PCORI’s Approaches 8 Years: Achievements and Future Outlook

Joe Selby, MD, MPH

Executive Director
May 29, 2018
PCORI’s Legislative Mandate – to Serve Decision-Makers

“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...

... and the dissemination of research findings with respect to the relative health outcomes, clinical effectiveness, and appropriateness of the medical treatments, services...”

Patient Protection and Affordable Care Act 2010
PCORI Has used 3 Major CER Funding Awards

Since 2012

**Broad**
- Investigator-initiated, *any topic* that could change practice
- CER, patient-centeredness and engagement required
- Up to $1.5 million, three years

Since 2013

**Targeted**
- Single stakeholder-driven topic, narrow questions
- CER, patient-centeredness, robust engagement expected
- Much larger, variable funding amounts, 3-5 years

Since 2015

**Pragmatic**
- Stakeholder- or investigator-recommended topics
- CER, patient-centeredness, robust engagement required
- Up to $10 million direct costs, 5 years

Financials:
- $773 Mil
- $535 Mil
- $415 Mil
About PCORI’s Research Portfolio

BY THE NUMBERS

Research Projects By Area

Most Studied Conditions*

- Mental/Behavioral Health: 101
- Cancer: 80
- Neurological Disorders: 69
- Cardiovascular Diseases: 62
- Multiple/Comorbid Chronic Conditions: 55

Most Studied Populations of Interest*

- Racial/Ethnic Minorities: 272
- Low Socioeconomic Status: 182
- Older Adults: 134
- Women: 122
- Individuals with Multiple Chronic Conditions: 101

*Number of projects (out of a total of 410). A project may study more than one condition or population of interest.

As of November 2017
**PCORI Research Focuses on High Cost, High-Impact Conditions**

<table>
<thead>
<tr>
<th>National Per Capita Expenditure</th>
<th>Condition</th>
<th># Studies</th>
<th>Investment (millions)</th>
</tr>
</thead>
<tbody>
<tr>
<td>$32,027</td>
<td>Stroke</td>
<td>10</td>
<td>$41</td>
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<tr>
<td></td>
<td>Heart Failure</td>
<td>14</td>
<td>$68</td>
</tr>
<tr>
<td></td>
<td>Hepatitis (Chronic Viral B &amp; C)</td>
<td>8</td>
<td>$59</td>
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<td></td>
<td>COPD</td>
<td>17</td>
<td>$89</td>
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<tr>
<td></td>
<td>Schizophrenia/Other Psychotic Disorders</td>
<td>11</td>
<td>$22</td>
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<tr>
<td></td>
<td>Chronic Kidney Disease</td>
<td>16</td>
<td>$67</td>
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<td></td>
<td>Asthma</td>
<td>16</td>
<td>$51</td>
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<td></td>
<td>Atrial Fibrillation</td>
<td>3</td>
<td>$5</td>
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<tr>
<td></td>
<td>Alzheimer's Disease/Dementia</td>
<td>12</td>
<td>$64</td>
</tr>
<tr>
<td></td>
<td>Depression</td>
<td>35</td>
<td>$122</td>
</tr>
<tr>
<td></td>
<td>Cancer</td>
<td>77</td>
<td>$285</td>
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<tr>
<td></td>
<td>Ischemic Heart Disease</td>
<td>19</td>
<td>$94</td>
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<td>Osteoporosis</td>
<td>1</td>
<td>$14</td>
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<tr>
<td></td>
<td>HIV/AIDS</td>
<td>4</td>
<td>$8</td>
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<tr>
<td></td>
<td>Arthritis</td>
<td>13</td>
<td>$43</td>
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<tr>
<td></td>
<td>Diabetes</td>
<td>29</td>
<td>$68 M</td>
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**PCORI has 224 studies determining what works best in care for the 16 highest cost conditions in the U.S.**

High cost clinical conditions identified by The Centers for Medicare & Medicaid Services for 2015.
The PCORI CER portfolio includes 392 active/completed studies funded as of September 12, 2017.
Analysis excludes Methods, Pilots, PPRNs and CDRNs, and Engagement Awards.
PCORI-Funded Research Results Highlights

For Many with Type 2 Diabetes, Daily Finger Sticks Offer Little Health Benefit

Oral Antibiotics Work as Well as IV Post-hospitalization with Fewer Costly Complications

Simple Questionnaire Enhances Shared Decision Making about Chest Pain

Available at www.PCORI.org/about-us/fact-sheets
Case Study: Type 2 Diabetes

- Should noninsulin-treated patients with type 2 diabetes self-monitor their blood glucose levels?
  - A PCORI-funded study found no clinically or statistically significant differences at 1 year in glycemic control or health-related quality of life between noninsulin-treated patients with type 2 diabetes who performed self-monitoring and those who did not.

JAMA Internal Medicine | Original Investigation

Glucose Self-Monitoring in Non–Insulin-Treated Patients With Type 2 Diabetes in Primary Care Settings
A Randomized Trial

Laura A. Young, MD, PhD; John B. Buse, MD, PhD; Mark A. Weaver, PhD; Maihan B. Vu, DrPH, MPH; C. Madeline Mitchell, MURP; Tamara Blakeney, BS; Kimberlea Grimm, BAS; Jennifer Rees, RN, CPF; Franklin Niblock, BS; Katrina E. Donahue, MD, MPH; for the Monitor Trial Group
The Results: Impact on Utilization and Costs

- Over five years, discontinuing self-monitoring in this population:
  - Avoids using 10 billion test strips
  - Saves $2.3 billion per year in healthcare costs
  - Saves $11.6 billion over 5 years
Remaining Letter of Intent Due Dates

<table>
<thead>
<tr>
<th></th>
<th>2018</th>
<th>2019</th>
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<tr>
<td></td>
<td>Feb</td>
<td>Jul</td>
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<td>Broads</td>
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<tr>
<td>Targeted</td>
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<td>Awards</td>
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PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE
As of Q2-18, **52 CER Studies have their primary results peer-reviewed and publicly available**, and this number is steadily increasing.

- **Primary results** are results that report on a comparison of clinical approaches using the pre-specified primary outcome(s). Also commonly referred to as primary publications, or public disclosure of results.

- Primary results can be **made publicly available** by being published in a peer-reviewed journal, and/or by completing the PCORI Peer Review Process and having abstracts posted to PCORI.org.

**Of the 52 CER studies with primary results:**
- 36 (69%) were first made available via publications.
- 16 (31%) were first made available via PCORI.org.

**Update:**
As of 5/15/18, **74 CER studies** have publicly available primary results.
Moving to Dissemination and Implementation of Findings from PCORI-funded Studies
The D&I Program is charged with heightening awareness of the results of PCORI-funded research, and with advancing efforts to put these findings into use to improve healthcare delivery and health outcomes.
PCORI Dissemination & Implementation Activities

**Dissemination activities to bring results to audiences that will have a strong interest in using them**

- Convening and Input from PCORI Stakeholders & Building Capacity of Communities and Others

**Implementation activities to change practice**

**NEW:** Implementation of Evidence from Major PCORI Research Investments (Open)

Implementation Awards for PCORI Findings (PCORI Awardees)

Implementation of Shared Decision Making (Open)

AHRQ/PCORI Collaborative Projects

PCORI Evidence Updates
CME/CE
Engagement Awards:
- Dissemination Initiatives
- Building Capacity for Dissemination
- Conference Support
Mental and Social Well-Being among Women with Cancer in One Breast Who Underwent Surgery to Remove Both Breasts

Objective
To compare psychosocial outcomes and long-term survival estimates in women with nonhereditary unilateral ductal carcinoma in situ (DCIS) or breast cancer who underwent contralateral prophylactic mastectomy (CPM) versus women who underwent segmental or unilateral mastectomy.

Study Design
<table>
<thead>
<tr>
<th>Design Element</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Study Design</td>
<td>Observational; cohort study</td>
</tr>
<tr>
<td>Population</td>
<td>252 women ages 18 or older with newly diagnosed nonhereditary unilateral ductal carcinoma in situ or stage I, II, or III breast cancer</td>
</tr>
<tr>
<td>Interventions/Comparators</td>
<td>• Contralateral prophylactic mastectomy</td>
</tr>
<tr>
<td></td>
<td>• Segmental mastectomy or unilateral mastectomy</td>
</tr>
<tr>
<td>Outcomes</td>
<td>Primary: psychosocial outcomes (i.e., quality of life, body image concerns, cancer worry, cancer distress, satisfaction with treatment decision)</td>
</tr>
<tr>
<td></td>
<td>Secondary: estimated overall survival, estimated quality-adjusted life years</td>
</tr>
</tbody>
</table>

Peer-Review Summary
Evidence Updates

- Activities to disseminate results from PCORI-funded research on Current Treatments for Localized Prostate Cancer and Symptom-Related Quality of Life include Evidence Updates for Clinicians and Patients.
New Initiatives to Better Understand the Evidence Around New and Emerging Technologies

• PCORI will establish a Horizon Scanning Program
  – Horizon scanning identifies and monitors target technologies and therapeutics in healthcare to help stakeholders and PCORI identify and monitor important new therapeutics and technologies before they enter the market

• PCORI will commission topic brief summaries on new and emerging technologies intended both to meet information needs and to inform more definitive research
  – PCORI is currently developing its first brief on CRISPR and CAR-T (expected in 6 months)

• PCORI will develop evidence maps to better identify areas where more research is needed
  – This will revitalize topic and research question priority setting and refinement using tools from Horizon Scanning, emerging topic briefs, evidence mapping, and consultation with stakeholders
PCORnet: If You Build It They Will Come!
PCORnet® embodies a "network of networks" that harness the power of partnerships.
Resulting in a national evidence system with demonstrated research potential

PCORnet represents:

~128 million patients who have had a medical encounter in the past 5 years

*some individuals may have visited more than one Network Partner and would be counted more than once

**Pool of patients**

- For clinical trials: 42,545,341
- For observational studies: 83,131,450
PCORnet: **Common Data Model (CDM)**

- Based on FDA Sentinel Common Data Model
- Licensed under Creative Commons (open-access, use, and share)
- Allows for interoperability
- Designed to promote multi-site research and expansion to additional sites, systems and plans

### PCORnet Common Data Model Domains, v3.0 and v3.1

#### DEMOGRAPHIC v1.0
Demographics record the direct attributes of individual patients.

#### ENROLLMENT v1.0
Enrollment is a concept that defines a period of time during which a person is expected to have complete data capture. This concept is often insurance-based, but other methods of defining enrollment are possible.

#### ENCOUNTER v1.0
Encounters are interactions between patients and providers within the context of healthcare delivery.

#### DIAGNOSIS v1.0
Diagnosis codes indicate the results of diagnostic processes and medical coding within healthcare delivery. Data in this table are expected to be from healthcare-mediated processes and reimbursement drivers.

#### PROCEDURES v1.0
Procedure codes indicate the discreet medical interventions and diagnostic testing, such as surgical procedures and lab orders, delivered within a healthcare context.

#### VITAL v1.0
Vital signs (such as height, weight, and blood pressure) directly measure an individual’s current state of attributes.

#### LAB_RESULT_CM v2.0
Laboratory result Common Measures (CM) use specific types of quantitative and qualitative measurements from blood and other body specimens. The common measures are defined in the same way across all PCORnet networks, but this table can also include other types of lab results.

#### CONDITION v2.0
A condition represents a patient’s diagnosed and self-reported health conditions and diseases. The patient’s medical history and current state may both be represented.

#### PRO_CM v2.0
Patient-Reported Outcome (PRO) Common Measures (CM) are standardized measures that are defined in the same way across all PCORnet networks. Each measure is recorded at the individual item level; an individual question/statement, paired with its standardized response options.

#### DISPENSING v2.0
Outpatient pharmacy dispensing, such as prescriptions filled through a neighborhood pharmacy with a claim paid by an insurer. Outpatient dispensing may not be directly captured within healthcare systems.

#### PRESCRIBING v2.0
Provider orders for medication dispensing and/or administration. These orders may take place in any setting, including the inpatient or outpatient basis.

#### PCORNET_TRIAL v2.0
Patients who are enrolled in PCORnet clinical trials.

#### DEATH v3.0
Reported mortality information for patients.

#### DEATH_CAUSE v3.0
The individual causes associated with a reported death.

#### HARVEST v3.0
Attributes associated with the specific PCORnet datamart implementation, including data refreshes.
PCORnet: Distributed Research Network

The Requestor sends a question to the PCORnet Coordinating Center through the Front Door.

The Coordinating Center converts the question into a query with an underlying executable code, and sends it to PCORnet partners.

PCORnet partners review the query and provide a response, which is sent back through the Front Door to the Requestor.
PCORnet: Collaborative Research Groups

Collaborative Research Groups (CRGs):
Catalyzing multi-network research

- Autoimmune and systemic inflammatory syndromes
- Cancer
- Cardiovascular health
- Diabetes and obesity
- Health disparities
- Health Services Research
- Kidney health
- Pediatrics
- Pulmonary

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE
Many of the research studies underway in PCORnet are externally funded or co-funded.

As of Q2-18, there are 64 externally- or co-funded studies underway in PCORnet.

Research Studies Ongoing in PCORnet (Cumulative)
Patients with known coronary artery disease, ≥ 1 additional RF, identified through EHR followed by direct patient email recruitment and e-consenting

Pts. contacted electronically with trial information and eConsent; Treatment assignment will be provided directly to patient

ASA 81 mg QD (n=10,000)  
ASA 325 mg QD (n=10,000)

Electronic F/U Q 4 months; supplemented with EHR/CDM/claims data

Duration: Enrollment over 24 months; maximum f/u of 30 months

Primary Endpoint: Composite of all-cause mortality, nonfatal MI, nonfatal stroke

Primary Safety Endpoint: Major bleeding complications

N=15,000

*Enrichment factors
- age > 65 years
- creatinine > 1.5
- diabetes
- known 3-vessel coronary artery disease
- current cerebrovascular disease and/or peripheral artery disease,
- known ejection fraction <50%
- current smoker
$7M approved to utilize PCORnet infrastructure to support stakeholder-prioritized research

PCORnet: Rapid Cycle Research & Projects

Pre-Approval: Less than 3 months

Post-Approval: 1 month

Approved Projects: up to 9 months

~ 1 year from Topic to Results
### PCORnet Approved Rapid-Cycle Research Projects:

<table>
<thead>
<tr>
<th>Topic Area</th>
<th>Research Question</th>
<th>Project Completed</th>
</tr>
</thead>
<tbody>
<tr>
<td>PCSK9</td>
<td>Trends and current prevalence of use of PCSK9 Inhibitors in persons who are eligible for statin therapy</td>
<td>April 2018</td>
</tr>
<tr>
<td>Cancer</td>
<td>Patterns of use of molecular and genetic biomarkers/targeted cancer treatment for new onset solid tumors</td>
<td>June 2018</td>
</tr>
<tr>
<td>T2DM</td>
<td>Trends in use of newer agents in treatment of T2D and the potential for outcomes studies</td>
<td>Sept 2018</td>
</tr>
<tr>
<td>Hep C</td>
<td>Do pts with Hep C who are prescribed newer direct acting antiviral meds experience higher rates of adverse events than pts with Hep C who are untreated?</td>
<td>Nov 2018</td>
</tr>
<tr>
<td>Opioids</td>
<td>Potential of PCORnet to conduct surveillance of Opioid prescribing patterns and adverse events</td>
<td>April 2019</td>
</tr>
</tbody>
</table>
Introducing the People-Centered Research Foundation (PCRF)

Kathy Hudson, PhD, Chief Executive Officer
PCORI, PCORnet, and PCRF Relationships

• PCORnet is a large national clinical research network that is independent from but was developed with initial funding from the Patient-Centered Outcomes Research Institute (PCORI) with unique data, clinical trial, and engagement capabilities.

• PCORnet aims to deliver answers to questions that matter most to patients, clinicians, and health systems faster and more affordably.

• PCRF is a nonprofit charitable organization created by leaders of networks participating in PCORnet that ensures coordination and long-term sustainability.

• PCORI currently has awarded funding to networks participating in PCORnet and to PCRF.
PCORI, PCORnet, and PCRF Relationships

• PCORnet is going through an evolution to ensure that all parts of the network are committed and able to conduct highly efficient, networked, and patient-centered studies, supported by a range of public and private research funders.

• Two initial areas of major focus for PCORnet are research related to “heart and mind” conditions; however, PCORnet is intended to be a resource for conducting critical research in other areas and is receptive to proposals across the clinical spectrum.
PCORI 2.0 – Future Outlook
PCORI’s 8-Year GAO Report has been published!

PCORI Committed Funds Primarily to Research and Data Capacity Efforts; Awards for Dissemination and Implementation of Findings Were Limited as Most Research Was Still Underway

Officials from most stakeholder organizations we interviewed generally agreed that PCORnet offers value by improving the data available to conduct CER

PCORI research awards have increasingly focused on conditions that impose a substantial health or financial burden on patients and the healthcare system

Current Priorities for Reauthorization

- Increase awareness of PCORI to policymaking community
- Showcase the value and impact of research
- Continued engagement with key stakeholders
- Frame the role of PCORI for the next 10 years
- Build upon third-party support
Key Goals and Targets for Action

Identify Congressional Champions and Prioritize PCORI Reauthorization

- **GAO Report**
  - Jan – March/April – Oct 2018

Reintroduce Reauthorization Bill

- **Midterm Elections**
  - Nov 2018
- **PCORTF Sunset**
  - Jan – June 2019
  - Sept 2019
  - 2020

As we work to reauthorize PCORI’s funding for the future, it is important to know that PCORI is committed to fulfilling our mandate from Congress and will continue to exist and support the generation of patient-centered research beyond 2019.
What Comes Next?

• Congress will need to reauthorize the PCOR Trust Fund and the funding mechanisms that support it by September 30, 2019

• PCORI hopes to continue its mission as a unique public-private partnership dedicated to supporting and informing “end-users” of research – patients, caregivers, clinicians, systems, and payers

• PCORI has invested in and built a clinical research infrastructure to support future CER, health systems, and other clinical research with the goal of sustainability with or without PCORI funding after 2019
Thank you!

Questions?