

Patient Centered Outcomes Research

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“Why are we doing research and what are we hoping to accomplish?”

Godat LN, et al. *TASCO*. 2020 Feb 4;5(1):e000422.

PCOR

- Many researchers would answer this question with a response related to **impact**.
- **Impact** can be measured in multiple ways
- One of which is by measuring through the eyes of patients and community stakeholders **the outcomes that *they* perceive to be most relevant**.
- This principle is central to doing research that is patient centered.

Godat LN, et al. TASC0. 2020 Feb 4;5(1):e000422.

PCOR



Patient-centered outcomes research focuses the attention on **patient's** beliefs, preferences, and needs, in contrast to physician-centered care



Thus, **active participation** of the patient as a stakeholder is an essential element of patient-centered outcomes research

Bardes CL. NEJM, 2012;366, 782-783.
Frank L, Basch E, Selby JV. JAMA, 2014;312, 1513-1514.

By engaging stakeholders and focusing on outcomes and processes that are prioritized by those who are most impacted, PCOR also has the effect of moving the plans for **effective implementation and **dissemination** of processes and policies **upstream** in the research development.**

Godat LN, et al. *TASCO*. 2020 Feb 4;5(1):e000422.

PCORI

- In 2010, Congress authorized the establishment of the Patient-Centered Outcomes Research Institute –PCORI
- Publicly supported, independent, nonprofit research institute
- PCORI funds CER addresses questions and concerns important to patients, generating and disseminating evidence that is relevant, trustworthy, and useful to patients and others it serves
- In late 2019, Congress reauthorized PCORI funding for another 10 years

PCORI – Strategic Goals

- Increase quantity, quality, and timeliness of useful, trustworthy research information available to support health decisions.
- Speed the implementation and use of patient-centered outcomes research evidence.
- Influence research funded by others to be more patient centered.

Research Done Differently®

- There are four essential elements to this approach:
- **Funding of patient-centered CER**
 - to address questions important to patients and other stakeholders and to generate meaningful evidence that will allow them to make better informed health and healthcare decisions.
- **Engagement**
 - involving patients and all key health decision makers across the continuum of PCORI's work, from research topic selection to dissemination and implementation of results, to produce research findings that are useful, relevant, and trustworthy to those we serve.
- **Dissemination and Implementation**
 - to ensure that the results of PCORI-funded studies are easily accessible and can be used by those who need them to make better informed decisions on health and healthcare.
- **Research Infrastructure**
 - including enhancing the ability of PCORnet® to improve capacity to conduct clinical research more efficiently, improving the science and methods of CER, and supporting the continuing development of the health research workforce.

Examples

PROs

- PROs are defined by the FDA and NQF as “... a report that comes directly from the patient (i.e., study subject) about the status of a patient’s health condition without amendment or interpretation of the patient’s response by a clinician or anyone else.”
- PROs are distinct from other patient outcomes such as physiological measures, process measures, clinician-reported measures, and caregiver-reported measures.
- Reflects a variety of information reported directly by the patient, such as
 - Health-related quality of life
 - Functional status
 - Symptoms
 - Treatment adherence

Snyder CF, et al. *Med Care*. 2013; 51(803): S73–S79.
U S. Food and Drug Administration. *Federal Register*. 2009;74(35):65132–3.
National Quality Forum. *Patient-Reported Outcomes*.
Acquadro C, et al. *Value Health*. 2003;6:522–31.

PROs

- PROMIS® (Patient-Reported Outcomes Measurement Information System) is a set of person-centered measures that evaluates and monitors physical, mental, and social health in adults and children.
- It can be used with the general population and with individuals living with chronic conditions.

<https://www.healthmeasures.net/explore-measurement-systems/promis/intro-to-promis>



It started with a “discussion



Which turned into a collaboration

Patient preferences

- Discrete Choice Experiments (DCE) are a quantitative technique used to measure individual preferences in a variety of healthcare settings by administering surveys that ask individuals to choose the best option between two or more hypothetical scenarios or choice sets.
- Options are described with a fixed set of attributes levels that vary in each scenario.
- The data collected can be used to assess the relative importance of each

Haac B, et al. *BMJ Open* 2017;7:e016676

Patient preferences

- A DCE was prospectively administered to 223 orthopaedic trauma patients at a level 1 trauma center.
- Patients preferred oral tablets over subcutaneous injections (marginal utility, 0.16; 95% CI: 0.11 - 0.21, $P < 0.0001$)
- Preferences changed in favor of subcutaneous injections with an absolute risk reduction of
 - 6.98% in bleeding
 - 4.53% in wound complications requiring reoperation
 - 1.27% in VTE
 - **0.07% in death from PE**

Haac B, et al. BMJ Open 2017;7:e016676

PREVENT CLOT

- The primary outcome was all-cause mortality.
- Evaluation of non-inferiority by testing whether the intention-to-treat difference in the probability of dying within 90 days of randomization between aspirin and LMWH is less than our non-inferiority margin of 0.75%.
- Secondary efficacy outcomes include
 - cause-specific mortality
 - non-fatal PE
 - DVT

O'Toole RV, et al. *BMJ Open* 2021;11:e041845.

I-REP Objectives

- Establish stakeholder Injury Research Engagement Panel (I-REP) to partner in PCOR/CER from conception to dissemination
- Develop emergency-setting-informed consent strategies and methods for increasing follow-up engagement
- Conduct reciprocal education between patients and researchers

Project Outcomes

Dissemination of findings to stakeholders to inform patient-centered outcomes and comparative effectiveness research (PCOR/CER) to improve engagement strategies/outcomes

Creation of sustainable Injury Research Engagement Panel (I-REP) to engage stakeholders in trauma research, ensuring appropriate research methods and questions

Development of patient/family-centered communications on participating in trauma PCOR/CER

Sustainment of the I-REP as an integral component of CNTR's PCOR/CER efforts

Thank you!
Questions?