What has been the impact of patient engagement?

Nancy J. Devlin, PhD
Director of Research
Office of Health Economics
ISPOR President-Elect (2018-2019)
What has been the impact of patient engagement?

Patient engagement:
- “the process of building the capacity of patients, families, carers, as well as health care providers, to facilitate and support the active involvement of patients in their own care, in order to enhance safety, quality and people-centredness of health care service delivery.
- There are many definitions of patient engagement, but all share an underlying theme: the facilitation and strengthening of the role of those using services as coproducers of health, and health care policy and practice”  
  WHO (2016)
- The move toward greater patient engagement is trans-continental
- But what evidence do we have of its impact? What would ‘good impact’ look like?

Patient Engagement in HEOR at ISPOR

- Patient Council (Comprised of the chairs of the Roundtables)
  - Patient Representatives Roundtable Series (PRR)
    - North America, Europe, Latin America (since 2013)
    - Inaugural Asia Pacific PRR – Tokyo, Japan, September 2018
- Patient Representatives in ISPOR Scientific & Health Policy Groups
  - Members of the Health Science and Policy Council
  - Code of Ethics Task Force
  - Precision Medicine: Assessing the Value SIG Working Group
  - Patient Engagement in Research SIG Working Group
- Patient Centered Special Interest Group
  - Consensus generated ISPOR definition for “patient engagement in research”
- ISPOR Collaboration with Patient Organizations
  - EUPATI (European Patients’ Academy on Therapeutic Innovation) – ISPOR Developed the HTA Module
  - EURORDIS 2nd Multi-Stakeholder Symposium on Improving Patient Access to Rare Disease Therapies – February 22-23, 2017 – ISPOR served on the program committee
  - Patient Access Partnership (PACT)
Patient Council

**Mission:** to facilitate communication regionally among patient representatives, creating a global network for discussion, consensus building, and experience sharing around issues related to patient engagement in healthcare research.

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Patient Representatives Roundtables (PRR)

**Goal:** Provide a platform for patient representatives to interact with other health care stakeholders and determine how patients can effectively participate in research, the development and assessment of new health technologies, and health policy decision making.

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**Europe**
What is the impact of patient involvement in the Health Technology Assessment (HTA) process

**Latin America**
Who are the stakeholders involved, what is the patient’s role, and what are the barriers and opportunities to patient engagement in HTA?

**North America**
How are patients engaged in value assessment and what type of patient-provided information is or is not used by assessors and decision makers

**Asia Pacific**
How do we address the value and impact of patient-provided information and what are the barriers and opportunities
ISPOR Patient Engagement in Research Working Group of the ISPOR Patient Centered Special Interest Group

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, values and expertise.”

(Proposed Definition)

Speakers

Jan Geissler,
Co-Chair of EUPATI FUTURES team
Chair of ISPOR Patient Representatives Roundtable – Europe

Nicola Bedlington,
Secretary General at European Patients Forum (EPF)

Suzanne Schrandt,
Director of Patient Engagement at Arthritis Foundation
Chair of ISPOR Patient Representatives Roundtable – North America
Addressing Patient Involvement in R&D and Spheres of Influence in the Cancer Patient Community

Jan Geissler
Co-Chair of EUPATI FUTURES team
Chair of ISPOR Patient Representatives
Roundtable – Europe

Dimensions of Patient Advocacy Today

Provide patient support
Shape health & regulatory policy
Support and shape research
Patient Involvement in Clinical Research in Practice

- Patient priorities in risk/benefit
- Patient-relevant endpoints
- Choice of QoL/PRO instruments
- Inclusion/exclusion criteria ("real-world")
- Mobility / logistics
- Drug administration
- Diagnostics (frequency, necessity)
- Ethical aspects (eg, cross-over)

→ Increase patient relevance, recruitment, patient-relevant outcomes, publications
Example: Influence Sphere of the Cancer Patient Community

![Diagram showing influence spheres of different cancer and disease categories.](https://www.ispor.org)
Building knowledge to become equal partners in R&D: European Patients’ Academy (EUPATI)

- Launched Feb 2012 as a public private partnership, 33 consortium members, including ISPOR, initially funded by the Innovative Medicines Initiative, coordinated by the European Patients’ Forum as a permanent programme
- builds competencies & expert capacity among patients and the public
- has developed and is providing objective, credible, correct and up-to-date public knowledge about medicines R&D
  - EUPATI Toolbox in 9 languages, >1 million users from 217 countries
  - EUPATI Patient Expert Training Course (58 disease areas, 31 European countries, course 3 running)
- National Platforms in 18 European countries
- has been a game changer and driver of patient engagement in R&D

See http://www.eupati.eu

EUPATI training areas

1. Discovery of Medicines
2. Pre-clinical Development
3. Clinical Development
4. Clinical Trials
5. Regulatory Affairs, Drug Safety, Pharmacovigilance
6. Health Technology Assessment

Texts, illustrations, videos, mini-course PPTs, all under “Creative Commons License” for free use

See http://www.eupati.eu
Knowledge is power: Impact of the EUPATI Patient Expert Training course

<table>
<thead>
<tr>
<th>Role</th>
<th>Before</th>
<th>EUPATI</th>
<th>After</th>
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<tbody>
<tr>
<td>Member of patient organisation, not actively involved</td>
<td>17%</td>
<td></td>
<td>2%</td>
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<tr>
<td>Active role in a patient organisation</td>
<td>62%</td>
<td></td>
<td>71%</td>
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<tr>
<td>Leadership role in a patient organisation</td>
<td>62%</td>
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<td>71%</td>
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<tr>
<td>Employee of a patient organisation</td>
<td>25%</td>
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<td>23%</td>
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<tr>
<td>Volunteer role in a patient organisation</td>
<td>60%</td>
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<td>67%</td>
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<tr>
<td>Presenting at conferences, workshops etc</td>
<td>63%</td>
<td></td>
<td>83%</td>
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<tr>
<td>Advising a pharmaceutical company</td>
<td>13%</td>
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<td>44%</td>
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<td>Advising a regulatory agency</td>
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<td>42%</td>
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<tr>
<td>Advising a reimbursement agency</td>
<td>4%</td>
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<td>8%</td>
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Survey of graduates of the EUPATI course (2016)

Patients play an increasing role in all aspects of European healthcare, policy and research

Public perception

Competent authorities

Policy makers

Research ethics committees

HTA agencies and committees

Clinical research
How Patient Engagement has Changed the EU Regulatory and Health Policy Perspective

EPF For a strong European Patient Movement…
Patient Involvement in Health Policy

Pharmacovigilance Directive
(Direct Patient Reporting of Adverse Events)

Patient Involvement in Health Policy

Clinical Trials Regulation
- Informed consent
- Transparency
Patient Involvement in Health Policy

Current proposal on HTA cooperation post 2020
- Joint Clinical Assessment
- Patient Involvement

Patient Involvement in Health Policy

Exchange of expertise, know how, leapfrogging, ‘soft policy’
- patient safety,
- quality of care,
- digital health,
- patient empowerment,
- patient access, etc
Collaboration with patients: the EMA journey... so far

- **1995**: EMA created
- **1996**: Dialogue with HIV patients
- **2000**: Patients join COMP as full members
- **2003**: Working group with patients created
- **2005**: Framework of interaction with patient and consumer organisations
- **2006**: Patients and Consumers Working Party (PCWP) created
- **2014**: Dedicated Department created & framework updated
- **2017**: Public Hearing/Involvement of young people
- **Ongoing...**: Systematic inclusion of real life experience EMA regulatory output

EMA - Three categories of patient representation

- Management Board (MB)
- EMA Scientific Committee(s)
- Patients representing their organisations
- Patients representing their community
- Patients as individual experts
- Scientific Advice / Protocol Assistance Procedures
- Scientific Advisory / ad hoc Expert Groups
- Scientific Committee Consultations
- Review of Documents
EMA and ISPOR involvement in PARADIGM

- builds on EUPATI’s frameworks, guidance documents and processes

- provides a framework that will enable structured, effective, meaningful, ethical, innovative, and sustainable patient engagement and demonstrates the ‘return on the engagement’

- develops processes and tools for research priority setting, design of clinical trials, early dialogue, and produces a set of metrics to measure the impact of patient engagement

ISPOR is a valued member of the PARADIGM International Liaison Group (PILG)

See http://www.imi-paradigm.eu

From policy to practice

- Particular emphasis on vulnerable groups
  - People with Alzheimer’s – Alzheimer Europe
  - Children and Young People with Chronic Diseases – St Joan de Deu Hospital, Barcelona
Landscape of Patient Engagement in the US and Lessons Learned

Suzanne Schrandt, JD
Director, Patient Engagement
Arthritis Foundation
Chair, ISPOR Patient Representatives
Roundtable – North America

Patient Engagement in the US

Agenda
- History
- What’s Happening Today
- Evidence and Rationale
- Promising Practices
What type of Patient Engagement are we talking about?

Origins of Patient Engagement in the US

- Research
- Healthcare Delivery
- Patient Advocacy
- Drug Development

The majority [at least 51%] of the health center board members must be patients served by the health center. These health center patient board members must, as a group, represent the individuals who are served by the health center in terms of demographic factors, such as race, ethnicity, and gender.

Sources:
Journal of Urban Health 84(4):478-93, DOI: 10.1007/s11524-007-9165-7, Flicka, Travers, Guta
Community-Oriented Primary Care: Health Care for the 21st Century, Ryne, Bogue, Kulkulka, Fulmer
Section 330(k)(3)(H) of the PHS Act; and 42 CFR 51c.304 and 42 CFR 56.304
ACTUP website accessed at https://actupny.com
Patient Engagement Now

Research
Drug/Device Development
Healthcare Delivery
Clinical Training

Patient-Centered Outcomes Research Institute (PCORI)
- Funder of CER
- Requires involvement of patient experts/partners
  - Engagement must exist in
    - Planning
    - Conduct
    - Dissemination
### Patient Engagement Now

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<tr>
<th>Research</th>
<th>FDA’s Patient-Focused Drug Development Initiative</th>
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<td>• FDA Guidances</td>
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<td>• PFDD Meetings</td>
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<td>• Patient Engagement Advisory Committee (CDRH)</td>
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<td>• Patient Engagement Collaboratives (PEC)</td>
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<th>Patient and Family Advisory Councils (PFACs)</th>
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<td>• Clinical Guidelines Development</td>
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<td>• Patient Safety</td>
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Patient Engagement Now

Pre and Post Graduate Clinical Training
- Involving patients as educators and speakers
- Including patients in creation and delivery of curriculum

Evidence: How Patient Engagement Works

Source: PCORI website accessed at https://pcori.org
Evidence: How Patient Engagement Works

Research

Drug/Device Development

Healthcare Delivery

Clinical Training

Source: PCORI website accessed at https://pcori.org

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Evidence: How Patient Engagement Works

| Table 5. Ratio of Reduction in Cost to Launch, Gain in ENPV, and Gain in NPV to a $100,000 Investment in Patient Engagement. |
|---|---|---|---|
| | Avoiding an Amendment | Improving Patient Experience | Combined |
| Pre-phase 2 | | | |
| Cost gain | 5× | – | 5× |
| ENPV gain | 38× | 301× | 349× |
| NPV gain | 245× | 382× | 619× |
| Pre-phase 3 | | | |
| Cost gain | 21× | – | 21× |
| ENPV gain | 150× | 570× | 750× |
| NPV gain | 320× | 309× | 649× |

Abbreviations: ENPV, expected net present value; NPV, net present value.

Evidence: How Patient Engagement Works

Research
Drug/Device Development
Healthcare Delivery
Clinical Training

Errors
Outcomes

Evidence: How Patient Engagement Works

Research
Drug/Device Development
Healthcare Delivery
Clinical Training
Promising Practices: Lessons Learned

- Don’t dabble!
- Engagement ≠ Recruitment
- Early and often
- Data is good, data + humans = great
- Bi-directional capacity-building

Get started today!

- Patient-Centered Outcomes Research Institute (PCORI) Engagement in Research literature database: [www.pcori.org](http://www.pcori.org)
- TOPPER Exchange/Engagement Toolkit: [https://www.hipxchange.org](https://www.hipxchange.org)
- Campus-Community Partnerships for Health: [www.ccphealth.org](http://www.ccphealth.org)
- Preparing for Roles as Expert Patients (PREP) Training: [www.arthritis.org](http://www.arthritis.org)
- Roadmap for Patient and Family Engagement: [https://air.org](https://air.org)
- Detroit Urban Research Center: [https://www.detroiturc.org](https://www.detroiturc.org)
- Community Tool Box, University of Kansas: [https://ctb.ku.edu](https://ctb.ku.edu)
- Institute for Patient and Family Centered Care (PFACs resource): [www.ipfcc.org](http://www.ipfcc.org)
- United States Food & Drug Administration Patient-Focused Drug Development: [https://www.fda.gov](https://www.fda.gov)
- Accreditation Council for Continuing Medical Education, Patient Engagement Criterion 24: [www.acme.org](http://www.acme.org)
- "Patient and public involvement in clinical guidelines: international experiences and future perspectives": [qualitysafety.bmj.com](http://qualitysafety.bmj.com)
- FasterCures Patient Engagement Resource Library: [www.fastercures.org](http://www.fastercures.org)
- Patient-Focused Medicines Development Resources and Quality Guidance: [https://patientfocusedmedicine.org](https://patientfocusedmedicine.org)
- DIA Patient Engagement Resources, Annual Meeting Track, Patient-Centered Drug Development Conferences: [www.globaldia.org](http://www.globaldia.org)
- Clinical Trials Transformation Initiative: [https://www.ctti-clinicaltrials.org](https://www.ctti-clinicaltrials.org)
ISPOR, the professional society for health economics and outcomes research (HEOR), is an international, multistakeholder, nonprofit dedicated to advancing HEOR excellence to improve decision-making for health globally. The Society is the leading source for scientific conferences, peer-reviewed and MEDLINE-indexed publications, good practices guidance, education, collaboration, and tools/resources in the field.

ISPOR's community of more than 20,000 individual and chapter members from 120+ countries includes a wide variety of healthcare stakeholders, including researchers, academicians, regulators and assessors, public and private payers, healthcare providers, industry, and patient representatives. The Society's leadership has served as an unbiased resource and catalyst for innovation in the field for more than 20 years.

www.ispor.org