PCORI and PCOR

David R. Flum, MD MPH
Questions to Address

• PCOR vs CER?

• PCORI update
  ▪ Creation story
  ▪ What it's looking for in topics
  ▪ Stakeholder engagement
  ▪ Approach w methods report
  ▪ D&I and open science policy

• How do you do PCOR?
  ▪ Caprice’s example
Generates evidence to inform healthcare decisions

Compare different prevention, treatment, diagnostic strategies
CER

Generates evidence to inform healthcare decisions

Compare different prevention, treatment, diagnostic strategies

PCOR

Incorporates patient-reported outcome
CER

Generates evidence to inform healthcare decisions

Compare different prevention, treatment, diagnostic strategies

PCOR

Active patient engagement

Incorporates patient-reported outcome
CER

Generates evidence to inform healthcare decisions

Compare different prevention, treatment, diagnostic strategies

PCOR

Active patient engagement

Incorporates patient-reported outcome

Dissemination and Implementation for improved decision making

<table>
<thead>
<tr>
<th>Step in CER Process</th>
<th>Purpose of Patient Engagement</th>
</tr>
</thead>
<tbody>
<tr>
<td>Topic solicitation</td>
<td>- Identify topics that are important to patients, caregivers, and the community</td>
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<td></td>
<td>- Propose topics to be investigated</td>
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<tr>
<td>Prioritization</td>
<td>- Solicit feedback on relevance and priority of topics</td>
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<tr>
<td></td>
<td>- Discuss the urgency of addressing topics</td>
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<tr>
<td>Framing the question</td>
<td>- Ascertain questions’ relevance and usefulness</td>
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<tr>
<td></td>
<td>- Assess “real-world” applicability</td>
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<tr>
<td>Selection of comparators</td>
<td>- Identify comparator treatments of interest</td>
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<tr>
<td>and outcomes</td>
<td>- Identify outcomes of interest</td>
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<tr>
<td></td>
<td>- Incorporate other aspects of treatment</td>
</tr>
<tr>
<td>Creation of conceptual</td>
<td>- Provide a “reality check”</td>
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<tr>
<td>framework</td>
<td>- Verify logic of conceptual framework</td>
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<tr>
<td></td>
<td>- Supplement with additional factors not documented in the literature</td>
</tr>
<tr>
<td>Analysis plan</td>
<td>- Verify importance of factors and variables</td>
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<td></td>
<td>- Ascertain whether there is a good proxy for a specific concept</td>
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<tr>
<td></td>
<td>- Inquire about potential confounding factors</td>
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<tr>
<td>Data collection</td>
<td>- Determine best approaches for data collection (e.g., trial, registry, medical charts)</td>
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<tr>
<td></td>
<td>- Assist with selection of data sources</td>
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<tr>
<td>Reviewing and interpreting</td>
<td>- Assess believability of results</td>
</tr>
<tr>
<td>results</td>
<td>- Suggest alternative explanations or approaches</td>
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<td></td>
<td>- Provide input for sensitivity analysis</td>
</tr>
<tr>
<td>Translation</td>
<td>- Interpret results to be meaningful</td>
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<tr>
<td></td>
<td>- Document which results are easy or difficult to understand</td>
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<tr>
<td></td>
<td>- Indicate which results are counterintuitive</td>
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<tr>
<td>Dissemination</td>
<td>- Facilitate engagement of other patients</td>
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<td></td>
<td>- Help other patients to understand findings</td>
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“Use Patient-Reported Outcomes when patients or people at risk of a condition are the best source of information.”

~Draft report of Patient-Centered Outcomes Research Institute (PCORI) Methodology Committee
Advancing the Science of PCOR

• Build evidence on effective methods in patient engagement
• Build infrastructure for patient involvement
• Develop evaluation strategies to assess:
  ▪ Quality of patient engagement
  ▪ Impact of patient engagement on the quality of research
• Reporting standards for conducting PCOR
On March 23, 2010, the 111th Congress created PCORI as part of the Patient Protection and Affordable Care Act (Public Law 111–148)
PCORI Resources

FY 2010 - 2012
- $210 million from the Patient-Centered Outcomes Research Trust Fund

FY 2013
- $150 million from the Patient-Centered Outcomes Research Trust Fund
- $1 fee per Medicare and private health insurance beneficiary
- Estimated at $325 million

FY 2014 - 2019
- $150 million from the Patient-Centered Outcomes Research Trust Fund
- $2 fee per Medicare and private health insurance beneficiary
- Estimated at $500 million annually
PCORI helps people make informed health care decisions – and improves health care delivery and outcomes – by producing and promoting high integrity, evidence-based information – that comes from research guided by patients, caregivers and the broader health care community.

*PCORI is an independent, non-profit organization authorized by Congress committed to continuously seeking input from patients and a broad range of stakeholders to guide its work.*
## Assessment of Options for Prevention, Diagnosis, and Treatment

- Comparisons of alternative clinical options to support personalized decision-making and self-care
- Identifying patient differences in response to therapy
- Studies of patient preferences for various outcomes

## Improving Healthcare Systems

- Improving support of patient self-management
- Focusing on coordination of care for complex conditions and improving access to care
- Comparing alternative strategies for workforce deployment

## Communication & Dissemination Research

- Understanding and enhancing shared decision-making
- Alternative strategies for dissemination of evidence
- Exploring opportunities to improve patient health literacy

## Addressing Disparities

- Understanding differences in effectiveness across groups
- Understanding differences in preferences across groups
- Reducing disparities through use of findings from PCOR

## Accelerating PCOR and Methodological Research

- Improving study designs and analytic methods of PCOR
- Building and improving clinical data networks
- Methods for training researchers, patients to participate in PCOR
- Establishing methodology for the study of rare diseases
What Makes PCORI Funding Different?

• Special features include:
  – Patient & Stakeholder Engagement Plan
  – Dissemination and Implementation Assessment
  – Reproducible and Transparent Research Plan
  – PCORI Criteria Outlined by Statute
  – Encourage broader range of applicants
  – Complies with Methodology Standards

Chapter 1. Introduction
Chapter 2. How the Methodology Committee Developed the Recommended Standards
Chapter 3. Overview of the Standards
Chapter 4. Methodological Standards for Patient-Centeredness of Research Proposals and Protocols
Chapter 5. Methods for Prioritizing Patient-Centered Outcomes Research
Chapter 6. Choosing Data Sources, Research Design, and Analysis Plan: Translation Framework and Development of a Translation Table
Chapter 7. General and Cross-Cutting Research Methods
Chapter 8. Design-Specific Methods
Chapter 9. Next Steps
Opportunity for growing the research enterprise

Standard setting for PCORI is aimed at other funding and regulatory agencies

Adapt or miss out
  – Re-focus research questions
  – Re-train researchers
  – Re-tool research infrastructure

Politics and the future
PCORI & Surgery

Caprice C. Greenberg, MD, MPH, FACS
Associate Professor of Surgery
WARF Professor of Surgical Research
Director, Wisconsin Surgical Outcomes Research (WiSOR)
University of Wisconsin - Madison
PCORI and Surgery

• 403 PCORI projects funded to date
• Nearly 10% of funded PCORI awards as of 2014 have surgery focus (37)
• Discipline of PI
  – 199 non-MD (49%)
  – 204 MD
• Medical Specialty
  – 37 Surgeons (18%)
  – 167 Non-surgeon
Surgical RCT are lacking

- Surgical treatments are less likely than medical therapies to have RCT evidence
- Only 40% of treatment questions involving surgical procedures could have been evaluated by an RCT
  - Sham operations
  - Blinding and randomization
  - Learning curve

* Farrokhayr, Annals of Surgery (2010); 251(3): 409
** Kuehn, JAMA (2009); 302(14)1513
Surgery is well-suited for CER

• Hard Outcomes
  – In-hospital endpoints are meaningful
  – Easier to link to intervention

• Intervention is clearly identifiable
  – Administrative data
  – Date of service is distinct

• Comparison of 2 reasonable alternatives
  – Assessment of new technologies and procedures
Examples of Funded Studies
Adil Haider, MD, MPH
The Johns Hopkins University, Maryland

Patient-Centered Approaches to Collect Sexual Orientation/Gender Identity Information in the Emergency Department
Background

• Few hospitals routinely collect sexual orientation and gender identity (SO/GI) patient information, despite its impact on the quality and patient-centeredness of care.

• Researchers have documented extensive health disparities among LGBT people, including higher risk of suicide, substance abuse, and HIV.

• There are nearly 130 million annual ED visits in the U.S.
Objectives

• The specific aims of this proposal are:
  – To gather patient and provider input on barriers, facilitators, and preferred strategies for the collection of SO/GI information in the hospital ED
  – Develop two approaches for the patient-centered collection of SO/GI information
  – Evaluate the feasibility and effectiveness of these approaches.

• The long-term objective is to develop best practices for patient-centered approaches to routinely collect SO/GI information in the hospital ED.
Randomized Controlled Trial of a Patient Activation Tool in Pediatric Appendicitis
Background

• Patient activation tools (PAT) may improve patient-centered outcomes in emergency care.
  – Emergency surgical procedures present stressful and difficult decisions. This is especially true in children, for whom caregivers must make difficult decisions quickly about the care of their child.
  – Methods that can provide information about treatment choices and empower families to be involved in decision making may allow them to be more confident in their decision making (decision self-efficacy) and be happier with their care (satisfaction).
Objective

- To test if adding an interactive PAT to the routine surgical consultation process will improve outcomes related to decision making and satisfaction with care.
David Penson, MD, MPH
Vanderbilt University

Generating Critical Patient-Centered Information for Decision Making in Localized Prostate Cancer
Background

• Surgery, radiation, and observation have all been deemed appropriate therapies for localized PCa.

• AHRQ’s 2008 evidence report on the comparative effectiveness of therapies for localized PCa concluded that “no one therapy can be considered the preferred treatment for localized PCa.”

• The report called for high-quality, prospective cohort studies that identify men at the time of diagnosis and collect comprehensive patient, tumor, and treatment selection characteristics in an effort to generate critical patient-centered information to aid in decision making in localized disease.
Objective

• The proposed study will generate the critical information that patients need to make decisions about PCa treatment that reflect their personal preferences and values.
Improving Patient Decisions about Bariatric Surgery
Background

• 15 million Americans are more than 100 pounds overweight. While weight loss surgery is a very effective treatment for these patients, it also has risks.

• There are four different types of weight loss surgery and the risks and benefits of these procedures vary widely and are strongly affected by patient factors such as age, sex, race, and the amount of excess body weight.
Objectives

• The goals of this research proposal are to develop and test a decision support tool for morbid obesity patients considering weight loss surgery.

• Tool will be developed 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 that have had weight loss surgery.

• Team will test the effects of the decision tool on patient decisions and outcomes by comparing it with usual care.
Caprice Greenberg, MD MPH
University of Wisconsin-Madison

Post-treatment surveillance for breast cancer
PCORI Funded to Alliance

1. Post-Treatment Surveillance in Breast Cancer: Bringing CER to the Alliance (A231302; PCORI CE1304-6543)

2. Patient centered, risk stratified surveillance after curative resection of colorectal cancer (A231303; PCORI CE1304-6855)

3. Improving the effectiveness of routine surveillance following lung cancer resection (A231402; PCORI CE1306-00727)
Post-Treatment Surveillance

- Cuts across cancer sites
- Little CER in survivorship
- Lack of data = variations
- Guidelines on expert consensus
- Not tailored to individuals
- Prospective RCT is not feasible at this time
Consistent Priority Topic

“Compare the effectiveness of imaging technologies in diagnosing, staging and monitoring patients with cancer, including PET, MRI, CT.”

IOM Priority Topics for CER

“A 48 yo woman has recently completed radiation for a small growth in her breast. Her doctors see no signs of disease, but recommend that she continue to be monitored for potential recurrence. What is her optimal management strategy?”

PCORI PFA
• Assessment of prevention, diagnosis, treatment options
• Improving healthcare system
• Communication and dissemination
• Addressing disparities
Primary Objectives

• Provide evidence to inform the design of a risk-stratified patient-centered approach to surveillance following active treatment for breast cancer
  – Tailored guidelines
  – Personalized decision support tool

• Identify the highest value prospective trials to further refine surveillance approach
## Surveillance Guidelines

<table>
<thead>
<tr>
<th>Goal</th>
<th>NCCN</th>
<th>ASCO</th>
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</table>
| **Detection of Recurrence**         | • Interval history and physical exam every 4-6 months for 5 years then every 12 months  
• Annual mammography  
• Routine bone scans, CT scans, MRI scans, PET scans in the asymptomatic patient provide no advantage in survival or ability to palliate recurrent disease, and therefore, are not recommended. | • 1<sup>st</sup> post-treatment mammogram 1 year after initial mammogram that leads to diagnosis, but no earlier than 6 months after definitive radiation therapy.  
• Subsequent mammograms as indicated for surveillance.  
• Perform mammography yearly if mammographic findings are stable after local-regional therapy complete.  
• Not recommended for routine surveillance: breast MRI, FDG-PET scans, CBC, chemistry studies, chest x-rays, bone scans, liver ultrasound, tumor markers. |
| **Identification & Management of Treatment Toxicities** | • Annual gynecologic exam (if on tamoxifen)  
• Baseline bone mineral density & periodically (if on aromatase inhibitor or if ovarian failure secondary to treatment) | • Regular pelvic exam |
Recommendation/Guideline Gaps

• Distant imaging recommendations based on Cochrane Review and 4 prospective RCTs from 1980’s
  – 2005 Cochrane review of 3,055 women comparing intensive testing to annual physician visit and mammography
    • No difference in overall or disease-free survival
    • No difference in QOL
  – Studies did not include advanced imaging (CT scans, bone scans, PET scans, MRI)
• Recommendations mostly unchanged in last 15 years
  – Studies do not attempt to tailor recommendations based on risk (e.g., molecular subtypes)
What’s the Problem?

• Study limitations
  – Did not include modern advanced imaging
  – No subset analysis based on molecular subtypes and known risk
  – Does not account for major advances in ability to treat recurrence

• Recommendations and guidelines
  – Mostly unchanged for > 15 yrs
  – Primarily expert opinion
  – Call for additional evidence
Major Practice Variation

• High rates of healthcare utilization
  – 30 episodes per survivor in first year

• Overuse
  – 80% receive imaging for metastatic disease

• Underuse
  – Only 60-70% receive mammograms

• Reflect clinician evaluation of strength of scientific evidence?
Goals of Surveillance

• Detect second locoregional event
• Detect distant metastases
• Monitor for delayed treatment toxicity
• Manage patient anxiety and fear of recurrence
• Assure continued primary care and preventive health services
Aim 1

• Existing data from past Alliance trials
• Evaluate risk and patterns of recurrence and treatment toxicities
• According to tumor characteristics, treatment modalities, and other patient characteristics

• Outcomes – local-regional, distant, toxicity (3)
  – Event rate
  – Time to event
  – Overall survival
  – Breast cancer specific survival
## Alliance Power Calculation

<table>
<thead>
<tr>
<th>Analysis</th>
<th>N</th>
<th>5 year event rate</th>
<th># of expected events</th>
<th>95% CI Total Width</th>
</tr>
</thead>
<tbody>
<tr>
<td>Alliance Local Recurrence</td>
<td>22,881</td>
<td>7 – 13%</td>
<td>1281 – 2379</td>
<td>2.7 – 2.8%</td>
</tr>
<tr>
<td>Alliance Toxicity (Cardiac)</td>
<td>12,173</td>
<td>2 – 6%</td>
<td>335 – 1006</td>
<td>2.9 – 3%</td>
</tr>
</tbody>
</table>
Aim 2

• National Cancer Data Base special study

• Compare the effectiveness following active treatment for Stage II or III breast cancer of routine surveillance imaging v. current approach

  – Breast MRI v. mammogram alone

  – Advanced body imaging v. none
Aim 2

- 70% of newly diagnosed cancers across 1500 hospitals
  - Enroll 10 patients per institution
  - Restrict to Stage II and III
- Special Study for primary data collection
  - Intention of scan
  - Downstream events
  - Recurrence
  - Comorbidity
Aim 2 - Outcomes

• Breast MRI
  – Detection by imaging v. sign/symptom
  – Local regional with distant metastasis
  – Breast-cancer specific and overall survival

• Distant Imaging
  – Detection by imaging v. sign/symptom
  – Treatment of metastatic disease
  – Survival
## NCDB Power Calculation

<table>
<thead>
<tr>
<th>N</th>
<th>Surveillance Rate</th>
<th>Recurrence detection with usual care</th>
<th>OR with surveillance</th>
<th>Power</th>
</tr>
</thead>
<tbody>
<tr>
<td>14,000</td>
<td>30%</td>
<td>11%</td>
<td>1.1</td>
<td>0.38</td>
</tr>
<tr>
<td>14,000</td>
<td>30%</td>
<td>11%</td>
<td>1.2</td>
<td>0.90</td>
</tr>
<tr>
<td>14,000</td>
<td>30%</td>
<td>11%</td>
<td>1.3</td>
<td>0.99</td>
</tr>
</tbody>
</table>
Aim 3

• Engage cancer survivors, providers, and health outcomes researchers in interpreting the results of Aim 1 and 2 to

1) Develop a patient-centered, risk-based tailored approach to post-treatment surveillance

2) Identify the highest priority, feasible comparators for prospective randomized trials
Why Run Through the Alliance?

- Access to data
- Stakeholder engagement activities
- Pilot sites for data abstraction
- Capacity for dissemination and implementation
- Infrastructure for future prospective trials
Key Study Outcomes

• Risk-stratified surveillance strategy based on individual risk of recurrence and toxicities; Integration of information into decision aid
• Approach to collection of patient-reported outcomes including fear of recurrence and anxiety that can be included in future iterations
• Evidence-based approach to design of intervention studies
Conclusion

• Surgery is well-suited for PCOR and CER
  – Short-term outcomes
  – Well-defined, easy to identify interventions
  – RCTs have unique challenges
• Surgeons are competing successfully for PCORI grants
• Requires a broad skill set and multi-disciplinary team but important potential area
Thank you

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