Expanding PCORI's Large Pragmatic Studies Initiative

David Hickam, MD, MPH
Program Director

February 19, 2015

pcori

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome!

David Hickam, MD, MPH
Program Director

Key Questions for This Presentation

- What is the purpose of PCORI's Pragmatic Studies Initiative?
- How does the Pragmatic Studies PFA differ from other PFAs?
- How should you proceed with your project ideas?

What Types of Research Does PCORI Support?

From the Authorizing Legislation:

> "The terms 'comparative clinical effectiveness research' and 'research' mean research evaluating and comparing health outcomes and the clinical effectiveness, risks, and benefits of two or more medical treatments, services, and items…"

What Is a Pragmatic CER Study?

- Answers a practical, real-world comparative effectiveness research question that is important to patients and decision-makers
- Assesses whether two or more options differ in effectiveness when administered as they are in real life, and are conducted in a clinical setting that is as close as possible to a real-world setting
- The methodological approach (including study design, outcome measures, and follow-up) is as simple as possible without sacrificing scientific rigor

Objective of the Large Pragmatic Studies Program

Generates evidence to provide useful information concerning which approaches to care might work best, given particular concerns, biology, settings, and preferences of the individuals

- By necessity, these studies must be sufficiently large to allow rigorous comparisons of subgroups of interest
- Projects can address comparisons of discrete clinical services or complex interventions

Traditional Randomized Controlled Trials

- Study sample tends to be homogeneous, highly motivated (and therefore more adherent), relatively free of comorbid conditions
- Research tends to take place in specialized research settings
- Research protocols are often strict and do not represent typical clinical practice

Common Practice in a Large Pragmatic Study

- Loose eligibility criteria
- Flexibility in application of the intervention of interest
- Outcomes assessed in usual circumstances
- Little or no follow-up specifically for research purposes
- No special strategy for adherence

ALLHAT, Example of a Pragmatic Trial

- Antihypertensive trial
- Thiazide-type diuretic vs. calcium channel blocker vs. ACE inhibitor
- >33,000 participants; 55 y/o+; HTN; one other risk factor
- Diverse representation with adequate subgroups of interest (e.g., African Americans, patients with diabetes)
- Follow-up 4 to 8 years; study outcomes assessed at follow-up visits; hospitalized outcomes based on clinic investigator reports

Study Designs Can Vary

Conventional randomized controlled trial

- Randomization of individual patients
- Ensuring appropriate distribution of patients from subgroups
- Patient accrual be a major challenge

Cluster randomized trial

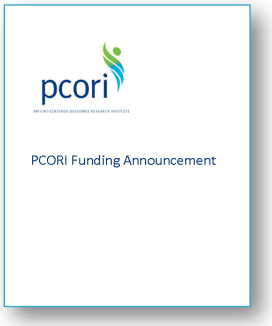
- Appropriate when provider behavior is a key part of the interventions being compared
- Sample sizes must be much larger

Large observational study

- Essential to control for confounding
- Must permit large sample sizes

Pragmatic Studies PFAs vs. Other PFAs

- Expect to have major impacts on patients, healthcare practices, and directions of future research
- Involvement of major stakeholder/patient organizations as research partners is mandatory
- Target specific priority topics
- More resources (10 million vs. $5 million or less)
- 5 years' duration vs. 3 years' duration or shorter



Engage Stakeholders/Patients To Help

- Formulate research questions
- Design the study to:
  - Integrate with routine clinic/office operations
  - Minimize disruption to participant's daily routine
- Refine recruitment strategies and proactively deal with recruitment issues
- Participate in data monitoring and safety activities
- Capitalize on existing resources (e.g., electronic health records, claims databases, networks) to
  - Collect study outcomes information
- Disseminate the study findings

Priority Topics for the Pragmatic Studies Program

- Management of ductal carcinoma *in situ*
- Treatments to prevent the transition from episodic to chronic migraine
- Smoking cessation therapies in high-risk persons
- Treatment strategies for osteoarthritis
- Treatments for multiple sclerosis
- Treatment strategies for autism spectrum disorder
- Treatment of opioid substance abuse
- Hemodialysis vs. peritoneal dialysis

Choosing the PCORI Program for a Submission

- Trade-off between importance of question and expected difference between interventions
  - Large expected difference: PCORI's broad programs
  - Small expected difference: requires large sample size, and the evidence gap must be important
- Carefully consider subgroups: has important impact on necessary sample size
- Length of follow-up may dictate a study with longer duration

Conclusions

- For many projects, the PCORI broad programs provide sufficient resources
  - Many more projects funded
  - Can fit project into focus areas of programs
  - Not based on priority clinical topics
- The pragmatic studies program provides resources to:
  - Substantially increase sample size
  - Adequately address subgroups
  - Lengthen duration of follow-up
- Pragmatic studies must have effective stakeholder partnerships

Questions?

Thank You!

David Hickam, MD, MPH
Program Director

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE