UNIVERSITY of MARYLAND
SCHOOL OF PHARMACY

Which Methods, When and How?

Eleanor Perfetto, PhD, MS
Professor, Pharmaceutical Health Services Research,
University of Maryland School of Pharmacy
Senior Vice President, Strategic Initiatives National Health Council

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

*Based on: Mullins CD, Adbulhalim AM, Lavallee DC. Continuous Patient Engagement in Comparative Effectiveness Research. JAMA 2012; 307(15): 1587-8.

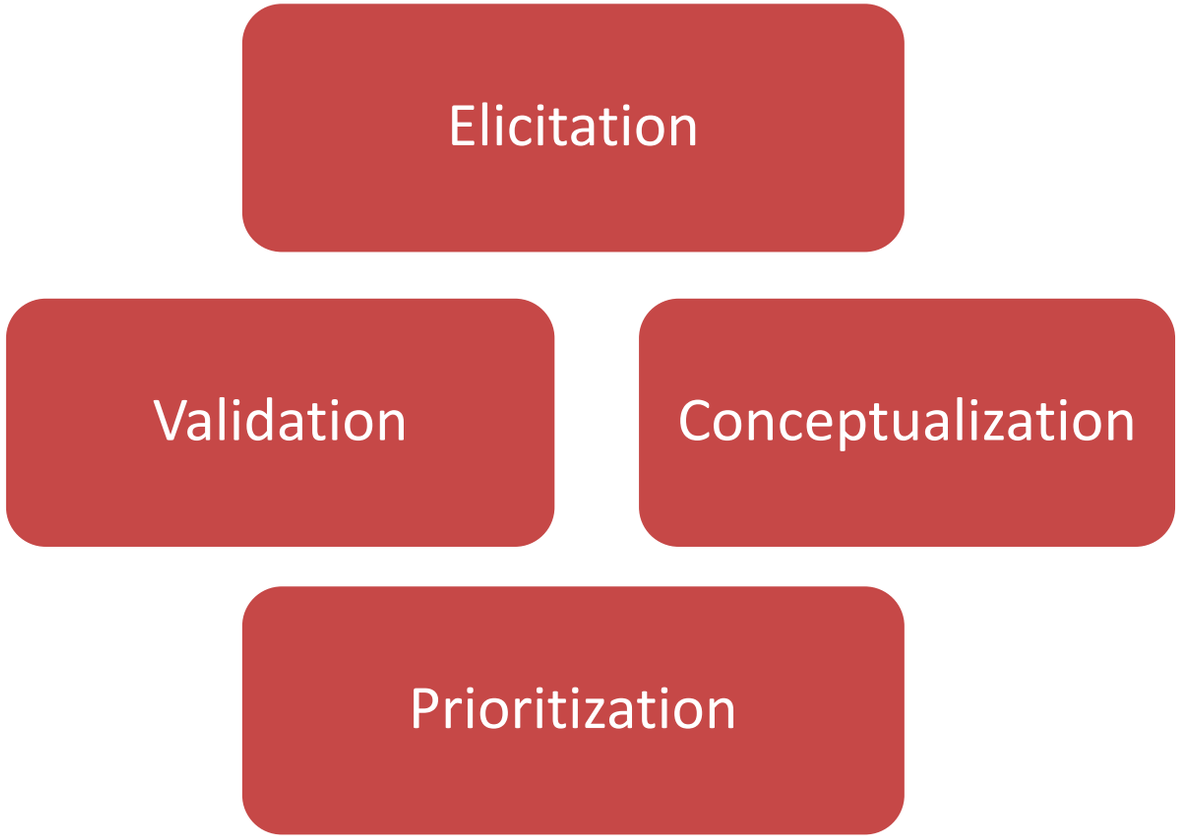
Revisiting the Roadmap: Examples of Engagement Methods

PCORI Engagement Rubric	10-Step Framework for Patient Engagement	Partnership	Survey	Focus Group	Delphi Panel	Interview	Crowd-sourcing	Advisory Group	Town Hall Meeting
Planning the Study	Step 1: Topic Solicitation	☐	☐	☐	☐	☐	☐	☐	☐
	Step 2: Prioritization	☐	☐	☐	☐	☐	☐	☐	☐
	Step 3: Framing the Question	☐	☐	☐	☐	☐	☐	☐	☐
	Step 4: Selection of Comparators and Outcomes	☐	☐	☐	☐	☐	☐	☐	☐
	Step 5: Creation of Conceptual Framework	☐	☐	☐	☐	☐	☐	☐	☐
Conducting the Study	Step 6: Analysis Plan	☐	☐	☐	☐	☐	☐	☐	☐
	Step 7: Data Collection	☐	☐	☐	☐	☐	☐	☐	☐
	Step 8: Reviewing & Interpreting Results	☐	☐	☐	☐	☐	☐	☐	☐
Disseminating the Study Results	Step 9: Translation	☐	☐	☐	☐	☐	☐	☐	☐
	Step 10: Dissemination	☐	☐	☐	☐	☐	☐	☐	☐

Revisiting the Roadmap: Examples of Engagement Methods

PCORI Engagement Rubric	10-Step Framework for Patient Engagement	Partnership	Survey	Focus Group	Delphi Panel	Interview	Crowd-sourcing	Advisory Group	Town Hall Meeting
Planning the Study	Step 1: Topic Solicitation	☑	☑	☑	☑	☑	☑	☑	☑
	Step 2: Prioritization	☑	☑	☑	☑	☑	☑	☑	☑
	Step 3: Framing the Question	☑	☑	☑	☑	☑	☑	☑	☑
	Step 4: Selection of Comparators and Outcomes	☑	☑	☑	☑	☑	☑	☑	☑
	Step 5: Creation of Conceptual Framework	☑	☑	☑	☑	☑	☑	☑	☑
Conducting the Study	Step 6: Analysis Plan	☑	☑	☑	☑	☑	☑	☑	☑
	Step 7: Data Collection	☑	☑	☑	☑	☑	☑	☑	☑
	Step 8: Reviewing & Interpreting Results	☑	☑	☑	☑	☑	☑	☑	☑
Disseminating the Study Results	Step 9: Translation	☑	☑	☑	☑	☑	☑	☑	☑
	Step 10: Dissemination	☑	☑	☑	☑	☑	☑	☑	☑

Patient Engagement in CER/PCOR



Overview of Methods: Elicitation

Focus group	Semi-structured group interview process moderated by a group leader – **used for breadth**, not necessarily depth.
Structured interview	Uses a **schedule of questions planned in advance** and which **do not deviate based on responses**.
Semi-structured interview	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 **open-ended questions** and **probing wherever necessary** to obtain data.
Informal interviews	An interviewer **speaks informally** with individuals in the population/community of interest **without use of a structured interview guide**.
Unstructured interviews	The interviewer does not have a structured interview guide prepared; however they do have a **sense of where they would like the conversation to progress**. Unstructured interviews **rely on natural, open-ended questions** that evolve based on the interviewee's response.

Overview of Methods: Elicitation

Registry	An **organized system for the collection, storage, retrieval, analysis, and dissemination** of information on individual persons who have either a particular disease or receive a treatment.
Survey	A series of typically **"closed-ended" questions** with a **limited set of answers** for each one. The use of closed-ended questions means that survey results are quantifiable.
Narrative analysis/inquiry	Field texts, such as stories, autobiography, journals, field notes, letters, conversations, interviews, family stories, photos (and other artifacts), and **life experience**, used and **understand the way people create meaning in their lives** as narratives.
Crowdsourcing	Members of an **online/virtual community** are asked about their perceptions/beliefs, etc., within a group setting.
Cognitive interview	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.

Overview of Methods: Conceptualization and Validation

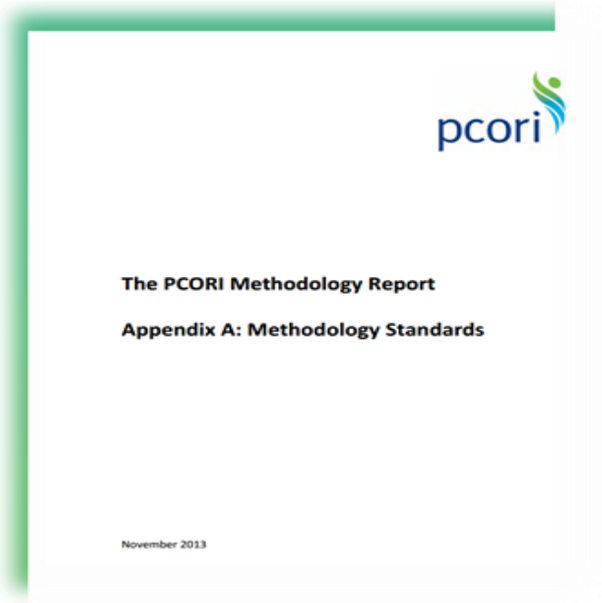
Cognitive debrief	Actively testing a questionnaire or instrument on a small group of target population representatives **to check understandability, interpretation, and cultural relevance of the language or translation**.
Cognitive interview	Used to **evaluate sources of response error in survey questionnaires**. 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.
Concept mapping	A conceptual diagram **depicting relationships** between "concepts" (ideas, images, words) **through arrows**.
Delphi panel	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.

Overview of Methods: Prioritization

Analytical hierarchy process	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.
Best-worst scaling	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.).
Conjoint analysis	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.
Discrete choice experiment	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**.

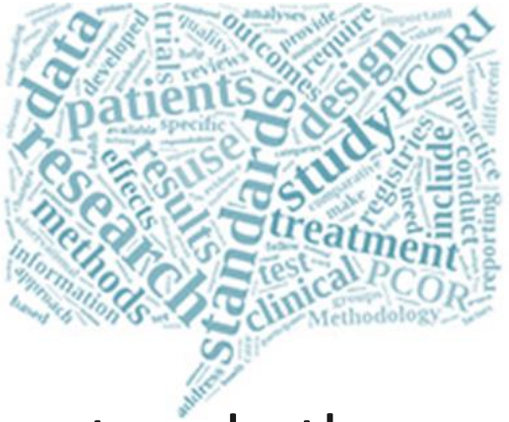
Patient-Centered Outcomes Research Institute (PCORI) Methodology Standards

- 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



Source: PCORI (Patient-Centered Outcomes Research Institute) Methodology Committee. (2013). The PCORI Methodology Report. Available at: http://www.pcori.org/research-results/research-methodology.

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

.

Source: PCORI (Patient-Centered Outcomes Research Institute) Methodology Committee. (2013). The PCORI Methodology Report. Available at: http://www.pcori.org/research-results/research-methodology.

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

Source: PCORI (Patient-Centered Outcomes Research Institute) Methodology Committee. (2013). The PCORI Methodology Report. Available at: http://www.pcori.org/research-results/research-methodology.

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".***

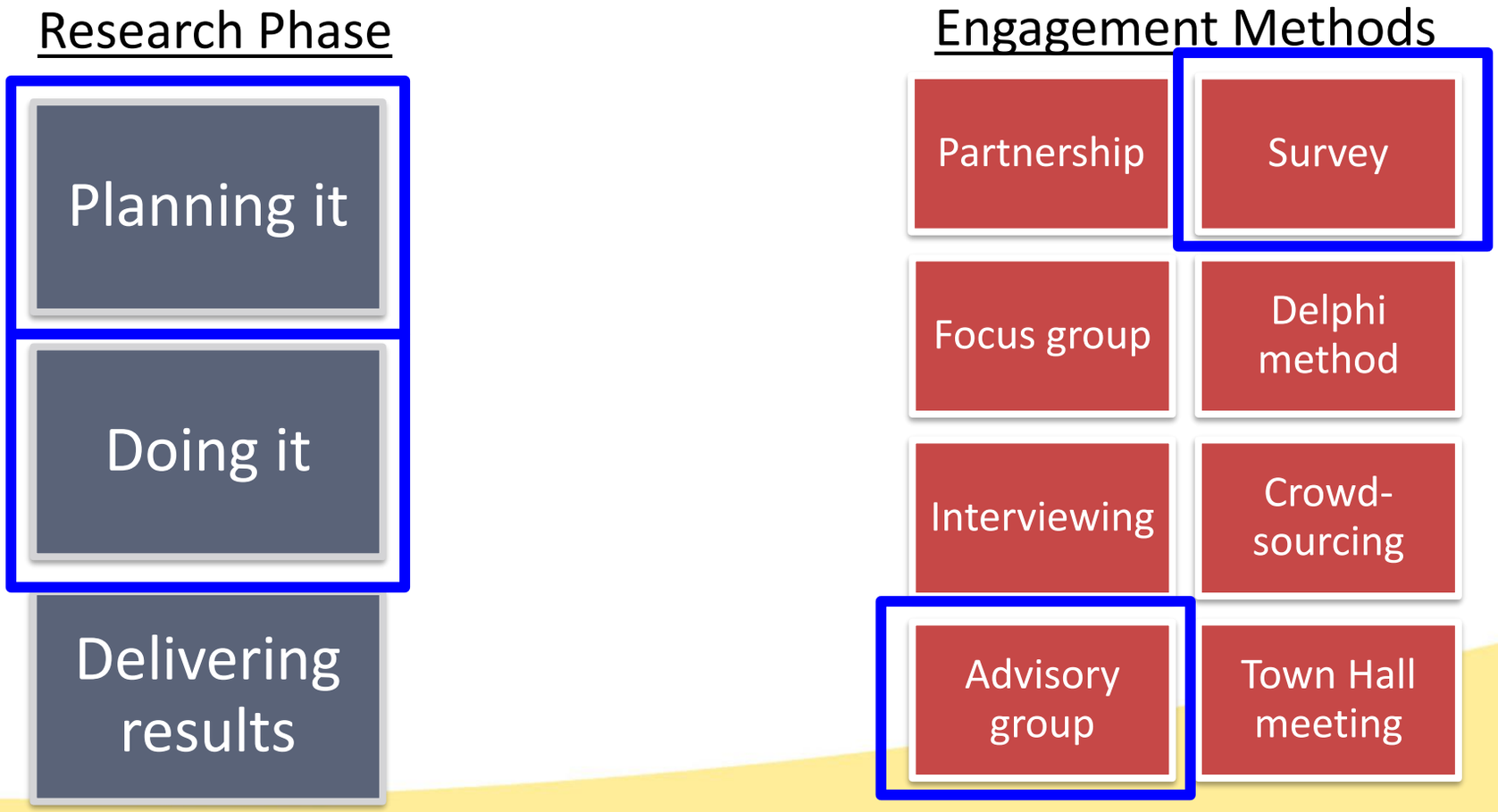
Patient-centered solution: As a result, this form of communication was added to the intervention protocol. Patients could choose text or telephone communication with the nurse.

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

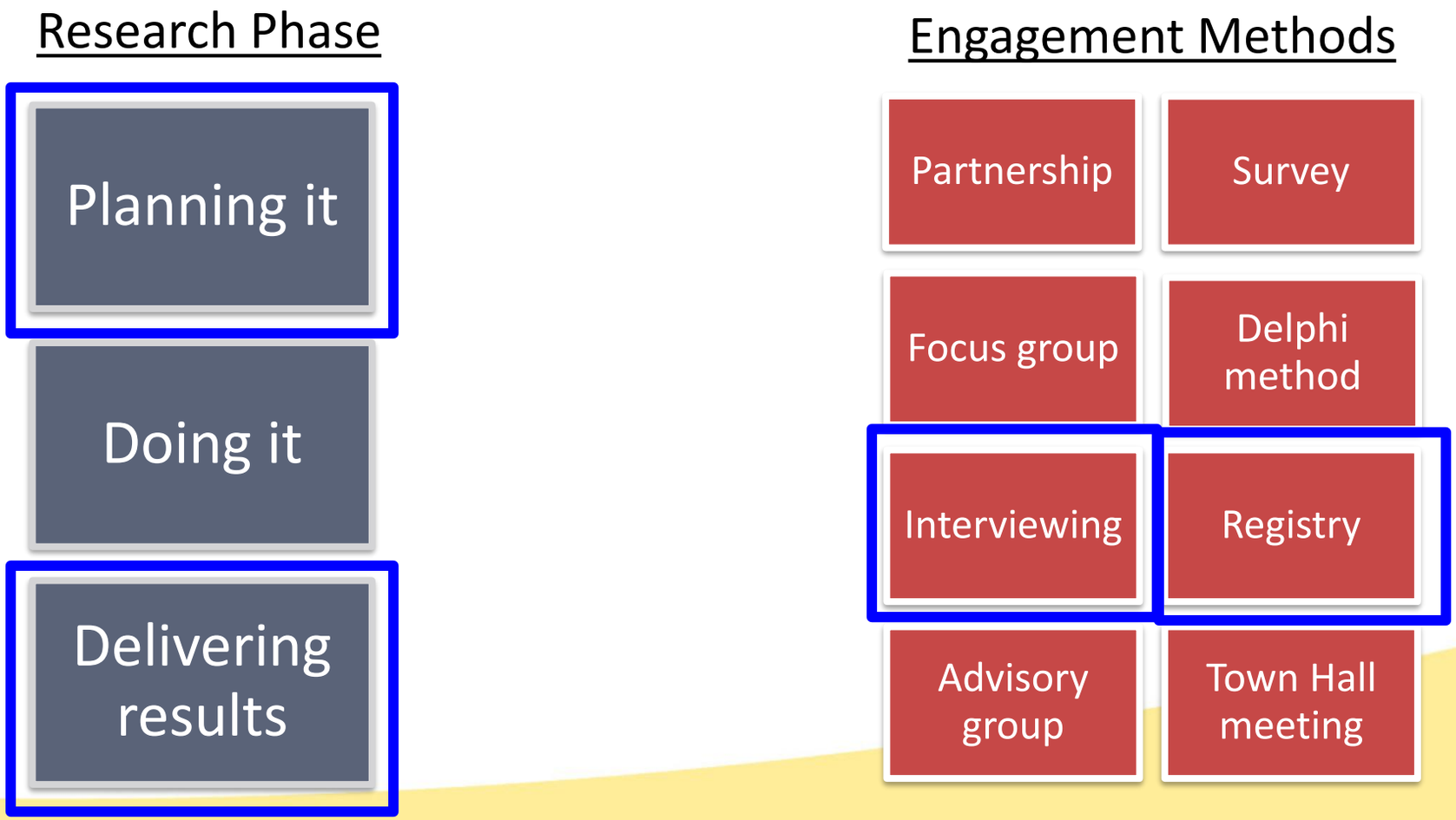


PCORI Example 2: Improving Patient Decisions about Bariatric Surgery

- 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



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

Research Phase

- Planning it
- Doing it
- Delivering results

Engagement Methods

- Partnership
- Survey
- Focus group
- Delphi method
- Interviewing
- Crowd-sourcing
- Advisory group
- Town Hall meeting

In Summary

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

Method Reference Guide

1. Saaty, Thomas L. Decision Making for Leaders: The Analytic Hierarchy Process for Decisions in a Complex World. Pittsburgh, Pennsylvania: RWS Publications. ISBN 0-9620317-8-X.2008
2. Dolan JG, Isselhardt BJ, Jr, Cappuccio JD. The analytic hierarchy process in medical decision making: a tutorial. Med Decis Making. 1989;9:40–50. doi: 10.1177/0272989X8900900108.
3. Flynn TN, Louviere JJ, Peters TJ, et al: Best-worst scaling: what it can do for health care research and how to do it. Journal of Health Economics 26:171–189, 2007
4. Patrick DL, Burke LB, Gwaltney CJ, et al. Content validity - Establishing and reporting the evidence in newly-developed patient-reported outcomes (PRO) Instruments for medical product evaluation: ISPOR PRO good research practices task force report: Part 2 – Assessing respondent understanding. Value Health 2011;14; 978-988.]
5. G. B. Willis, “Cognitive interviewing: A “how to” guide,” Research Triangle Institute, Tech. Rep., 1999, reducing Survey Error through Research on the Cognitive and Decision Processes in Surveys: A short course presented at the 1999 Meeting of the American Statistical Association.
6. Centers for Disease Control, National Center of Health Statistics. Cognitive Interviewing and Questionnaire Design: A Training Manual. http://www.srl.uic.edu/links/CMS_WP07_Willis_1994_CogIntTraining.pdf
7. Patrick DL, Burke LB, Gwaltney CJ, et al. Content validity - Establishing and reporting the evidence in newly-developed patient-reported outcomes (PRO) Instruments for medical product evaluation: ISPOR PRO good research practices task force report: Part 2 – Assessing respondent understanding. Value Health 2011;14; 978-988.
8. Burke JG, O'Campo P, Peak GL, Gielen AC, McDonnell KA, Trochim WM. An introduction to concept mapping as a participatory public health research method. Qual Health Res. 2005 Dec;15(10):1392-410.
9. Trochim WM, Kane M (2005). Concept mapping: an introduction to structured conceptualization in health care. International Journal for Quality in Health Care, 7(3),187-191.
10. Bridges, JFP, Hauber, AB, Marshall, D, et al. Conjoint Analysis Applications in Health—a Checklist: A Report of the ISPOR Good Research Practices for Conjoint Analysis Task Force. Value Health 2011;403-13.
11. Hauber AB, González JM, Groothuis-Oudshoorn CG, Prior T, Marshall DA, Cunningham C, IJzerman MJ, Bridges JF. Statistical Methods for the Analysis of Discrete Choice Experiments: A Report of the ISPOR Conjoint Analysis Good Research Practices Task Force. Value Health. 2016 Jun;19(4):300-15. doi: 10.1016/j.jval.2016.04.004.
12. Johnson FR, Lancsar E, Marshall, D, et al. Constructing experimental designs for discrete-choice experiments: Report of the ISPOR conjoint analysis experimental design good research practices task force. Value Health 2013;16:3-13.

Method Reference Guide cont'd

13. Johnson FR, Lancsar E, Marshall, D, et al. Constructing experimental designs for discrete-choice experiments: Report of the ISPOR conjoint analysis experimental design good research practices task force. Value Health 2013;16:3-13.
14. Morgan, D. L. (1997). Focus groups as qualitative research (2nd ed.). Thousand Oaks, CA: Sage.
15. Krueger, R.A. & Casey, M.A. (2015). Focus Groups: A Practical Guide for Applied Research (5th ed.) SAGE Publications
16. Berry, R. (1999). Collecting data by in-depth interviewing. Paper presented at the British Educational Research Association Annual Conference, University of Sussex at Brighton, September 25. [Online] Available: http://www.leeds.ac.uk/educol/documents/000001172.htm
17. RWJ Foundation. Informal Interviewing. Available from: http://www.qualres.org/HomeInfo-3631.html
18. RWJ Foundation. Unstructured Interviews. Available from: http://www.qualres.org/HomeUnst-3630.html
19. Chase, S. (2005). Narrative inquiry: Multiple lenses, approaches, voices. In Denzin, N.K. & Lincoln, Y.S. (Eds.), The SAGE handbook of qualitative research (3rd ed.), pp. 651-679. Thousand Oaks, London, & New Delhi: Sage Publications.
20. Clandinin, D.J. & Connelly F.M. (2000). Narrative inquiry: Experience and story in qualitative research. San Francisco: Jossey-Bass publishers.
21. Reid DJ, Reid FJM: Online focus groups: An in-depth comparison of computer-mediated and conventional focus group discussions. International Journal of Market Research. 2005, 47: 131-162.
22. Stewart K, Williams M: Researching online populations: the use of online focus groups for social research. Qualitative Research. 2005, 5: 395-416. 10.1177/1468794105056916.
23. Gliklich RE, Dreyer NA, Leavy MB, editors. Registries for Evaluating Patient Outcomes: A User's Guide [Internet]. 3rd edition. Rockville (MD): Agency for Healthcare Research and Quality(US); 2014 Apr. Available from: http://www.ncbi.nlm.nih.gov/books/NBK208616/
24. Cohen D, Crabtree B. "Qualitative Research Guidelines Project." July 2006. http://www.qualres.org/HomeStru-3628.html
25. Data Collection Methods To Answer Evaluation Questions. March 2016. Agency for Healthcare Research and Quality, Rockville, MD. http://www.ahrq.gov/professionals/quality-patient-safety/talkingquality/assess/collectionmethods.html