“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.”

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

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
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*

Patient Engagement In Research Working Group

Co-Chairs:

François Houyéz, 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
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

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
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

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.
Acknowledgements

- University of Maryland, School of Pharmacy
  - Maya Hanna
  - Elisabeth Oehrlein
  - Francis Nguyen

- ISPOR Headquarters
  - Clarissa Cooblall
  - Theresa Tesoro

Building on Previous Work

Building on Previous Work

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.
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.
Draft Results (Nov. 10, 2015)

- 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

Nota Bene

- 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)
<table>
<thead>
<tr>
<th>Term</th>
<th>Definition</th>
</tr>
</thead>
<tbody>
<tr>
<td>1 patient centricity</td>
<td>“a dynamic process through which the patient regulates the flow of information to and from him/her via multiple pathways to exercise choices 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.”</td>
</tr>
<tr>
<td>2 patient centered care</td>
<td>“treating the patient as a unique individual.”</td>
</tr>
<tr>
<td>3 patient centeredness</td>
<td>“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.”</td>
</tr>
<tr>
<td>4 person centeredness</td>
<td>“(i) To address the person’s specific and holistic properties, (ii) to address the person’s difficulties in everyday life, (iii) to consider the person as an expert on their own condition and put the emphasis on participation and empowerment, and (iv) to respect the person ‘behind’ the impairment or the disease.”</td>
</tr>
<tr>
<td>5 patient-centred outcome measure</td>
<td>“It identifies outcomes that are desired and valued by individuals (patients). It is developed to reflect patient priorities. Measurement is undertaken at appropriate times and points within routine clinical care. The resultant information is used to inform the health-care professional/patient decision-making process, service evaluation, audit and planning.”</td>
</tr>
<tr>
<td>6, 7 public involvement</td>
<td>(1) “...emphasizes the involvement of lay people as taxpayers who may or may not have special knowledge of the subject under discussion. (2) “...the spectrum of processes and activities that bring the public into the decision-making process.”</td>
</tr>
<tr>
<td>8, 9 patient empowerment</td>
<td>(1) “A social process of recognizing, promoting and enhancing people’s abilities to meet their own personal needs, 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 patients work in partnership with the healthcare system to enable patients to become more responsible for, and involved in, their treatment and healthcare...developing the patient’s own skills and promoting a more balanced partnership in decision making.”</td>
</tr>
</tbody>
</table>

Example: Dimensions of Patient Centeredness

[Image of a table showing dimensions and brief descriptions of patient centeredness]

- 3.23 impact factor

### Web-based Search Results

231 term entries representing 164 organizations worldwide.

#### Privately Funded

<table>
<thead>
<tr>
<th>Type</th>
<th>N</th>
<th>Definition</th>
<th>No Definition</th>
</tr>
</thead>
<tbody>
<tr>
<td>For-Profit</td>
<td>5</td>
<td>4</td>
<td>1</td>
</tr>
<tr>
<td>Not-for-Profit</td>
<td>82</td>
<td>39</td>
<td>43</td>
</tr>
</tbody>
</table>

#### Government Funded

<table>
<thead>
<tr>
<th>Type</th>
<th>N</th>
<th>Definition</th>
<th>No Definition</th>
</tr>
</thead>
<tbody>
<tr>
<td>Definition</td>
<td>17</td>
<td></td>
<td></td>
</tr>
<tr>
<td>No Definition</td>
<td>14</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

#### 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.

<table>
<thead>
<tr>
<th></th>
<th>Government Agency (31 entries)</th>
<th>Private For-Profit (15 entries)</th>
<th>Private Not-for-Profit (185 entries)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Unique definition of term(s)</td>
<td>17</td>
<td>3</td>
<td>1</td>
</tr>
<tr>
<td>Term(s) used, but not defined</td>
<td>13</td>
<td>11</td>
<td>--</td>
</tr>
<tr>
<td>No use of terms identified</td>
<td>--</td>
<td>--</td>
<td>15</td>
</tr>
<tr>
<td></td>
<td>Health Care/Research Organization</td>
<td>Pharmaceutical Industry</td>
<td>Health Insurance</td>
</tr>
<tr>
<td></td>
<td>10</td>
<td>12</td>
<td>17</td>
</tr>
</tbody>
</table>
Term Specific Results

Definitions by Term (out of 69 definitions)

- Consumer Centered/Centric/centeredness
- Patient Centered/Centric/Centeredness
- Patient Engagement
- Patient/Consumer Involvement
- Patient/Consumer Empowerment
- Patient Focused Drug Development
- Patient Centered Outcomes Research
- Patient Reported Outcomes

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

Example Definitions:
“Patient Centered/Centric/Centeredness”

- \textit{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.
Themes:
“Patient Centered” and Related Definitions

Based on 27 unique definitions

- Respected needs, values & preferences
- Holistic treatment
- Respected as patient
- Shared treatment goals
- Voice to be heard/empowered
- Mutually beneficial partnerships
- Patient & family engaged
- Patient driven outcomes

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.”
Themes:
“Patient Engagement” Definitions

Based on 17 unique definitions

- Sharing experiences
- Driving research priorities
- Patient/Provider partnership
- Collaboration in health care and research
- Process of active solicitation
- Ownership of health care decisions
- Voice in decision-making
- Assessing risk/benefit

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
Limitations (2)

- Informative definitions may have been missed


    “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!

Summary & Next Steps

- 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
Russell Wheeler,  
Patient Advocate for Leber's Hereditany Optic Neuropathy, Winchester, UK

Summary & Next Steps

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- Coming up with our own definitions
  - Based on what we found
  - Based on what makes sense
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**

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Thank you!

Questions?