Which Methods, When and How?

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Overview

• Provide an overview of patient engagement methods approaches
• Discuss methods in the context of the 10-step framework
• Review patient-engagement methods examples
When?
As part of the 10-step Framework For Continuous Patient Engagement In Research*

A. PLANNING RESEARCH
1. Topic Solicitation
2. Prioritization
3. Framing the Question

B. DOING IT
4. Selection of Comparators and Outcomes
5. Creation of Conceptual Framework
6. Analysis Plan
7. Data Collection

C. DELIVERING SOLUTIONS
8. Reviewing & Interpreting Results
9. Translation
10. Dissemination

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Revisiting the Roadmap: Examples of Engagement Methods

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Patient Engagement in CER/PCOR

- Elicitation
- Validation
- Conceptualization
- Prioritization
Overview of Methods: Elicitation

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<td>Focus group</td>
<td>Semi-structured group interview process moderated by a group leader – <strong>used for breadth</strong>, not necessarily depth.</td>
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<td>Structured interview</td>
<td>Uses a <strong>schedule of questions planned in advance</strong> and which <strong>do not deviate based on responses</strong>.</td>
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<td>Semi-structured interview</td>
<td>A type of interview used to elicit information to achieve a holistic understanding of the interviewee’s point of view or situation; involves asking informants <strong>open-ended questions</strong> and <strong>probing wherever necessary</strong> to obtain data.</td>
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<tr>
<td>Informal interviews</td>
<td>An interviewer <strong>speaks informally</strong> with individuals in the population/community of interest <strong>without use of a structured interview guide</strong>.</td>
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<tr>
<td>Unstructured interviews</td>
<td>The interviewer does not have a structured interview guide prepared; however they do have a <strong>sense of where they would like the conversation to progress</strong>. Unstructured interviews <strong>rely on natural, open-ended questions</strong> that evolve based on the interviewee’s response.</td>
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## Overview of Methods: Elicitation

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<td><strong>Registry</strong></td>
<td>An <em>organized system for the collection, storage, retrieval, analysis, and dissemination</em> of information on individual persons who have either a particular disease or receive a treatment.</td>
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<td><strong>Survey</strong></td>
<td>A series of typically “closed-ended” questions with a <em>limited set of answers</em> for each one. The use of closed-ended questions means that survey results are quantifiable.</td>
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<td><strong>Narrative analysis/inquiry</strong></td>
<td>Field texts, such as stories, autobiography, journals, field notes, letters, conversations, interviews, family stories, photos (and other artifacts), and <em>life experience</em>, used and <em>understand the way people create meaning in their lives</em> as narratives.</td>
</tr>
<tr>
<td><strong>Crowdsourcing</strong></td>
<td>Members of an <em>online/virtual community</em> are asked about their perceptions/beliefs, etc., within a group setting.</td>
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<td><strong>Cognitive interview</strong></td>
<td>Used to <em>evaluate sources of response error in survey questionnaires</em>. These can be conducted using a “think aloud” approach, where a participant considers how they will respond to a specific survey question, or through “verbal probing techniques” where an interviewer asks the participant specific questions to further understand comprehension, recall, etc.</td>
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## Overview of Methods: Conceptualization and Validation

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<td>Cognitive debrief</td>
<td>Actively testing a questionnaire or instrument on a small group of target population representatives to check understandability, interpretation, and cultural relevance of the language or translation.</td>
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<td>Cognitive interview</td>
<td>Used to evaluate sources of response error in survey questionnaires. These can be conducted using a “think aloud” approach, where a participant considers how they will respond to a specific survey question, or through “verbal probing techniques” where an interviewer asks the participant specific questions to further understand comprehension, recall, etc.</td>
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<td>Concept mapping</td>
<td>A conceptual diagram depicting relationships between “concepts” (ideas, images, words) through arrows.</td>
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<td>Delphi panel</td>
<td>Engage a large number of experts and/or stakeholders in a process of coming to agreement without necessitating their leaving their usual domain. This usually involves circulating documents or papers in rounds so that all comments and suggestions can be noted, and subsequent comments can be changed based on the replies of the other experts.</td>
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Overview of Methods: Prioritization

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<td>Analytical hierarchy process</td>
<td>Technique for complex decision-making (multi-criteria decision-making) that builds on answering a hierarchy of less complex “sub-problems” through pairwise comparisons to come to a decision.</td>
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<td>Best-worst scaling</td>
<td>Examines tradeoffs that individual patients are willing to make through a series of surveys containing a subset of attributes from a “master list” of possible, competing attributes. Participants indicate the best and worst attributes (or most/least important, most/least appealing, etc.).</td>
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<td>Conjoint analysis</td>
<td>Statistical method used to derive preferences, priorities, and relative importance for characteristics of different interventions. Discrete choice experimentation (see below) is an example of a method used to elicit preferences.</td>
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<td>Discrete choice experiment</td>
<td>Choices among sets of alternative profiles motivated by differences in the levels of the attributes that define the profiles. By controlling the attribute levels experimentally and asking respondents to make choices among sets of profiles in a series of choice questions, it allows researchers to understand choices to quantify the impact of changes in attribute levels on choice. The estimates of these impacts reflect the strength of preference for changes in attribute levels.</td>
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Patient-Centered Outcomes Research Institute (PCORI) Methodology Standards

• In 2013, PCORI created/endorsed a set of 47 Methodology Standards for best research practices in PCOR and CER.

• The report includes vignettes that illustrate different ways that good methodology makes a difference to patients and their care, including stories of patients’ experiences navigating choices and weighing options.

• 11 Topic Areas:
  – #1-5: Cross-Cutting Standards
    • e.g. formulating research questions, patient-centeredness, HTE, etc.
  – #6-11: Specific Study Designs and Methods
    • e.g. systematic reviews, data registries, adaptive and Bayesian trial designs

PCORI Cross-cutting Standard: Patient Centeredness

PC-1: Engage people representing the population of interest and other relevant stakeholders in ways that are appropriate and necessary in a given research context.

Example: A **Social Burden Measurement Tool** for Alpha-1 and other rare diseases

**Background:**
- Alpha-1 is a genetic disease that causes serious liver and lung disease in adults.

**Patient engagement strategy:**
- A research team formed partnerships with the Alpha-1 community
- An **advisory board** of community and patient partners meets monthly to provide insights and has been instrumental in recruitment
- Rethinking the questions: *Who gets tested in the family? Who should know the results? Should they get married? Should they have children?*

**Patient-centered solution:**
- These patient-driven questions were incorporated into the tool

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Example: How Best to Communicate with Patients

**Background:**

Hospital X is not meeting quality standards for 30-day readmissions. Might a tailored program with a visiting nurse (after discharge) and prescription drug regimen review reduce re-hospitalization versus standard discharge planning?

**Patient engagement strategy:** Patient focus groups of recently discharged patients and their family members help craft the intervention. Patients voiced, “I am most responsive to text messaging”.

**Patient-centered solution:** As a result, this form of communication was added to the intervention protocol. Patients could choose text or telephone communication with the nurse.
PCORI Example 1: A Randomized Pragmatic Trial Comparing the Complications and Safety of Blood Clot Prevention Medicines Used in Orthopedic Trauma Patients

• Not any studies to date that compare low molecular weight heparins (LMWHs) with aspirin in preventing blood clots in fracture patients.

• Study compares rates: death, blood clots in the lung, complications after surgery, patient satisfaction, out-of-pocket costs, and minor blood clots

• Patients and stakeholders took an active role in developing this research proposal. The research team comprises trauma survivors, blood clot survivors, caregivers, frontline clinicians, professional organizations, medical insurers, and experts in this field of research.

• In preparation, surveyed 232 trauma patients to determine outcomes of importance.

• Patients and caregiver team members have been crucial to designing this study so that it answers an important research question for patients and physicians while being respectful to the challenging circumstances faced by patients and their caregivers.
Which Methods, When & How?

Example 1: Comparing the Complications and Safety of Blood Clot Prevention Medicines Used in Orthopedic Trauma Patients

Research Phase

Planning it

Doing it

Delivering results

Engagement Methods

Partnership

Survey

Focus group

Delphi method

Interviewing

Crowd-sourcing

Advisory group

Town Hall meeting
PCORI Example 2: Improving Patient Decisions about Bariatric Surgery

- Four types of weight loss surgery; risks and benefits vary widely and are strongly affected by patient factors such as age, sex, race, and the amount of excess body weight. The treatment options also vary in other ways e.g., type of diet post surgery, that should be considered.

- Research develops and tests a decision support tool for morbid obesity patients considering surgery.

- Tool will be based on data regarding the risks (complications and death) and benefits (weight loss, patient satisfaction, and improvements in quality of life after surgery) from 35,000 patients enrolled in a statewide clinical registry that have previously had weight loss surgery.

- The decision tool will be on a website; patients will enter data about themselves and receive a customized report of expected risks and benefits of the different surgery types based on their personal characteristics.

- This tool will also provide patients with information about other attributes of the treatment options that should be considered based on data from interviews with bariatric patients and providers.

- We will test the effects of our decision tool on patient decisions and outcomes by comparing it with usual care at all of the weight loss surgery programs.
Which Methods, When & How?

Example 2: Improving Patient Decisions about Bariatric Surgery

Research Phase

Planning it
Doing it
Delivering results

Engagement Methods

Partnership
Survey
Focus group
Delphi method
Interviewing
Registry
Advisory group
Town Hall meeting
PCORI Example 3: Causal Analyses of Electronic Health Record Data for Assessing Comparative Effectiveness of Treatment Regimens

- Effective management of chronic conditions such as Type 2 diabetes requires periodic clinical monitoring and frequent re-evaluation of treatment decisions over the course of the patient’s illness.

- Existing causal inference methods are not well adapted to RWD that are highly variable in timing and content such as electronic health record (EHR) data.

- Study will advance and adapt existing causal inference methods so they can be adequately applied to RWD and better inform the impact of both real-world adherence and frequency of clinical monitoring in CER.

- Using both simulated and existing EHR data from a nonrandomized type 2 diabetes study, we will evaluate the applicability and practical performance of new causal inference tools in RW CER.
Which Methods, When & How?

Example 3: Causal Analyses of Electronic Health Record Data for Assessing the Comparative Effectiveness of Treatment Regimens
In Summary

• A variety of methods exist for continuous patient engagement in CER/PCOR
• There is no one method for any one step;
• These methods are not limited to the examples provided, and can be used throughout the 10-step framework