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Improving healthcare decisions

IP15: TRANSFORMING HEALTHCARE: THE IMPACT OF PATIENT ENGAGEMENT

ISPOR Europe 2018 – Barcelona, Spain

13 November 2018 | 14:00 - 15:00 | Room: 113+114 (P1)

WiFi Network: ISPOR | Password: CERTARA2018

Nancy J. Devlin, PhD
Director of Research
Office of Health Economics

ISPOR President-Elect (2018-2019)

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What has been the impact of patient engagement?

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)

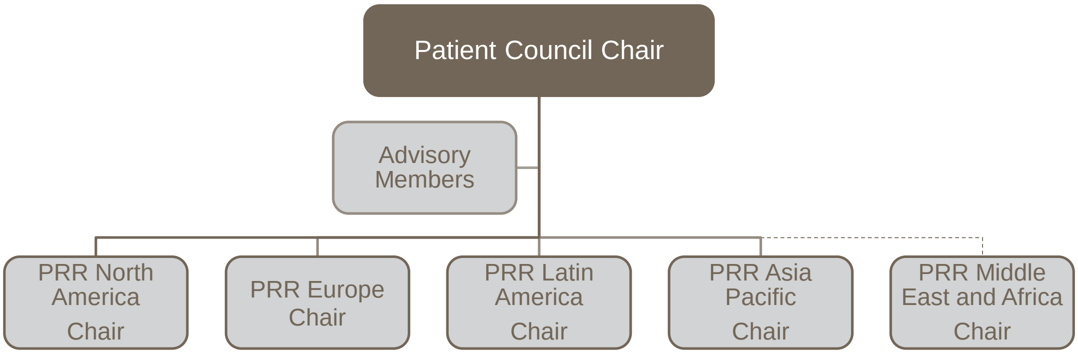
- The move toward greater patient engagement is **trans-continental**
- **But what evidence do we have of its impact? What would 'good impact' look like?**

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.



Patient Council Chair

Advisory Members

PRR North America Chair

PRR Europe Chair

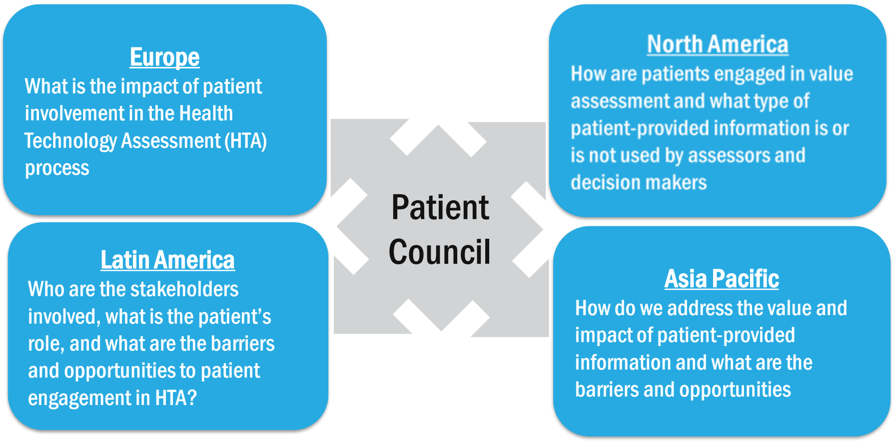
PRR Latin America Chair

PRR Asia Pacific Chair

PRR Middle East and Africa Chair

Patient Representatives Roundtables (PRR)

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



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

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

Patient Council

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?

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

Jan Geissler
Co-Chair of EUPATI FUTURES team

Chair of ISPOR Patient Representatives
Roundtable – Europe

Addressing Patient Involvement in R&D and Spheres of Influence in the Cancer Patient Community

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Dimensions of Patient Advocacy Today

Provide
patient support

Shape
health & regulatory policy

Support and shape
research

Patient Involvement in Clinical Research in Practice

Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap. Geissler, Ryll, Leto, Uhlenhopp, Therapeutic Innovation & Regulatory Science (2017), doi: 10.1177/2168479017706405, and at www.eupati.eu

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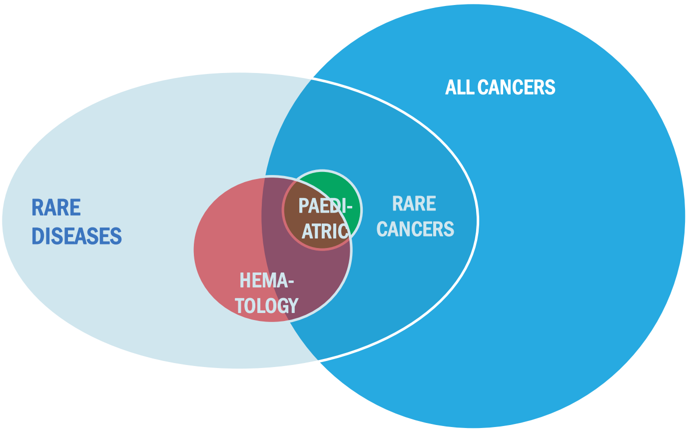
Patient Involvement in Clinical Research in Practice

- 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)

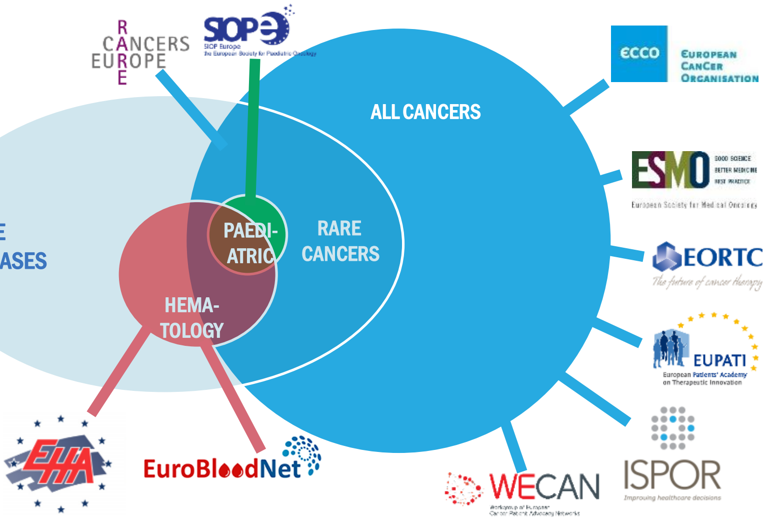
→ Increase patient relevance, recruitment, patient-relevant outcomes, publications

Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap. Geissler, Ryll, Leto, Uhlenhopp, Therapeutic Innovation & Regulatory Science (2017), doi: 10.1177/2168479017706405, and at www.eupati.eu

Example: Influence Sphere of the Cancer Patient Community



Example: Influence Sphere of the Cancer Patient Community



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

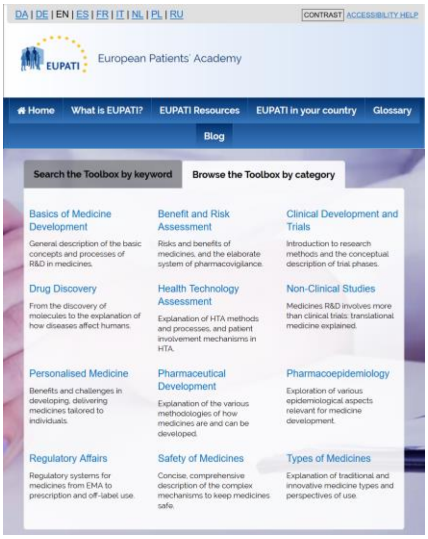
EUPATI
European Patients' Academy
on Therapeutic Innovation

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



Basics of Medicine Development
General description of the basic concepts and processes of R&D in medicines.

Benefit and Risk Assessment
Risks and benefits of medicines, and the elaborate system of pharmacovigilance.

Clinical Development and Trials
Introduction to research methods and the conceptual description of trial phases.

Drug Discovery
From the discovery of molecules to the explanation of how diseases affect humans.

Health Technology Assessment
Explanation of HTA methods and processes, and patient involvement mechanisms in HTA.

Non-Clinical Studies
Medicines R&D involves more than clinical trials: translational medicine explained.

Personalised Medicine
Benefits and challenges in developing, delivering medicines tailored to individuals.

Pharmaceutical Development
Explanation of the various methodologies of how medicines are and can be developed.

Pharmacoepidemiology
Exploration of various epidemiological aspects relevant for medicine development.

Regulatory Affairs
Regulatory systems for medicines from EMA to prescription and off-label use.

Safety of Medicines
Concise, comprehensive description of the complex mechanisms to keep medicines safe.

Types of Medicines
Explanation of traditional and innovative medicine types and perspectives of use.

See http://www.eupati.eu

Knowledge is power:
Impact of the EUPATI Patient Expert Training course

Role	Before	EUPATI	After
Member of patient organisation, not actively involved	17%	→	2%
Active role in a patient organisation	62%	→	71%
Leadership role in a patient organisation	62%	→	71%
Employee of a patient organisation	25%	→	23%
Volunteer role in a patient organisation	60%	→	67%
Presenting at conferences, workshops etc	63%	→	83%
Advising a pharmaceutical company	13%	→	44%
Advising a regulatory agency	21%	→	42%
Advising a reimbursement agency	4%	→	8%

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

Driving force
Co-researcher
Reviewer
Advisor
Info provider
Research subject

Clinical research

Nicola Bedlington
Secretary General
European Patients Forum (EPF)

www.ispor.org

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

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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*

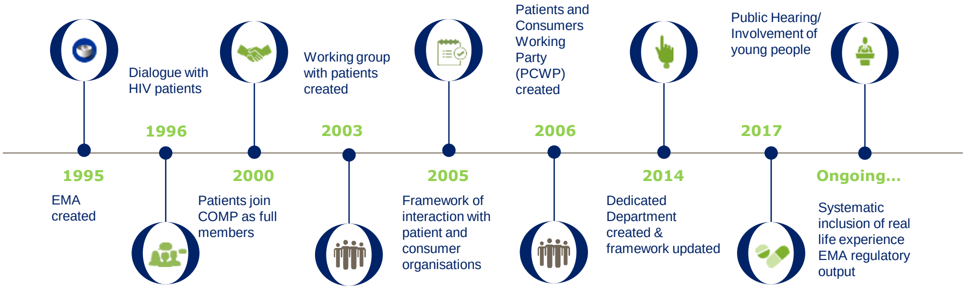
-*Patient Involvement*

Patient Involvement in Health Policy

Exchange of expertise, know how, leap-frogging, ‘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

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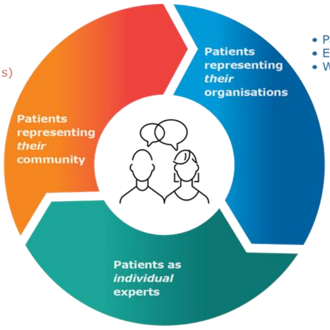
EMA - Three categories of patient representation



- Management Board (MB)
- EMA Scientific Committee(s)

- Patients' and Consumer's Woking Party (PCWP)
- EMA Consultations
- Workshops

- Scientific Advice / Protocol Assistance Procedures
- Scientific Advisory / ad hoc Expert Groups
- Scientific Committee Consultations
- Review of Documents

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EMA and ISPOR involvement in PARADIGM

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

- **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

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

- Particular emphasis on vulnerable groups
  - People with Alzheimer's – Alzheimer Europe
  - Children and Young People with Chronic Diseases – St Joan de Deu Hospital, Barcelona

Research and priority setting

Design of clinical trials

Focus areas

Early dialogues with regulators and HTA bodies www.ispor.org

Suzanne Schrandt, JD
Director, Patient Engagement
Arthritis Foundation

Chair, ISPOR Patient Representatives
Roundtable – North America

Landscape of Patient Engagement in the US and Lessons Learned

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Patient Engagement in the US

Agenda

- History
- What's Happening Today
- Evidence and Rationale
- Promising Practices

$$\frac{1}{1,000,000}$$

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 health center. These health center patie individuals who are served by the health ethnicity, and gender.

patients[4] served by the up, represent the factors, such as race,

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, Rhyne, 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 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

- Research
- Drug/Device Development
- Healthcare Delivery
- Clinical Training

FDA's Patient-Focused Drug Development Initiative

- FDA Guidances
- PFDD Meetings
- Patient Engagement Advisory Committee (CDRH)
- Patient Engagement Collaboratives (PEC)

Patient Engagement Now

- Research
- Drug/Device Development
- Healthcare Delivery
- Clinical Training

- Patient and Family Advisory Councils (PFACs)
- Clinical Guidelines Development
- Patient Safety

Patient Engagement Now

Research

Drug/Device Development

Healthcare Delivery

Clinical Training

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

Research

Drug/Device Development

Healthcare Delivery

Clinical Training

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

Evidence: How Patient Engagement Works

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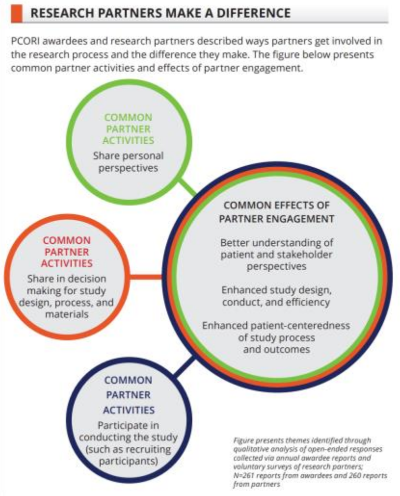


RESEARCH PARTNERS MAKE A DIFFERENCE

PCORI awardees and research partners described ways partners get involved in the research process and the difference they make. The figure below presents common partner activities and effects of partner engagement.

Figure presents themes identified through qualitative analysis of open-ended responses collected via annual awardee reports and voluntary surveys of research partners; N=261 reports from awardees and 260 reports from partners

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

Evidence: How Patient Engagement Works

Research

Drug/Device Development

Healthcare Delivery

Clinical Training

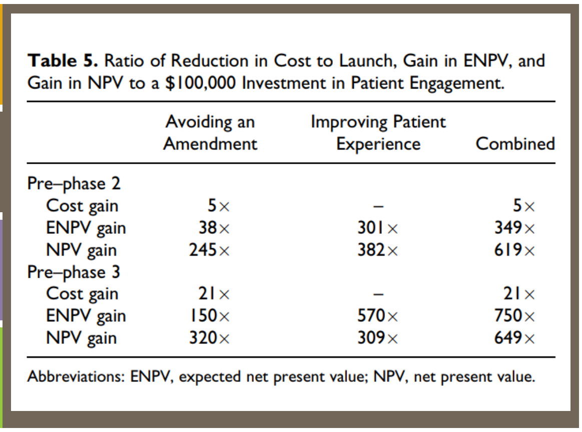


Table 5. Ratio of Reduction in Cost to Launch, Gain in ENPV, and Gain in NPV to a $100,000 Investment in Patient Engagement.

	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.

Source: Levitan, Getz, et al. accessed at https://journals.sagepub.com/doi/pdf/10.1177/2168479017716715

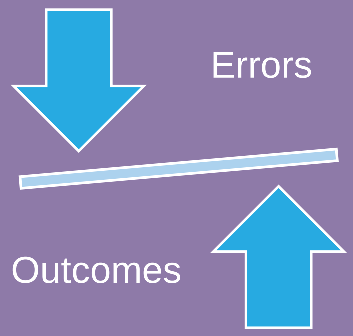
Evidence: How Patient Engagement Works

Research

Drug/Device Development

Healthcare Delivery

Clinical Training



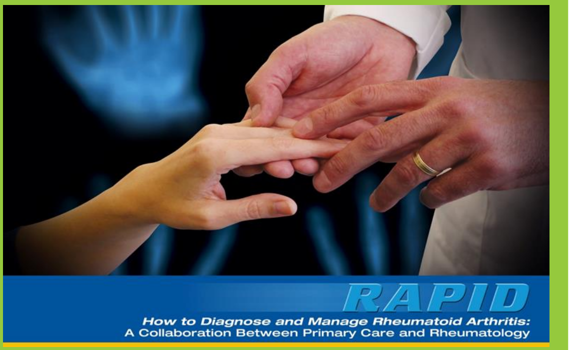
Evidence: How Patient Engagement Works

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!

(This list is not exhaustive)

- Patient-Centered Outcomes Research Institute (PCORI) Engagement in Research literature database: www.pcori.org
- PCORI Engagement Rubric: “The PCORI Engagement Rubric: Promising Practices for Partnering in Research", Ann Fam Med 2017;15: 165-170; https://doi.org/10.1370/afm.2042
- “Conceptual and practical foundations of patient engagement in research at the patient-centered outcomes research institute”, Quality of Life Research. (2015) 10.1007/s11136-014-0893-3
- TOPPER Exchange/Engagement Toolkit: https://www.hipxchange.org
- Campus-Community Partnerships for Health: www.ccphealth.org
- Preparing for Roles as Expert Patients (PREP) Training: www.arthritis.org
- Roadmap for Patient and Family Engagement: https://air.org
- Detroit Urban Research Center: https://www.detroiturc.org
- Community Tool Box, University of Kansas: https://ctb.ku.edu
- Institute for Patient and Family Centered Care (PFACs resource): www.ipfcc.org
- United States Food & Drug Administration Patient-Focused Drug Development: https://www.fda.gov
- Medical Device Innovation Consortium, Science of Patient Input: www.mdic.org
- Accreditation Council for Continuing Medical Education, Patient Engagement Criterion 24: www.acme.org
- “Patient and public involvement in clinical guidelines: international experiences and future perspectives”: qualitysafety.bmj.com
- FasterCures Patient Engagement Resource Library: www.fastercures.org
- Patient-Focused Medicines Development Resources and Quality Guidance: https://patientfocusedmedicine.org
- DIA Patient Engagement Resources, Annual Meeting Track, Patient-Centered Drug Development Conferences: www.globaldia.org
- Clinical Trials Transformation Initiative: 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.

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