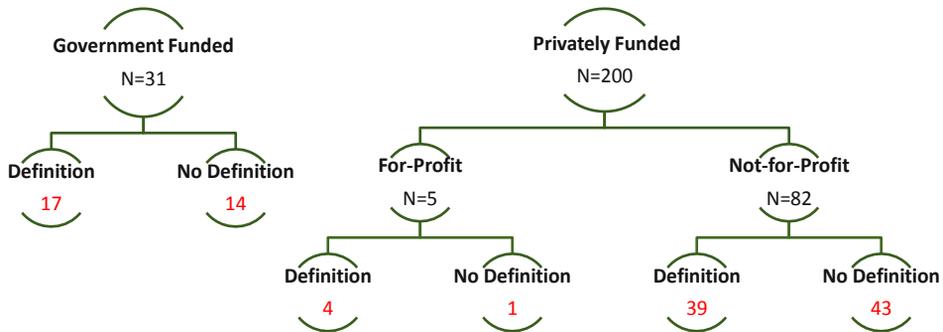
Dimension	Brief description
<b>Principles</b>	
Essential characteristics of the clinician	A set of attitudes towards the patient (e.g. empathy, respect, honesty) and oneself (self-effectiveness) as well as medical competency
Clinician-patient relationship	A partnership with the patient that is characterized by trust and caring
Patient as a unique person	Recognition of each patient's uniqueness (individual needs, preferences, values, feelings, beliefs, concerns and ideas, and expectations)
Biopsychosocial perspective	Recognition of the patient as a whole person in his or her biological, psychological and social context
<b>Enablers</b>	
Clinician-patient communication	A set of verbal and nonverbal communication skills
Integration of medical and non-medical care	Recognition and integration of non-medical aspects of care (e.g. patient support services) into health-care services
Teamwork and teambuilding	Recognition of the importance of effective teams characterized by a set of qualities (e.g. respect, trust, shared responsibilities, values, and vision) and facilitators of the functioning of such teams
Access to care	Facilitation of timely access to healthcare that is tailored to the patient (e.g. decentralized services)
Coordination and continuity of care	Facilitation of healthcare that is well coordinated (e.g. regarding follow-up arrangements) and allows continuity (e.g. a well-working transition of care from inpatient to outpatient)
<b>Activities</b>	
Patient information	Provision of tailored information while taking into account the patient's information needs and preferences
Patient involvement in care	Active involvement of and collaboration with the patient regarding decisions related to the patient's health while taking into account the patient's preference for involvement
Involvement of family and friends	Active involvement of and support for the patient's relatives and friends to the degree that the patient prefers
Patient empowerment	Recognition and active support of the patient's ability and responsibility to self-manage his or her disease
Emotional support	A set of behavior that ensures physical support for the patient (e.g. pain management, assistance with daily living needs)
Emotional support	Recognition of the patient's emotional state and a set of behavior that ensures emotional support for the patient

- 3.23 impact factor

Scholl I, Zill JM, Härter M, Dirmaier J (2014) An Integrative Model of Patient-Centeredness – A Systematic Review and Concept Analysis. PLoS ONE 9(9): e107828. doi:10.1371/journal.pone.0107828 <http://journals.plos.org/plosone/article?id=info:doi/10.1371/journal.pone.0107828>

# Web-based Search Results

231 term entries representing 164 organizations worldwide.



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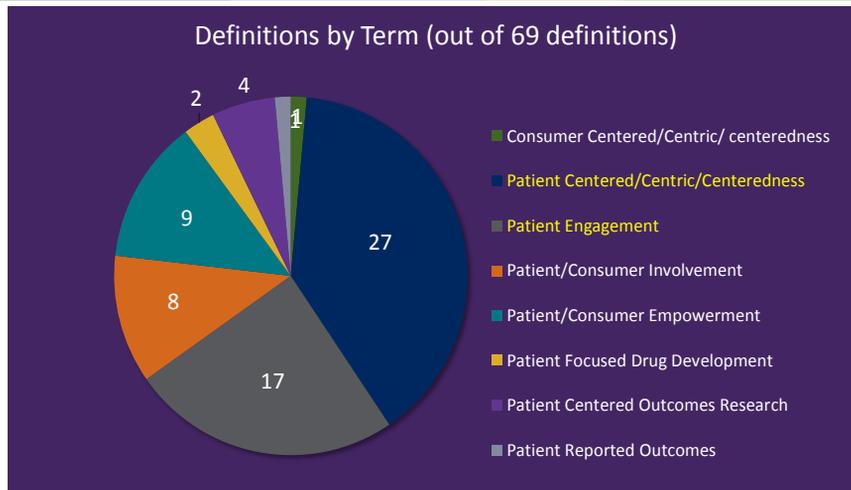
# Web-based Search Results

Out of 231 entries, 60 unique definitions of terms were identified, 133 terms used but not defined, and 37 organizations with no evidence of use of the searched terms.

	Government Agency (31 entries)	Private For-Profit (15 entries)		Private Not-for-Profit (185 entries)		
	Health Care/ Research Organization	Pharmaceutical Industry	Health Insurance	Volunteer Health Agencies	Membership Organizations	Health Care/ Research Organization
Unique definition of term(s)	17	3	1	10	12	17
Term(s) used, but not defined	13	11	--	46	51	12
No use of terms identified	--	--	--	15	22	--

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## Term Specific Results



“Patient Centered/Centric/Centeredness” and “Patient Engagement” are the most defined terms

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## Example Definitions: “Patient Centered/Centric/Centeredness”

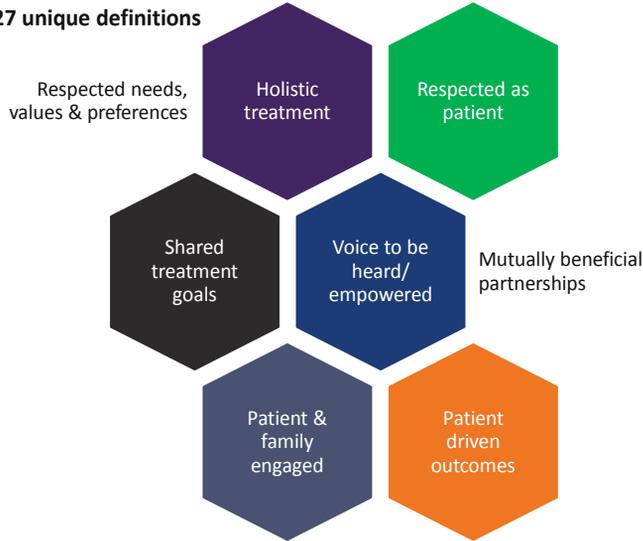
- “..**patient values** guide all clinical decisions..”
- “..decisions **respect patients’ wants, needs, preferences & values..**”
- “..**timely access** to healthcare that is **tailored to the patient..**”
- “..planning, delivery & evaluation of health care that **is grounded in mutually beneficial partnerships** among patients, families, & health care practitioners..”
- “.. people & their caregivers communicate & **make informed healthcare decisions, allowing their voices to be heard** in assessing the value of healthcare options..”
- “..**shared aspirations for treatment** and **acknowledgement of people’s life goals..**”
- “..**partnerships** that lead to the best outcomes, enhance the quality & safety of health care..”
- “.. 3 elements: **communication with patients; partnerships; and a focus beyond specific conditions**, on health promotion and healthy lifestyles..”
- “..treating the **whole person-** emotionally, physically & practically..”

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# Themes:

## “Patient Centered” and Related Definitions

Based on 27 unique definitions



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## Example Definitions:

### “Patient Engagement”

- Patients **taking ownership of their care**; bringing the voices of patients and people to the forefront of health care; and patient, family and community **partnerships with health professionals**
- “..knowledge, skills, ability, & willingness of **patients to manage and evaluate** their own and family members’ health and care..”
- “..those who become **active members of their healthcare team..**”
- “..providers **actively soliciting the knowledge, experience, judgment & values of patients..**”
- “..engaging patients & families in **making relevant, transparent, & effective decisions** about their care..”
- “..**active collaboration between patients and providers** to design, manage and achieve positive health outcomes...at various levels across the health care system..”
- “..**feedback from patients & their families/caregivers about their experience /engagement in decision making around care..**”
- “..a way for patients to become **partners in healthcare..**”
- “..patients **have a role in steering industry towards areas where new therapeutic options are needed** and are **engaged on all aspects related to the design of a clinical trial** to allow for patient-relevant clinical trial outcomes.
- “..patients are **well informed and expect that their voice is heard** by regulators when it comes to the way studies are designed and the **assessment of the benefits and risks of specific medicines..** “
- “..patients **directly reporting additional data** that stems from their **day-to-day experience living with their condition**

focused on research

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# Themes: “Patient Engagement” Definitions

Based on 17 unique definitions



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## Limitations

- Need to complete data capture (articles requested) and validation of approaches
- Concern over predominance of lower-tier publication reports
  - What does this mean?
- Dilemma of using “definition” or “defined as”
  - With, too limiting
  - Without, too broad

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## Limitations (2)

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- Informative definitions may have been missed
  - **Example:** Perfetto EM, Burke L, Oehrlein EM, Epstein RS. Patient-Focused Drug Development: A New Direction for Collaboration. *Med Care*. 2015 Jan;53(1):9-17. Proposed a definition of patient engagement:  
  
“systematic, methodologically sound actions taken to include patients in healthcare decision-making, health research and development and similar activities.”
- However, it didn't show up in the literature search!
  - Why? “Definition” or “defined as” *not* in title, keywords, or abstract
  - Implications!

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## Summary & Next Steps

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- Complete literature assessment
- Data quality checking
- Validating themes
- Stratify definitions by research versus health care
- Validating definitions (face validity with external experts)
- Coming up with our own definitions
  - Based on what we found
  - Based on what makes sense

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**Russell Wheeler,**  
Patient Advocate for Leber's  
Hereditary Optic  
Neuropathy, Winchester, UK

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# 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**

*Forsythe, et al. JGIM, 2015 & Peretto, ISPOR 2015 Annual Meeting*

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Thank you!

Questions?

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