Patient-Centered Outcomes Research (PCOR)

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PCOR Outline

- Terminology.
- Rationale.
- History.
- Methodology.
- Resources – UTMB & beyond.
PCOR Terminology

- **Patient-centered**: addresses questions & concerns most relevant to patients.

- **Engagement**: meaningful involvement of patients & other stakeholders throughout the research process.

- **Shared decision-making**: collaboration between clinician & patient to make informed treatment decisions.

- **Mixed methods**: integrating qualitative & quantitative research methodologies & information.

- **Patient-reported outcomes**: self-report of a person’s health condition/status without interpretation of his or her response by a clinician or anyone else.
Rationale for PCOR

“Healthcare outcomes research seeks to understand the end results of particular health care practices and interventions. End results include effects that **people experience and care about**, such as change in the ability to function... where cure is not always possible – end results include quality of life as well as mortality.” (AHRQ 2000)

i.e... 1) **Perceived by patient**, & 2) **important to patient**.

- “.. cannot actually measure health in a laboratory.” (Kane & Radosevich 2010)
- Focuses on what we ultimately want healthcare to achieve → improved functioning & better QOL.

http://www.ahrq.gov/clinic/outfact.htm
Measuring Health

- Health (in US) is often measured narrowly & from a deficit perspective; e.g. morbidity or mortality.

- “Health is a state of complete physical, mental and social well-being and not merely the absence of disease or infirmity.” (WHO 1946)

  - Dynamic state measured along a continuum.
International Classification of Functioning, Disability, & Health
WHO – 2001

Health condition
(disorder or disease)

Body Functions & Structure ← Activity ← Participation

Contextual factors

Environmental Factors

Personal Factors
HRQOL

- Subjective assessments of both positive & negative aspects of life.

- Universal understanding, but no consensus definition.

- Complex concept: current health status is obviously an important factor, but so too are employment, education, housing, community, culture, values, & spirituality.

- Includes both physical & mental functioning.
As an oncologist, when I sit with patients to discuss starting a new chemotherapy regimen, their first questions are often “How will it make me feel?” and “How did patients like me feel with this treatment?” Regrettably, this information is generally missing from U.S. drug labels and from published reports of clinical trials – the two information sources most commonly available to people trying to understand the clinical effects of cancer drugs.
Effect of Ramipril on Walking Times and Quality of Life Among Patients With Peripheral Artery Disease and Intermittent Claudication
A Randomized Controlled Trial

JAMA, February 6, 2013—Vol 309, No. 5

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**Objective** To determine the efficacy of ramipril for improving walking ability, patient-perceived walking performance, and quality of life in patients with claudication.

**Conclusions and Relevance** Among patients with intermittent claudication, 24-week treatment with ramipril resulted in significant increases in pain-free and maximum treadmill walking times compared with placebo. This was associated with a significant increase in the physical functioning component of the SF-36 score.
45 year-old twins euthanized because they were going blind

Identical twin brothers who were born deaf were killed by Belgian doctors after seeking euthanasia when they found out they would also soon go blind because of a genetically caused form of glaucoma. The 45-year-old brothers from Antwerp, Belgium, chose death because they were frightened of losing their independence in an institution and had “nothing to live for,” their brother...

Nathan Verhelst Chooses Euthanasia After Failed Gender Reassignment Surgeries

A 44-year-old man in Belgium chose to end his life via euthanasia on Monday after a series of failed gender reassignment surgeries. “I was the girl no one wanted,” Nathan Verhelst told the Flemish newspaper Het Laatste Nieuws, mere hours before his death. Verhelst, who was born female, told the paper’s reporters he grew up in an unloving family, feeling like a boy trapped in a...

Belgium looks at euthanasia for minors, Alzheimer's sufferers

The proposed changes to the law were submitted to parliament Tuesday by the Socialist party and are likely to be approved by other parties, although no date has yet been put forward for a parliamentary debate. "The...
Funding Agency Missions

- NIH: *seek fundamental knowledge about the nature & behavior of living systems & the application of that knowledge* to enhance health, lengthen life, & reduce illness and disability.

- NIDILRR: *generate new knowledge & promote its effective use to improve the abilities of people with disabilities to perform activities of their choice in the community, & also to expand society’s capacity to provide full opportunities & accommodations for its citizens with disabilities.*
Funding Agency Missions

- AHRQ: produce evidence to make health care safer, higher **quality**, more **accessible**, **equitable**, & affordable, & to work... with other partners to make sure that the evidence is **understood** & **used**.

- PCORI: helps people make informed healthcare decisions, & improves healthcare delivery & outcomes, by producing & promoting high-integrity, evidence-based information that comes from **research guided by patients, caregivers, & the broader healthcare community**.

- RWJF: improve the health & health care of all Americans.
Provider Quality Reporting

- CMS hospital, nursing home, home health agency, & physician quality reporting systems all include patient surveys on perceived quality & effectiveness of care.


  [Link](https://www.medicare.gov/hospitalcompare/profile.html#profTab=1&ID=450018&cmprID=450018&dist=50&loc=77555&lat=29.3106102&lng=-94.7772493&cmprDist=0.3&Distn=0.3)

NQF has endorsed 24 measures related to Person- & Family- Centered Care.
History of PCOR

- **Bayh-Dole Act Technology Transfer at Universities & Medical Centers (1980)**
- **AHRQ Effective Health Care Program (2003)**
- **AHRQ (“AHCPR”) (1989)**
- **PROMIS (2004)**
- **NIH Roadmap “Translational Research” (2004)**
- **NIH Roadmap (2004)**
- **FDA Guidance for PROs (2006)**
- **PCORI (2010)**
- **First CTSAs (2009)**
- **PCORI Methodology Standards (2012)**
- **ARRA Funding $1.1B CER (2009)**

Timeline:
- 1980
- 1990
- 2000
- 2010
- 2020
Guidance for Industry
Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)

December 2009
Clinical/Medical

Patient Reported Outcomes Measurement Information System... funded through NIH Common Fund 2004.

Set of reliable, precise measures of patient-reported health status for physical, mental, & social well-being.

Measures what patients are able to do & how they feel.

Provide information about the effects of therapy that cannot be found in traditional clinical measures.

Intended to compliment traditional measures of health.

http://www.nihpromis.org/
2013 Roundtable on Value & Science-Driven Health Care
Explored issues, attitudes, & approaches to increasing patient engagement in & demand for 1) shared decision making & better communication about the evidence in support of testing & treatment options, 2) the best value from the health care they receive, & 3) the use of data generated in the course of their care experience for care improvement.
We support research that will provide reliable, useful information to help people make informed healthcare decisions and improve healthcare delivery and outcomes.

This research answers patient-centered questions, such as:

- “Given my personal characteristics, conditions, and preferences, what should I expect will happen to me?”
- “What are my options, and what are the potential benefits and harms of those options?”
- “What can I do to improve the outcomes that are most important to me?”
- “How can clinicians and the care delivery systems they work in help me make the best decisions about my health and health care?”

http://www.pcori.org/
PCORI Priorities

- Comparative Clinical Effectiveness Research (CER).
  - Compare outcomes to determine the effectiveness, including risks and benefits, of two or more approaches to health care.

- CER Methods and Infrastructure.
  - Improve methods for patient-centered CER.
  - Development of a large, highly representative electronic-data infrastructure, called PCORnet, for improving the conduct of patient-centered CER.
PCORI Conditions of Interest

- Affect large numbers of people across a range of populations.
- Place a heavy burden on individuals, families, specific populations, & society.
- Rare diseases, which are difficult to study.
PCORI Populations of Interest

- Racial & ethnic minorities.
- Older adults.
- Low-income.
- Residents of rural areas.
- Women.
- Children.
- Individuals with special healthcare needs; e.g. disabilities, multiple chronic diseases, & genetic conditions.
- Patients with low health literacy/numeracy and limited English proficiency.
- Lesbian, gay, bisexual, transgender persons.
- Veterans & members of the armed forces and their families.
PCORI Methodology Standards for...

1. Formulating research questions.
2. Patient-centeredness.
3. Data integrity & rigorous analyses.
4. Preventing & handling missing data.
5. Heterogeneity of treatment effects.
6. Data registries.
7. Data networks as research-facilitating structures.
10. Studies of diagnostic tests.
11. Systematic reviews.

http://www.pcori.org/research-results/research-methodology
Welcome and Overview

James Hulbert, Brigette Scott, and Scott Solomon
Contracts Management and Administration

February 19, 2015

PCORI’s Past Funding Rates

Current Funding Commitments

Patient-Centered Outcomes Research Institute

Health
Preliminary Competencies for Comparative Effectiveness Research

Jodi B. Segal, M.D.¹, Wishwa Kapoor, M.D.², Timothy Carey, M.D.³, Pamela H. Mitchell, Ph.D.⁴, Michael D. Murray, PharmD⁵, Kenneth G. Saag, M.D.⁶, Glen Schumock, PharmD⁷, Daniel Jonas, M.D.⁸, Michael Steinman, M.D.⁹, Rosemarie Filart, M.D., M.P.H.¹⁰, Morris Weinberger, Ph.D.¹¹, and Harry Selker, M.D.¹²

WWW.CTSJOURNAL.COM    VOLUME 5 • ISSUE 6

Figure 1. Groups of individuals who may benefit from acquiring competency in the skills of CER. Dark boxes represent individuals within the CER workforce; light boxes represent individuals outside of the CER workforce but within the translational pathway.

- Lists (10) foundational & (20) applied competencies for CER investigators & research teams.
- Starting point for organizations developing curricula to teach the necessary knowledge & skills.
- R24 *Infrastructure Development Grant*... transform UTMB into a center of rigorous, high quality, CER & PCOR, focused on healthcare decision-making by older adults, their providers, & the health systems providing their care.

- Funding: Agency for Healthcare Research & Quality (AHRQ).

- PI: Jim Goodwin, MD.
- Crowder: Community Dialogue to Identify Ethical Issues in PCOR.
- Reistetter: Comparative Effectiveness of Patient-Centered Outcomes Following IRF & SNF Stroke Rehabilitation.

Graham: Improving healthcare for elders during disasters.


Sidossis: Patient- & family-centered home nutrition program for burned children.
Collaborate with knowledgeable investigators...

Monique Pappadis, PhD
John Prochaska, DrPH
Susan Weller, PhD

Comparative Effectiveness Research Training & Instruction (CERTaIN) – R25 at MD Anderson.

http://www.utmb.edu/pcor/resources.asp
Is Obamacare’s Research Institute Worth The Billions?

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