The Patient-Centered Outcomes Research Institute

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PCORI’s Research Agenda is Driven by Stakeholders' Needs

“The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis...

The Institute shall identify national priorities for research, taking into account factors of disease incidence, prevalence, and burden in the United States (with emphasis on chronic conditions), gaps in evidence in terms of clinical outcomes, practice variations and health disparities in terms of delivery and outcomes of care, the potential for new evidence to improve patient health, well-being, and the quality of care...
Who Are Our Stakeholders?

Payers, Physicians, Caregivers/Family Members, Purchasers, Policy Makers, Patients/Consumers, Hospitals/Health Systems, Industry, Training Institutions, Patient/Caregiver Advocacy Organizations
How We Select Research Topics: Approach One

- Investigator-Initiated Approach
  - Aligned with our national priorities
  - Topic identified by research team in collaboration with stakeholders
  - PCORI’s first funding stream
    - 321 studies in progress; $554M awarded to-date
How We Select Research Topics: Approach Two

- **Patient- and Other Stakeholder- Initiated Approach**
  - Designed for targeted PCORI Funding Announcements (PFAs) and priority topics in Pragmatic Clinical Studies (PCS) PFAs
  - Allows us to focus dedicated resources on high-priority topics
  - Topics submitted to PCORI directly from patients and other stakeholders
    - Approximately 2,000 topics submitted to-date
      - 117 topics from 10 physician organizations
    - Pathway and topic status available on our website
  - 20 targeted studies on 8 topics; $183M awarded
    - 5 awards related to nominations from physician organizations
  - 14 PCS studies; $177M awarded
    - 2 awards related to topics nominated by physician organizations
Example of Topic Refinement: Obesity and Hypertension

- **Topic nomination:** Effectiveness of various strategies (e.g., clinical interventions, selected social interventions [such as improving the built environment in communities and making healthy foods more available], combined clinical and social interventions) to prevent obesity, hypertension, diabetes, and heart disease in at-risk populations such as the urban poor and American Indians.

- **Source:** Society of General Internal Medicine (IOM 100)

- **Topic refinement:**
  - Topic 1: Obesity treatment options for racial/ethnic minorities and SES populations
  - Topic 2: Hypertension control strategies among high-risk populations

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**Patient-centeredness**

**Impact on health**

**Assessment of current options**

**Likelihood of implementation**

**Durability**

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE
**Topic 1:** Comparative effectiveness of obesity treatment options set in primary care, in adults for racial/ethnic minorities, populations with low socioeconomic status, and/or rural populations.

**Awarded Projects:**

- **Peter Katzmarzyk: The Louisiana Trial to Reduce Obesity in Primary Care**
  - $10 million over 5 years, awarded in 2014
  - Compares the effectiveness of a high-intensity, health literacy-appropriate, and culturally-tailored obesity treatment program delivered by health coaches in a primary care setting to the primary care obesity treatment reimbursed by CMS in a low-income, racial and ethnic minority population.

- **Christie Befort: Midwestern Collaborative for Treating Obesity in Rural Primary Care**
  - $10 million over 5 years, awarded in 2014
  - Compares the effectiveness of a high-intensity lifestyle obesity treatment intervention delivered in-person by patient-centered medical home (PCMH) staff and via telephone by obesity treatment specialists to the primary care obesity treatment reimbursed by CMS in rural primary care practices.
  - Physician Engagement: American Academy of Family Physicians
Topic 2: Compare alternative, evidence-based approaches to reduce inadequate control of hypertension among high-risk populations with an above average lifetime risk of cardiovascular disease, including racial/ethnic minority groups, patients with low socioeconomic status, and individuals residing in rural areas.

Awarded Projects:

- **Lisa Cooper: Comparative Effectiveness of Health System vs. Multi-level Interventions to Reduce Hypertension Disparities**
  - $12 million over 5 years, awarded in 2015
  - Compares the effectiveness of clinic-based standard of care to a collaborative, stepped approach which includes community health workers and subspecialists to improve hypertension control for racial/ethnic minorities and low SES patients.

- **Monika Safford: Collaboration to Improve Blood Pressure in the US Black Belt-Addressing the Triple Threat**
  - $9 million over 5 years, awarded in 2013
  - Compares the effectiveness of telephone-based peer coaching to clinic-based primary care to improve hypertension control in low-income and rural African-Americans.
Overview of the PCORI Portfolio
Most Common Clinical Conditions in PCORI’s Portfolio

- Mental/Behavioral Health: 48 projects
- Cancer: 41 projects
- Cardiovascular Diseases: 29 projects
- Respiratory Diseases: 24 projects
- Nutritional and Metabolic Disorders: 23 projects
- Multiple/co-morbid Chronic Conditions: 22 projects
- Functional Limitations and Disabilities: 20 projects
- Neurological Disorders: 19 projects

N = 252

Note: Coding was inclusive and reports on projects up to Fall 2014.
Most Common Clinical Conditions in PCORI’s Portfolio
By Number of Projects and Investment

Note: Coding was inclusive and reports on projects up to Fall 2014.
Most Common Intervention Settings in PCORI’s Portfolio

Note: Coding was inclusive and reports on projects up to Fall 2014.
## Awards From Targeted Funding Announcements

<table>
<thead>
<tr>
<th>Targeted PFA (Year Awarded)</th>
<th>Number of Projects</th>
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<tbody>
<tr>
<td>Clinical Strategies for Managing and Reducing Long-Term Opioid Use for Chronic Pain (2016)</td>
<td>2</td>
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<tr>
<td>Treatment of Multiple Sclerosis (2016)</td>
<td>4</td>
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<td>Management Strategies for Treatment-Resistant Depression (2016)</td>
<td>3</td>
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<tr>
<td>New Oral Anticoagulants (NOACs) in the Extended Treatment of Venous Thromboembolic Disease (2016)</td>
<td>2</td>
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<tr>
<td>Clinical Management of Hepatitis C Infection (2015)</td>
<td>2</td>
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<tr>
<td>Testing Multi-Level Interventions to Improve Blood Pressure Control in High-Risk Populations (2015) <em>(Administered by the National Heart, Lung, and Blood Institute)</em></td>
<td>2</td>
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<tr>
<td>PCOR for Treatment Options in Uterine Fibroids (2014) <em>(Administered by the Agency for Healthcare Research and Quality)</em></td>
<td>1</td>
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<tr>
<td>Clinical Trial of a Mutifactorial Fall Injury Prevention Strategy in Older Persons (2014) <em>(Administered by the National Institute on Aging)</em></td>
<td>1</td>
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<tr>
<td>Obesity Treatment Options Set in Primary Care for Underserved Populations (2014)</td>
<td>2</td>
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<tr>
<td>The Effectiveness of Transitional Care (2014)</td>
<td>1</td>
</tr>
<tr>
<td>Asthma Treatment Options for African Americans and Hispanics/Latinos (2013)</td>
<td>8</td>
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Spotlight Studies
Pragmatic Trial of More vs. Less Intensive Strategies for Active Surveillance of Patients with Small Pulmonary Nodules

**Principal Investigator:** Michael Gould, MD, MS

- $14 million over 5 years, awarded in 2015

**Overview:**

- Compares two protocols for more intensive versus less intensive CT surveillance for patients with small pulmonary nodules on patient-reported outcomes, resource utilization, exposure to radiation, and adherence to both protocols.

**Methods**

- Cluster randomized controlled trial involving more than 46,000 patients

**Physician Engagement**

- American Chest Physicians, American Thoracic Society

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**Decisional dilemma**

Clinicians need conclusive information on the benefits and risks of CT surveillance to determine what protocol to use and whether protocols differ in effectiveness among specific groups of patients.
Enabling a Paradigm Shift: A Preference-Tolerant RCT of Personalized vs. Annual Screening for Breast Cancer

Principal Investigator: Laura Esserman, MD, MBA
- $14 million over 5 years, awarded in 2015

Overview
- Compares the effectiveness of personalized, risk-based breast cancer screening to standard annual screening on diagnosis, safety, morbidity, uptake of preventative therapies and psychosocial impact on women.

Methods
- Randomized controlled trial of 65,000, and an observational cohort (for those who decline randomization) of women of breast cancer screening age 40 to 80 years old

Decisional dilemma
Physicians need better information to understand the benefits (reduction in breast cancer mortality) and harms (false-positives, unnecessary biopsies) of annual breast cancer screening and how this differs by groups of women according to risk.
A Practical Intervention to Improve Patient-Centered Outcomes after Hip Fractures among Older Adults (Regain Trial)

Principal Investigator: Mark Neuman, MD
- $12 million over 5.25 years, awarded in 2015

Overview
- Compares the effectiveness of general anesthesia (unconsciousness produced by medications) to single-shot spinal anesthesia (a common nerve block providing temporary numbness of the lower extremities) on overall health and disability, as well as safety and hospital utilization, in an elderly patient populations with hip fractures.

Methods
- Pragmatic design of a head-to-head randomized controlled trial.

Physician Engagement
- American Association of Orthopedic Surgeons, American Society of Anesthesiologists, Canadian Orthopedic Trauma Society, Gerontological Society of America

Decisional dilemma
Physicians need more information on the benefits and harms of general vs. spinal anesthesia use during hip fracture surgery on patients’ ability to regain their prior level of independence after surgery.
Comparing Outcomes of Drugs and Appendectomy (CODA)

Principal Investigator: David Flum, MD, MPH
- $13 million over 5 years, awarded in 2015

Overview
- Compares the effectiveness of appendectomy to antibiotics-first for the treatment of uncomplicated appendicitis on both clinical outcomes and the patient-reported outcomes (PROs) that matter most to patients.

Methods
- Randomized controlled trial of 1,552, and a concurrent observational cohort of 500 (for those who decline randomization) of adults with uncomplicated appendicitis.

Physician Engagement
- American College of Surgeons, American College of Emergency Physicians

Decisional dilemma
Surgeons need conclusive information on the benefits and harms of treating appendicitis with antibiotics first, rather than resorting to an appendectomy.
Comparative Effectiveness of Intravenous vs. Oral Antibiotic Therapy for Serious Bacterial Infections

Principal Investigator: Ron Keren, MD, MPH

- $1.7 million over 2.5 years, awarded in 2012

Overview

- Compares the effectiveness of oral antibiotics vs intravenous antibiotics delivered via a PICC line in children who require prolonged (at least 1 week) home antibiotic therapy after hospitalization for a serious bacterial infection.

Decisional dilemma

Physicians need better evidence on the benefits and harms of delivering antibiotics orally vs. PICC line for children with serious bacterial infections.

Methods

- Mixed-methods approach

JAMA Pediatrics

CME/CE Activity

Osteomyelitis in Children

Comparative Effectiveness of Intravenous vs Oral Antibiotics for Postdischarge Treatment of Acute Osteomyelitis in Children

This program is accredited for:
- Physicians
- Physician Assistants
- Nurses
- Nurse Practitioners
- Pharmacists
- Case Managers
- Certified Health Education Specialists
Enhancing Genomic Laboratory Reports to Enhance Communication and Empower Patients

Principal Investigator: Marc Williams, MD

- $1.4 million over 3 years, awarded in 2013

Overview

- Examines whether an enhanced genomic laboratory report which incorporates the patient perspective can help providers and patients answer patient-oriented questions on lab results.

Methods

- Mixed-methods approach

- VIDEO LINK: https://youtu.be/bZwSbF9m2rc

Decisional dilemma

Physicians need conclusive information on the best way to communicate complex results from genetic reports with patients.
Looking Forward
Increasing Value and Reducing Waste

- 2009 estimate: 85% of research funding is avoidably wasted

Stages in research production that lead to waste. Moher et al.

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**Increasing value and reducing waste in biomedical research: who's listening?** Moher D, et al. *Lancet* 2015; Online: Sept.28
### PCORI’s Approach Aligns

- 17 recommendations to reduce waste and increase value across 5 stages of research production

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<thead>
<tr>
<th>Stage</th>
<th>Recommendations</th>
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<tr>
<td>1. Questions relevant to end users</td>
<td>• Users engaged&lt;br&gt;• In-process research and processes online</td>
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<tr>
<td>2. Appropriate design, conduct and analysis</td>
<td>• Methodology Standards and training&lt;br&gt;• COI policies</td>
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<tr>
<td>3. Efficient research regulation and management</td>
<td>• Efficiency of recruitment and retention</td>
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<td>4. Accessible, full reports</td>
<td>• Peer review and public availability of full reports</td>
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<tr>
<td>5. Unbiased and useable reports</td>
<td>• Infrastructure to support good reporting and archiving</td>
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Areas for Scientific Focus

• Prioritized, targeted investments
  – Stakeholder perspectives
  – Portfolio analysis/Benchmarking
  – Reducing disparities

• Evidence synthesis portfolio
  – Differences in treatment response (IPD MA, predictive analytics in trials)
  – Confirmation of research results

• Supporting the clinical encounter
  – Effective communication of evidence (including reducing low-value care)
  – Systems support
  – Appropriately rigorous methodologies in clinical research.
Important Considerations for Disseminating Research Findings

The primary questions and challenges to be addressed when assessing evidence include:

• *Is the evidence ready for use and adoption now?*
  – Evidence Context

• *What stakeholder priorities, needs, and concerns does the evidence address?*
Dissemination Activities Start Well Before Findings Are Ready

Effective dissemination and implementation start at the point of research topic selection, as emphasized by stakeholders—long before research is conducted and evidence is ready to be shared. To understand the *needs of audiences* who will use evidence to make health and healthcare decisions, research must address *questions that are relevant* to those audiences. To that end, those individuals and organizations who may partner with PCORI to disseminate and implement evidence should be engaged as *partners from the beginning*. 
PCORI’s Obligation Under its Authorizing Legislation

Conduct Peer Review of Primary Research

• Assess scientific integrity

• Assess adherence to PCORI’s Methodology Standards
PCORI’s Obligation Under its Authorizing Legislation (cont.)

Release of Research Findings

• No later than 90 days after “conduct or receipt”

• Make available to clinicians, patients, and general public

• Make comprehensible and useful to patients and providers for healthcare decisions

• Include considerations specific to certain sub-populations, risk factors, and comorbidities

• Describe process and methods, including conflicts of interest

• Include limitations and further research needed
Implications for PCORI Dissemination Activities

Initial Release of Findings (Website Posting)
  • Lay-language Abstract
  • Clinician Abstract

Next: Initiation of Dissemination Activities
  • Journal articles, webinars, CME/CE
  • Opportunities for “intermediaries” such as physician groups to disseminate and implement findings
Specific Areas of Interest

The examples provided below are intended to be illustrative, not exhaustive or prescriptive. Areas of interest include, but are not limited to the following:

• Translation/adaptation of the content/delivery mechanism of effective CER results/products to improve their penetration and use at the policy, health systems, clinical practice, caregiver, and patient levels

• Development, demonstration, and evaluation of processes or products to incorporate PCORI research results into decision making settings for patients, clinicians, policy makers and other stakeholders

• Demonstration of the capacity and ability to take research results and products found effective through PCORI research studies “to scale” in diverse settings and populations

• De-implementing or reducing the use of strategies and procedures that are not evidence-based, have been prematurely widely adopted, or are harmful or wasteful, in place of evidence-based approaches
Continuing Need for Clinician Involvement for Effective Dissemination

- Determine why the evidence matters to patients, clinicians, others.
- Anticipate barriers to use in decision making
- Active Dissemination and implementation participation

*Opportunities:*
  - Clinician input on dissemination and implementation plans
  - Engagement Awards
Dissemination and Implementation Timeline

2013
Start development process

2014
Develop initial framework for public discussion

2015
Finalize framework
First primary research projects completed
Start process of peer review and release of research findings

<< Develop infrastructure for D&I >>

2016
Initial D&I Activities

2017
Target D&I to specific audiences in collaboration with AHRQ
Eugene Washington PCORI Engagement Awards Program

- Support projects that will build a community better able to participate in patient-centered comparative clinical effectiveness research, as well as serve as channels to disseminate study results

- Also support meetings/conferences to exchange information or explore issues or areas of knowledge as they relate to patient-centered comparative clinical effectiveness research

- A programmatic funding opportunity - not research awards - for projects up to two years in duration, with total costs up to $250,000
Types of Engagement Awards

**Knowledge Awards**
- Increase understanding of what patients and other stakeholders need in order to make informed healthcare decisions.
- Generate findings about how patients and other stakeholders want to receive CER findings, as well as how they can make use of findings to reach health and healthcare decisions.

**Training and Development Awards**
- Equip patients and other stakeholders, teams, and organizations with the skills necessary to meaningfully participate in CER as partners throughout the research process.
- Develop meaningful patient and other stakeholder relationships, as well as promote new partnerships.

**Dissemination Awards**
- Develop facilitators for dissemination and implementation of CER findings.
- Identify, build, and strengthen partnerships being used for disseminating CER findings.
- Develop and maintain networks for the purpose of using and sharing CER findings.
Conclusions and Thank You