Strategies to Prevent Unsafe Opioid Prescribing in Primary Care among Patients with Acute or Chronic Non-cancer Pain

LOI Applicant Town Hall Session

September 6, 2017
12:00 - 1:00 p.m. ET
Agenda

1. Welcome
2. Background on PCORI
3. Overview of the Funding Announcement
4. Engagement
5. Administrative Requirements
6. Questions

Submitting Questions:

Submit questions via the chat function in GoToWebinar

Ask a question via phone at the end of the presentation
Welcome to the Town Hall

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About PCORI
pcori.org
Our Mission

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.
Focus on Comparative Clinical Effectiveness Research (CER)

CER includes:

• Studies that compare health outcomes and the clinical effectiveness, risks, and benefits of two or more approaches to healthcare
  ▪ Clinical effectiveness research
  ▪ Improving healthcare delivery CER
  ▪ Communications and dissemination CER
  ▪ CER to reduce/eliminate health and healthcare disparities

• All applicants should:
  ▪ Explain how the research is comparative
  ▪ Name the comparators
  ▪ State why the comparisons are important to decision-makers
What is Patient Centered Outcomes Research?

• Examines comparative effectiveness questions: comparison of options for managing a specific clinical condition

• Features collaboration involving researchers, patients, and other stakeholder partners
  ▪ Research conducted in real world delivery settings
  ▪ Leveraging partnerships to ensure project success

• Can use various designs and approaches
  ▪ Randomized controlled trials
  ▪ Prospective registries
  ▪ Other observational designs
Overview of the Funding Announcement
Objective of this PFA:

- Prevent unsafe opioid prescribing while ensuring adequate pain management utilizing:
  - Payer or health system strategies
  - Patient and provider communication interventions addressing benefits and harms of treatments

Available Funds and Duration:

- A total of $20 million (direct and indirect) for this cycle
- Up to 4 projects not exceeding $5 million in total costs per project
- Projects should be completed within 3 years
Opioid abuse resulted in more than 20,000 deaths from prescription opioids in 2015 (Rudd et al, 2016)

Pain advocacy community has expressed concerns about the unintended harms to pain sufferers that may occur by restricting access to opioids.

Any policies in this area must strike a balance between our desire to minimize abuse of prescription drugs and the need to ensure access for their legitimate use.

What stakeholder groups have identified this as an important question?

- Payers, particularly the National Association of State Medicaid Directors
- Friends and family members who lost someone to prescription opioid abuse;
- Patients with chronic pain;
- Worker’s compensation organizations;
- State and federal policymakers
Abundance of Evidence Gaps

• Wide variation among states in opioid prescribing rates; indicating a lack of consensus about when to prescribe opioids (CDC, 2016)

• Little evidence exists on how to prevent unsafe prescribing of opioids; research focus largely on patients on chronic opioid therapy (Dy et al, 2016)

  ▪ No systematic reviews, RCTs, or controlled observational studies addressing the effects of opioid prescribing policies on clinical outcomes (Chou et al., 2009)

  ▪ A number of strategies targeted to providers and/or patients to promote safe opioid prescribing have been developed but not rigorously evaluated (HHS, 2014)

  ▪ Strategies that have proven successful in managing chronic pain and reducing the risk of opioid misuse for chronic pain have not been tested to promote safer initiation of opioids (Chang, et al. 2015)

• Guidelines recommend use only when alternatives are ineffective (CDC, 2016; Dy et al., 2016)
Rationale for Re-Issuance of this PFA

• Continuing concern about effective strategies to address the opioid crisis

• There are additional gaps in the evidence that warrant more robust comparative effectiveness research that are specific to each of the two questions.

• PCORI is particularly interested in receiving applications that target settings of high need, such as dentist’s offices, emergency departments, rural areas, and states with high rates of opioid prescribing, and those that compare specific strategies of interest to payers.
Funded Studies Under Prior Release of the PFA

<table>
<thead>
<tr>
<th>Project Title</th>
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<tr>
<td>A Naturalistic Experiment Evaluating the Impact of Medicaid Treatment Reimbursement Changes on Opioid Prescribing and Patient Outcomes among Patients with Low Back Pain</td>
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<tr>
<td>Provider-Targeted Behavioral Interventions to Prevent Unsafe Opioid Prescribing for Acute Non-Cancer Pain in Primary Care</td>
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Two Research Questions for Targeted PFA

**Question 1:** What is the comparative effectiveness of different payer or health system strategies that aim to prevent unsafe opioid prescribing while ensuring access to non-opioid methods for pain management with the goal of reducing pain and improving patient function and quality of life outcomes, while reducing patient harm?

**Question 2:** What is the comparative effectiveness of different patient- and provider-facing interventions that facilitate improved knowledge, communication and/or shared decision making about the harms and benefits of opioids and alternative treatments on prevention of unsafe prescribing and improved patient outcomes?
Research Question 1: Payer/Health System Strategies

- **Research Question**: What is the comparative effectiveness of different payer or health system strategies that aim to prevent unsafe opioid prescribing while ensuring access to non-opioid methods for pain management with the goal of reducing pain and improving patient function and quality of life outcomes, while reducing patient harm?

- **Population**: Potential new users of opioids or patients who have used opioids for < 3 months with either acute or chronic pain. Outside of end-of-life care. Does not include treatment for active cancer.
  - Patients with risk factors for dependence, abuse, and harm
  - Conditions where safer alternatives may be as or more effective
  - Conditions at risk of becoming chronic (e.g., nonstructural low back pain)

- **Interventions**: Must include interventions to prevent unsafe prescribing while ensuring adequate or improved pain management. Interventions must be evidence based or in widespread use.
Outcomes:

- **Primary:** Pain, quality of life, functional outcomes, reduction in unsafe prescribing
- **Examples of Secondary Outcomes:** Anxiety/depression, sleep, disability, harms (tolerance, dependence, addiction/opioid use disorder, overdose, death), provider satisfaction, provider self-efficacy, emergency department utilization

**Study Design:** Cluster RCT (encourage two active comparators plus usual care arm); or large, prospective observational study; encourage mixed methods

**Setting:** Primary care, broadly defined to include primary care practices, emergency departments, dentists offices, urgent care centers

**Time:** 3 years
Research Question 2: Improved Knowledge, Communication and/or Shared Decision Making

• **Research Question:** What is the comparative effectiveness of different patient- and provider-facing interventions that facilitate improved knowledge, communication and/or shared decision making about the harms and benefits of opioids and alternative treatments on prevention of unsafe prescribing and improved patient outcomes?

• **Population:** Potential new users of opioids or patients who have **used opioids for < 3 months** with either **acute or chronic pain**. Outside of end-of-life care. Does not include treatment for active cancer.
  - Patients with risk factors for dependence, abuse, and harm
  - Conditions where safer alternatives may be as or more effective
  - Conditions at risk of becoming chronic (e.g., nonstructural low back pain)
Research Question 2 (cont.)

- **Interventions:**
  - Must include interventions to prevent unsafe prescribing while ensuring adequate or improved pain management
  - Must be evidence based or in widespread use
  - May include combinations of patient and provider education, psychological management strategies, and/or self-management strategies
  - Encourage two active comparators but dependent on interventions selected

- **Outcomes:**

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<tr>
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<th>Primary Outcomes</th>
<th>Examples of Secondary Outcomes</th>
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<tr>
<td><strong>Patient</strong></td>
<td>- Knowledge</td>
<td>- Decisional regret&lt;br&gt;- Satisfaction&lt;br&gt;- Patient involvement preference&lt;br&gt;- Harms (tolerance, dependence, addition/opioid use disorder, overdose, death)</td>
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<td></td>
<td>- <strong>Patient anxiety (from potential health outcomes)</strong></td>
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<td>- Quality of life (including pain control)</td>
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<td>- Functional outcomes</td>
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<td><strong>Provider</strong></td>
<td>- Rate of opioid initiation</td>
<td>- Satisfaction&lt;br&gt;- Length of visit&lt;br&gt;- Confidence and <strong>self-efficacy</strong></td>
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<td></td>
<td>- <strong>Reduction in unsafe prescribing</strong></td>
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<td>- Repeat opioid prescriptions</td>
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<td>- Knowledge</td>
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Study Design: RCT or cluster RCT

Setting: Primary care, broadly defined to include primary care practices, emergency departments, dentist offices, urgent care centers

Time: 3 years
Tips for Success

• Clearly identify which of the two priority questions (or both) you are trying to address in the application
• Clearly describe comparators for the study
• Document evidence of efficacy/effectiveness for the intervention and comparator(s) and/or demonstrate that they are in widespread use
• Justify your power calculations based on prior evidence of anticipated effect sizes
• Clearly demonstrate the feasibility of the study
  ▪ Show that have the team to do this and you are the right team
  ▪ Define and support your recruitment and retention plan
  ▪ Document that sites are already committed to participating
  ▪ Include realistic timelines for site start-up, IRB approval, and recruitment
2017 PCORI Methodology Standards

In any study, methods are critical. PCORI’s Methodology Committee developed Methodology Standards to which patient-centered CER must adhere.

The 48 standards can be grouped into 2 broad categories and 12 topic areas.

**Cross-Cutting Standards**
- Formulating Research Questions
- Patient Centeredness
- Data Integrity & Rigorous Analyses
- Preventing/Handling Missing Data
- Heterogeneity of Treatment Effects

**Design-Specific Standards**
- Data Registries
- Data Networks
- Causal Inference Methods*
- Adaptive & Bayesian Trial Designs
- Studies of Medical Tests
- Systematic Reviews
- Research Designs Using Clusters

*The first standard for Causal Inference Methods (CI-1) is considered cross-cutting and applicable to all PCOR/CER studies.
Patient and Stakeholder Engagement
Patients and Other Stakeholders

PCORI Community:
- Patient/Consumer
- Caregiver/Family Member of Patient
- Hospital/Health System
- Training Institution
- Payer
- Industry
- Policy Maker
- Patient/Caregiver Advocacy Org
- clinician
- purchaser
- member of patient

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE
Patient and Stakeholder Engagement

- Evidence that patients, caregivers, clinicians, and other stakeholders have been and will be engaged in:
  - Formulating the research questions
  - Defining the characteristics of study participants, comparators and outcomes
  - Selecting the important outcomes to be assessed
  - Monitoring study conduct and progress
  - Designing plans for dissemination of study results
- Clear statement of the roles and the decision-making authority of all patient and stakeholder research partners
- An organizational structure, including a Study Advisory Committee or similar entity, which will bring together national patient and stakeholder groups to further the goals of the study
Patient-Centeredness vs. Patient Engagement

- **Patient-Centeredness**
  - Does the LOI mention outcomes (both benefits and harms) important to patients?
  - Are the interventions being proposed for comparison available to patients now?
- **Patient and Stakeholder engagement**
  - Does the LOI mention intent to build an interdisciplinary study team that includes appropriate patient and stakeholder representation in consultation with PCORI?
Evidence of Appropriate Engagement of Relevant Patients and Other Stakeholders

• Applicants are expected to consult with patients and other stakeholders on their decisional dilemma and evidence needs or to reference previously documented decisional dilemmas in preparation for the submission of LOIs.

• Identify the patients and stakeholders you consulted in determining that the proposed study addresses their evidentiary needs for decision-making and indicate your commitment to continuing to engage them actively in the conduct of the study.
The Engagement Rubric

The rubric is intended to provide guidance to applicants, merit reviewers, awardees, and engagement/program officers (for creating milestones and monitoring projects) regarding engagement in the conduct of research. It is divided into three segments:

1. Planning the Study
2. Conducting the Study
3. Disseminating the Study Results
Budgeting

- Financial compensation of partners
- Expenses of partners (transportation, childcare, caregiver)
- Budgeting for program staff dedicated to engagement tasks
- Costs of engagement meetings and events (travel, food, audio visual)
- Additional time and resource to incorporate partner feedback into various project process
Public Posting of Partner Names

• Many members of the patient and stakeholder community have requested that PCORI make the names of partnering individuals and organizations available to credit the contributions of the full research team adequately.

• You should provide PCORI only those names of patient or stakeholder partners for whom you have obtained appropriate permission to disclose their identity to PCORI and for PCORI to use their names in public communications.

• If partners wish to remain anonymous, you may use pseudonyms or categorical descriptors (e.g., caregiver to husband with COPD, breast cancer survivor of 20 years).

• If you are selected for funding, the individuals and organizations you provided (including those described by pseudonym or categorical descriptor) will be listed on the project description page along with the other information about your project (such as abstract and PI).
Engagement Resources

• PCORI’s “The Engagement Rubric”

• Sample Engagement Plans

• Compensation Framework

• Engagement Budgeting

• Engagement in Research website page
  http://www.pcori.org/funding-opportunities/what-we-mean-engagement

• PCORI’s Methodology Standards PC-1 to PC-4
Administrative Requirements
Timeline – Off Cycle 2 2017

- Full Announcement Released: September 1, 2017
- Online System Opens: September 1, 2017
- Letter of Intent (LOI) Deadline: October 2, 2017
- Application Deadline: January 10, 2018
- Merit Review: March 2018
- Awards Announced: August 2018
- Earliest Start Date: October 2018
Eligibility to Submit a Letter of Intent

- Any private sector (non-profit or for-profit) research organization.

- Any public sector research organization (university or college hospital or healthcare system, laboratory or manufacturer, unit of local, state, or federal government).

- Non-domestic components of organizations based in the US and foreign organizations may apply, as long as there is demonstrable benefit to the US healthcare system and US efforts in the area of patient-centered research can be clearly shown.

- Individuals are not permitted to apply.
Letter of Intent (LOI)

- An LOI is required and must be submitted prior to the deadline.

- To submit an LOI, download the Letter of Intent Template specifically for the Strategies to Prevent Unsafe Opioid Prescribing in Primary Care among Patients with Acute or Chronic Non-Cancer Pain – Cycle 2 2017 from the Funding Center to begin your LOI.

- You must answer all questions.

- Do not upload additional documents as part of your LOI. Letters of endorsements or support are not accepted at this stage.

- Only those LOIs deemed most responsive (programmatically and administratively) to this PFA will be invited to submit a full application.
Using the PCORI Online System

• Submit your LOI through PCORI Online:
  https://pcori.force.com/engagement

• Register as a New User and create your LOI as soon as possible

• Please note that the PI and AO cannot be the same person

• Enter information into all required fields in the system

• **PCORI Online Training Resources**
What PCORI looks for when reviewing LOIs?

• Relevance of the topics to one or both of the specific research questions in the funding announcement.
  ▪ Population focused on “potential” new users or opioid use for <3 months
  ▪ Setting is primary care
  ▪ Outcomes include pain and function as well as measures of unsafe prescribing

• Clarity and credibility of applicants’ responses to the LOI questions such as well-described comparators, clear research methods (e.g., study design, sample size, effect size)

• Whether costs are reasonable given proposed scope
Tips for applicants that are invited to submit a full application

• You were invited to submit a full application based on the information provided in the LOI – changes made after the LOI require PCORI approval
• Start and submit early
• Ensure that all team members can see the application in the PCORI Online system (check during the LOI stage)
• Inform your AO of your intent to submit
• Submit the completed application before the due date or by 5:00 PM ET on the due date
• View a recording of the [Cycle 3 2016 Applicant Town Hall](#)
Changes from LOI to Application

• If invited to submit a full application, please contact PCORI (pfa@pcori.org) if you wish to make any significant changes to the proposed study including the following:
  ▪ PI
  ▪ Institution
  ▪ Study design
  ▪ Budget or period of performance
  ▪ Research question
  ▪ Aims
  ▪ Comparators
• PCORI must approve any changes before the full application is submitted
Preparing Your Application

• To begin, all applicants should:
  ▪ **Thoroughly** read the funding announcement and review the PCORI Application Guidelines Document
  ▪ Review the PCORI Research Plan Template
  ▪ Have a copy of your approved LOI readily accessible
  ▪ Carefully consider the feedback you received on your LOI
Applicant Resources: Where Can I Find Help?

Visit pcori.org/apply
- Application Guidelines
- FAQs
- PCORI Online User Manuals
- Sample Engagement Plans

Schedule a Call with a Program Officer
- Submit a request at pcori.org/content/research-inquiry
- E-mail sciencequestions@pcori.org

Contact our Helpdesk
- E-mail pfa@pcori.org
- E-mail help@pcori.org – PCORI Online help
Q&A

Ask a question via the chat function in GoToWebinar.

Ask a question via phone (raise your hand and we will unmute your line).

If we are unable to address your question during this time, e-mail the Helpdesk at pfa@pcori.org.
Adjournment