Symptom Management in Patients with Advanced Illnesses

PCORI Pre-LOI Applicant Town Hall Session

July 12, 2017
1:00 – 2:00 PM EST
Town Hall Overview

I. Welcome and Introductions
II. Background
III. PFA Overview
IV. LOI Process and Review
V. Applicant Resources
VI. Questions

Submitting Questions:
Submit questions via the chat function in GoToWebinar
Ask a question via phone at the end of the presentation
I. Welcome and Introductions

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Engagement Officer, Public and Patient Engagement

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Merit Review Officer, Merit Review

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Associate, Contracts Operations, Contracts Management and Administration
II. Background
PCORI’s 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.

PCORI is particularly interested in pragmatic research conducted in real-world settings that will facilitate widespread dissemination and implementation of findings.
Symptom Management PFA

- Patients, caregivers, researchers, and stakeholder organizations have highlighted three specific palliative care areas as needing further CER:
  - Community-based models of care delivery
  - Advanced care planning
  - Symptom management
- In 2016, PCORI released a PFA on community-based palliative care delivery for adult patients with advanced illness that focused on care models and advanced care planning. Those awards will be announced in August 2017.
Symptom Management Evidence Gaps

• Gaps in research on symptom management in advanced illness extend across disease types, age groups, and symptoms

• Evidence produced to date has often been limited to results of trials with small sample sizes and few head-to-head comparisons of treatments for pharmacological and non-pharmacological approaches, and more often concentrates on advanced cancer and adult populations

• Studies frequently lack caregiver outcomes and adequate follow-up

• Much research is needed to address the gaps to understand how best to manage the symptoms associated with advanced illness treatments, so this is viewed as an important needed area for PCORI to support
III. PFA Overview
Symptom Management PFA Goal

Expand understanding about how various symptom management approaches convey benefits and harms on patients. Our focus is on real-world clinical dilemmas that patients, caregivers, and healthcare providers have identified as important to them.
Symptoms of Interest

- Symptoms of interest include and **are** limited to:
  - Pain
  - Fatigue
  - Dyspnea
  - Insomnia
  - Anorexia/cachexia
  - Nausea/vomiting
  - Depression and/or anxiety
Patients with an advanced illness are broadly defined as those who experience a high symptom burden with a life-limiting disease diagnosis.

- Individuals living with only a chronic disorder such as hypertension are not the focus of this funding announcement.

Conditions may include but are **NOT** limited to:

- Advanced heart failure
- Advanced cancer
- Chronic obstructive pulmonary disease (COPD)
- End-stage liver or kidney disease
- Neurodegenerative diseases
Design Considerations: Population

• Patients with advanced illnesses and their caregivers (as appropriate) of any age group with a life expectancy of greater than six months
  – PCORI is particularly interested in studies that will increase the evidence for treatments that are effective in pediatric and adolescent populations, where fewer studies exist
  – Studies on populations with advanced illnesses other than cancer, which have also been represented less in prior research, are also of interest
  – Applicants should propose sufficient sample size to influence the evidence base regarding symptom management in the proposed population
Design Considerations: Interventions

• Proposed interventions must include:
  – A pharmacological treatment
  – At least one meaningfully different pharmacological or non-pharmacological treatment approach

• Interventions proposed for study must comprise the basis of actual symptom management decisions encountered by patients, caregivers, and clinicians

• Interventions must have adequate evidence of efficacy and/or be in widespread use
Design Considerations: Outcomes

• Well-validated patient assessment measures (e.g., quality of life)
• Symptom measures
• Adverse events
• Caregiver measures (e.g., burden) as appropriate (e.g., in studies of pediatric or adolescent patients)
Design Considerations: Timing

• Follow-up of patient participants should be at least six months
• Life expectancy for participants should be projected to be a minimum of six months
• Applicants should power studies adequately with a sufficient sample size considering this requirement, and plan for a potentially more significant attrition rate due to advanced disease burden
Design Considerations: Setting

• Any healthcare setting or provider team may be appropriate under this PFA, if the parameters described above are met, including life expectancy.
Budget Parameters

- PCORI has allocated a total of up to $25 million for this PFA
- The proposed budget for studies under this initiative may be up to $2 million in direct costs
  - At the LOI stage, you must provide total direct costs and duration, and a brief summary of how the funds will be used and why the level of funding and duration requested are appropriate
  - PCORI may consider a larger budget if investigators submit a strong rationale at the LOI stage
- The maximum project period is three years
- Note that PCORI funding does not cover clinical healthcare costs
Application Requirements: Who Can Apply?

- Any private sector research organization
  - Non-Profit Organizations
  - For-Profit Organizations
- Any public sector research organization
  - Local, State, or Federal Government
  - Hospitals or Healthcare Systems
  - Universities or Colleges
  - Laboratories
- Foreign Organizations
- Nondomestic Components of Organizations based in the US

**NOTE:** PI must be an employee of the prime applicant institution. Individuals are not eligible to submit research applications to PCORI.
IV. LOI Process and Review
LOI Requirement

• The LOI is due online by **July 25, 2017, by 5:00 PM (EST)**
• Applicants are required to submit an LOI and only those deemed most responsive to this PFA will be invited to submit a full application
• Applicants will be notified by **August 22, 2017** whether or not they have been invited to submit a full application
• Applications will be due **October 25, 2017**
LOI Outline of Sections in the Template

- Specific Aims
- Background
- Significance
- Study Design
- Engagement Approach
- Study Population and Setting
- Interventions
- Outcomes
- Analytic Plan
- Sample Size and Power
- Prior Relevant Experience
- Duration and Total Direct Costs
Engagement Considerations

• Evidence of appropriate engagement of relevant stakeholders and researchers
• Funding 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
LOI Technicalities

• Download the **Letter of Intent Template specifically for the Cycle 2 2017 Symptom Management in Patients with Advanced Illnesses** from the [Funding Center](#) to begin your LOI

• LOIs are limited to 3 pages excluding references. LOIs that exceed the page limit will not be reviewed

• Please answer all questions, including the question on brief justification for the cost (e.g., “Will not exceed $2 million” is not a sufficient answer)

• Additional documents should not be included as part of your LOI
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 individual
• Enter information into all required fields in the system
• PCORI Online Training Resources
LOI Review

• LOIs will be reviewed administratively to ensure each meets the requirements stated, e.g., limits for pages, budget, duration, prior to being referred for programmatic review.

• Programmatic review will consider responsiveness to this specific targeted PFA, especially the clinical dilemma being addressed, along with the design considerations described in the PFA (population, outcomes, timing, etc.).
Non-responsiveness Examples – PLEASE AVOID:

- Tests efficacy (or comparative efficacy) within a tight, protocol-controlled research setting (as opposed to a more real-world and pragmatic CER).
- Conducts a cost-effectiveness analysis in the form of dollar-cost per quality-adjusted life-year to compare two or more alternatives.
- Directly compares the costs of care between two or more alternative approaches.
- Measures the relative costs of care of two or more alternative approaches as the primary criteria for choosing the preferred alternative.
- Conducts studies of the natural history of disease, instrument development, pharmacodynamics, and fundamental science of biological mechanisms.
- Evaluates new or existing decision-support tools. This includes developing and evaluating a decision-support or shared-decision tool or system for patients, clinicians, or both.
- Develops clinical prediction or prognostication tools.
## Key Dates

<table>
<thead>
<tr>
<th>Activity</th>
<th>Date</th>
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<tbody>
<tr>
<td>Online System Opens:</td>
<td>June 23, 2017</td>
</tr>
<tr>
<td>Pre-LOI Applicant Town Hall:</td>
<td>July 12, 2017 (Today)</td>
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<tr>
<td>LOI Deadline:</td>
<td>July 25, 2017</td>
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<tr>
<td>LOI Decisions Announced:</td>
<td>August 22, 2017</td>
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<tr>
<td>Application Deadline:</td>
<td>October 25, 2017</td>
</tr>
<tr>
<td>Post-LOI Applicant Town Hall:</td>
<td>September 13, 2017</td>
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<tr>
<td>Merit Review Dates:</td>
<td>January 2018</td>
</tr>
<tr>
<td>Awards Announced:</td>
<td>May 2018</td>
</tr>
<tr>
<td>Earliest Project Start Date:</td>
<td>July 2018</td>
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V. Applicant Resources
How is “advanced illness” defined for this PFA? Are chronic illnesses such as diabetes or hypertension potential patient populations of interest?

• Those who experience a high symptom burden with a life-limiting disease diagnosis. Individuals living with only a chronic disorder such as hypertension are not the focus of this funding announcement.

The funding announcement says that candidate interventions should be efficacious and/or widely used. What does that mean?

• Efficacious programs and interventions are those for which data demonstrate a positive impact on the outcome(s) of interest. The efficacy of interventions should be clearly described and cited in the research application. Widely used interventions are those that are being broadly circulated and implemented in clinical practice, often despite a clear evidence base.

For more FAQs and answers, see link on next slide
Applicant Resources


• **FAQs:** [http://www.pcori.org/funding-opportunities/what-you-need-know-apply/have-question/symptom-management-patients-advanced](http://www.pcori.org/funding-opportunities/what-you-need-know-apply/have-question/symptom-management-patients-advanced)

• **Methodology Standards:** [https://www.pcori.org/research-results/about-our-research/research-methodology/pcori-methodology-standards](https://www.pcori.org/research-results/about-our-research/research-methodology/pcori-methodology-standards)
Applicant Resources – Engagement


• Sample Engagement Plans: http://www.pcori.org/sites/default/files/PCORI-Sample-Engagement-Plans.pdf


• Engagement in Research Website: http://www.pcori.org/funding-opportunities/what-we-mean-engagement
Where can I find help?

Visit [pcori.org/apply](http://pcori.org/apply) – this is the Funding Center

- 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](http://pcori.org/content/research-inquiry)
- E-mail sciencequestions@pcori.org

Contact our Helpdesk

- E-mail pfa@pcori.org – administrative and technical inquiries
- E-mail help@pcori.org – PCORI Online help
VI. 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, email the Helpdesk at pfa@pcori.org.