

# Patient and public driven research

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Patient Engagement Advisory Committee (PEAC) Meeting  
October 12, 2017



# Introduction

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Director, Public and  
Patient Engagement



# Our discussion for today

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- Overview of key issues in study recruitment, enrollment and retention
- Share what PCORI has learned about the importance of patient and stakeholder engagement in research
- Provide an overview of what PCORI has learned about how researchers improve study recruitment, enrollment and retention
- Explain how stakeholder engagement is making a difference in this area



# PCORI is...

- An independent research institute authorized by Congress in 2010 and governed by a 21-member Board of Governors representing the entire healthcare community
- Funds comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process
- Seeks answers to real-world questions about what works best for patients based on their circumstances and concerns



# We fund research that...

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## Meets these criteria:

### 1. Potential for the study to fill critical gaps in evidence

Addresses a clinical uncertainty or decisional dilemma experienced by patients and other stakeholders

### 2. Potential for the study findings to be adopted into clinical practice and improve delivery of care

Has the potential to lead to improvements in clinical practice and patient outcomes

### 3. Scientific merit (Research design, analysis, and outcomes)

Has a research design of sufficient technical merit to ensure that the study goals will be met

### 4. Investigator(s) and environment

The proposed project has a team with appropriate investigators (e.g. qualifications and experience), as well as an environment with sufficient capacity (e.g. resources, facilities, and equipment)

### 5. Patient-centeredness

Focuses on improving patient-centered outcomes and employs a patient-centered research design

### 6. Patient and stakeholder engagement

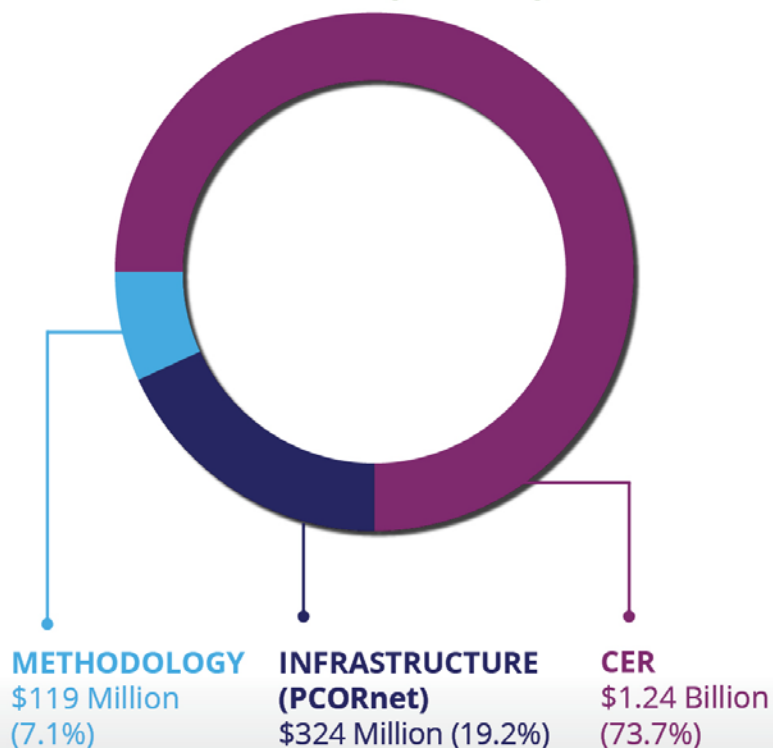
Includes patients and other stakeholders as partners throughout the entire research process



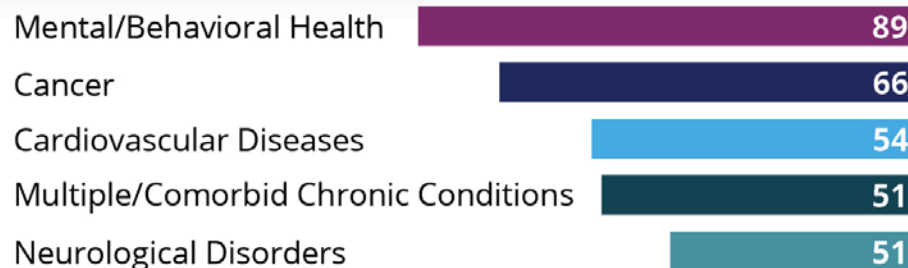
# Funding research that matters

## BY THE NUMBERS

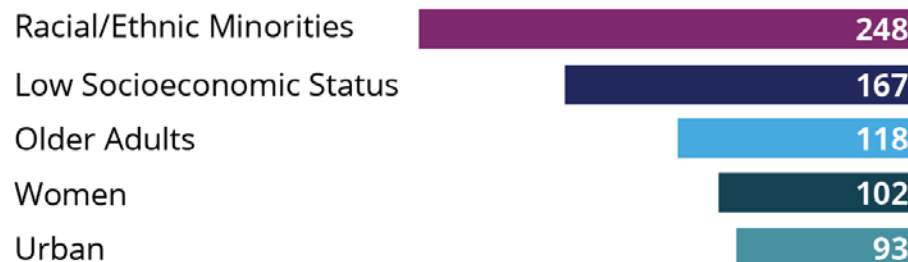
### Research Projects By Area



### Most Studied Conditions\*



### Most Studied Populations of Interest\*



\*Number of projects (out of a total of 365). A project may study more than one condition or population of interest.

As of March 2017

# What we mean by...

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## “Patient-centeredness”

- The project aims to answer questions or examine outcomes that matter to patients within the context of patient preferences
- Research questions and outcomes should reflect what is important to patients and caregivers



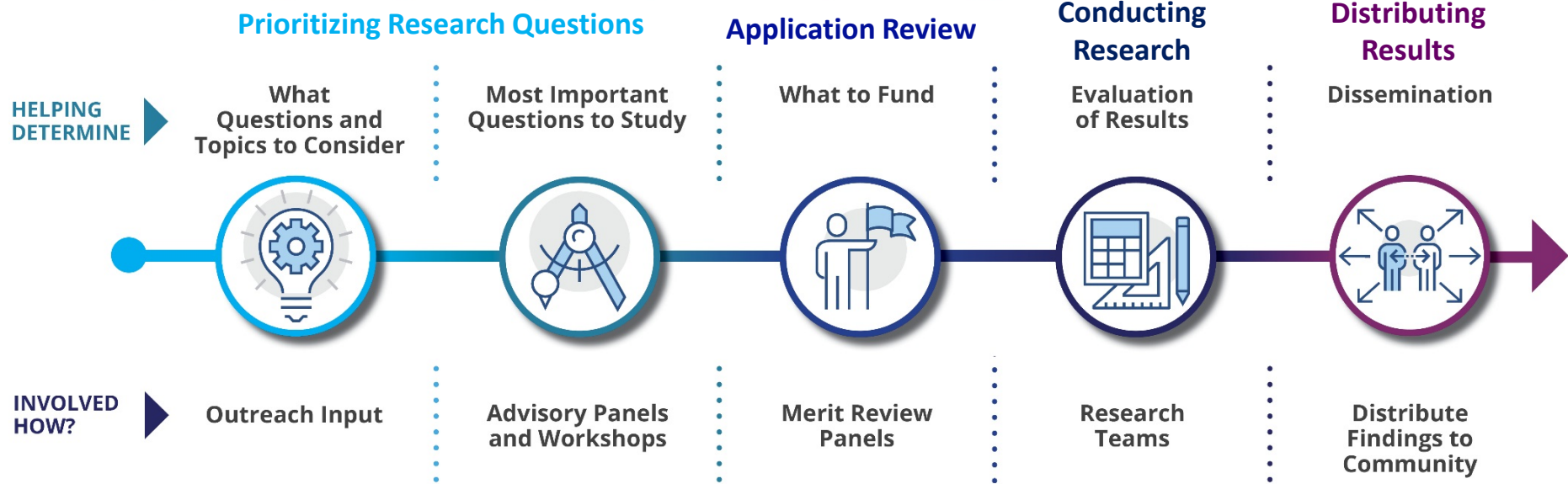
## “Patient and stakeholder engagement”

- Patients are partners in research, not just “subjects”
- Active and meaningful engagement between scientists, patients, and other stakeholders
- Community, patient, and caregiver involvement already in existence or a well-thought-out plan



# Stakeholder driven

## PATIENTS AND OTHER STAKEHOLDERS ARE INVOLVED IN...





# **A Look At The Field: What We Know About Awareness, Participation & Barriers to Recruitment**



# Research!America Surveys

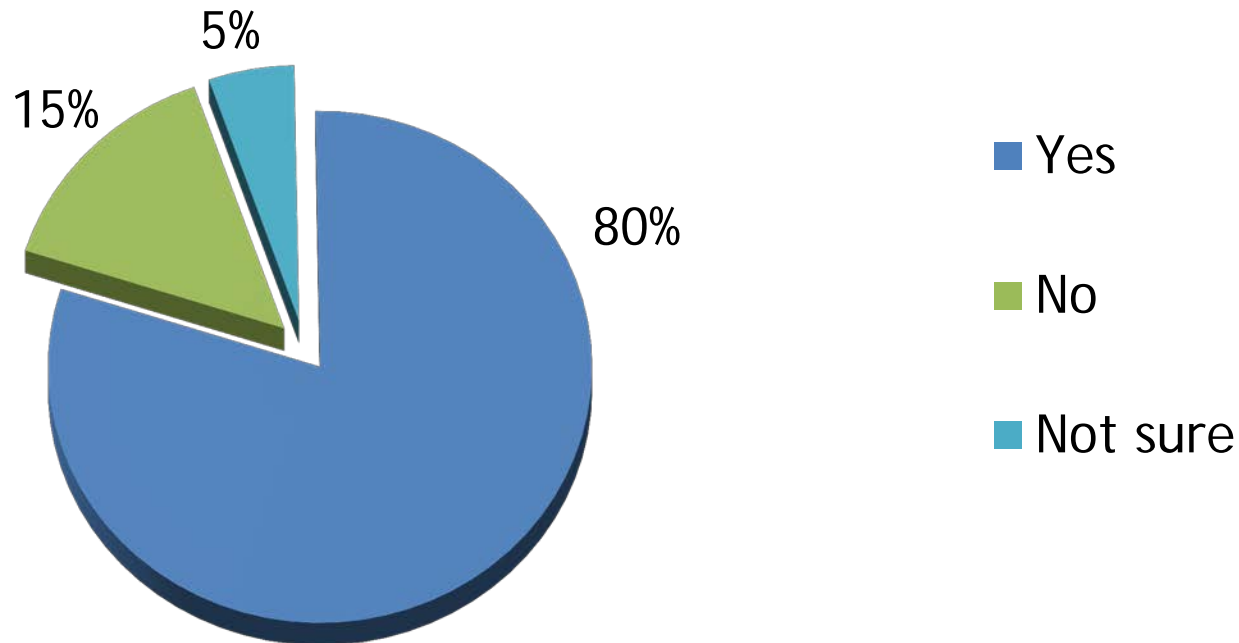
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- Commissioning public opinion surveys on research issues for 24 years:
  - National Surveys
  - State-Based Surveys
  - Issue-Specific Surveys
- Online surveys are conducted with a sample size of 1000-2000 adults and sampling error of +/-3.1%. The data are weighted in two stages to ensure accurate representation of the U.S. adult population.



# Wide Majority of Americans Have Heard of Clinical Trials

One kind of medical research is often referred to as a clinical trial. In this, volunteers choose to participate to test the safety and effectiveness of certain treatments, drugs or devices. Have you ever heard of a clinical trial?

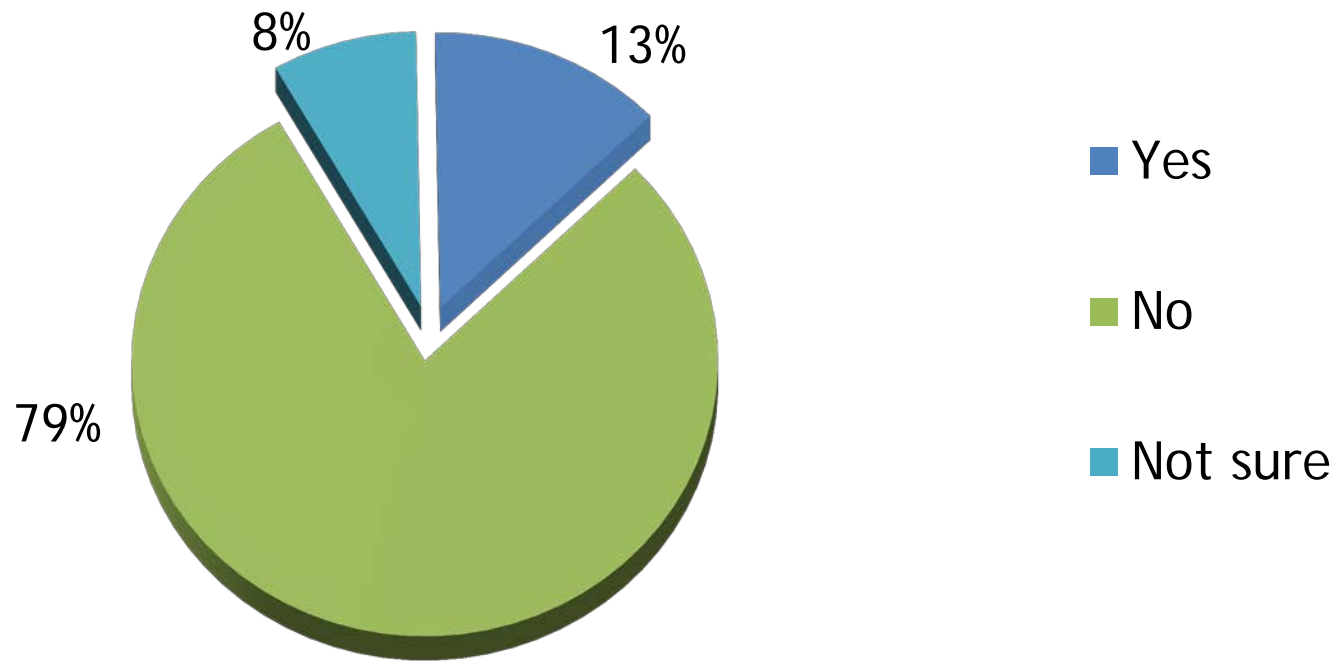


**Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.**



# Wide Majority of Americans Have Not Participated in Trials

Have you or someone in your family ever participated in a clinical trial?



**Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in January 2016.**



# Awareness, Trust, Risk are Barriers to Better Participation

Fewer than 10% of Americans participate in clinical trials. Which of the following do you think is a reason that individuals don't participate in clinical trials? (multiple responses allowed)

Not aware/lack of information	53%
Lack of trust	53%
Too risky	51%
Adverse health outcomes	44%
Little or no monetary compensation	35%
Privacy issues	27%
Too much time	27%
Not sure	11%

**Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.**



# Data from systematic reviews on recruitment efforts

*“While there are several possible consequences of poor recruitment, perhaps the most crucial is the potential for a trial to be underpowered.” (Treweek et al 2013)*

- Best practices may include:
  - data-driven approaches to understand demographics of patients and implications of the location of sites
  - effectively budgeting for recruitment and outreach/communication
  - planning risk mitigation strategies upfront
  - engaging patients/stakeholders early and throughout the process
- Not clear which is best, but promising strategies include:
  - telephone reminders
  - opt-out strategies
  - financial incentives

Source: Treweek S, Lockhart P, Pitkethly M et al. Methods to improve recruitment to randomised controlled trials: Cochrane systematic review and meta-analysis. *BMJ Open* 2013;3:e002360 [doi:10.1136/bmjopen-2012-002360](https://doi.org/10.1136/bmjopen-2012-002360)



# What are trends in the field?

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**Recommendations from the Clinical Trials Transformation Initiative (CTTI) on the recruitment planning continuum include the following activities:**

- Trial design and protocol development
  - Ensure relevance of scientific question
  - Develop *realistic* eligibility criteria
- Trial feasibility and site selection
  - Conduct data-driven feasibility analysis
  - Ensure appropriate site selection
  - Engage in suitable site performance monitoring
- Recruitment communication planning
  - Identify and engage all stakeholders and partners
  - Develop material and select appropriate channels for delivery
  - Monitor and evaluate process & performance

Source: <https://www.ctti-clinicaltrials.org/sites/www.ctti-clinicaltrials.org/files/Recruitment-MeetingSummary.pdf>



# What Have We Learned So Far At PCORI?



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# Recruitment Benchmarks

Through literature searches and working with other funders, we identified *points of reference for research projects*:



Three points of reference:

Around 10% of research projects are not successfully completed

Less than  
**5%**

47% of studies meet agreed upon enrollment timeline<sup>1,2,3</sup>

**About  
70%\***

\*PCORI contracts  
can be modified

Study timelines are typically extended to nearly double their original duration to meet desired enrollment levels<sup>3</sup>

Average  
Extension:  
**6 months**

1. Mary Jo Lamberti et al. **Evaluating the Impact of Patient Recruitment and Retention Practices.** *Clinical Trials*, 2012
2. Kenneth Getz. **Enrollment Performance- Weighing the "Facts."** *Applied Clinical Trials*, 2012
3. Tufts Center for the Study of Drug Development. **89% of Trials Meet Enrollment, but Timelines Slip, Half of Sites Under-enroll.** *Tufts CSFDD Impact Reports*. January/February 2013, Vol. 15 No. 1.

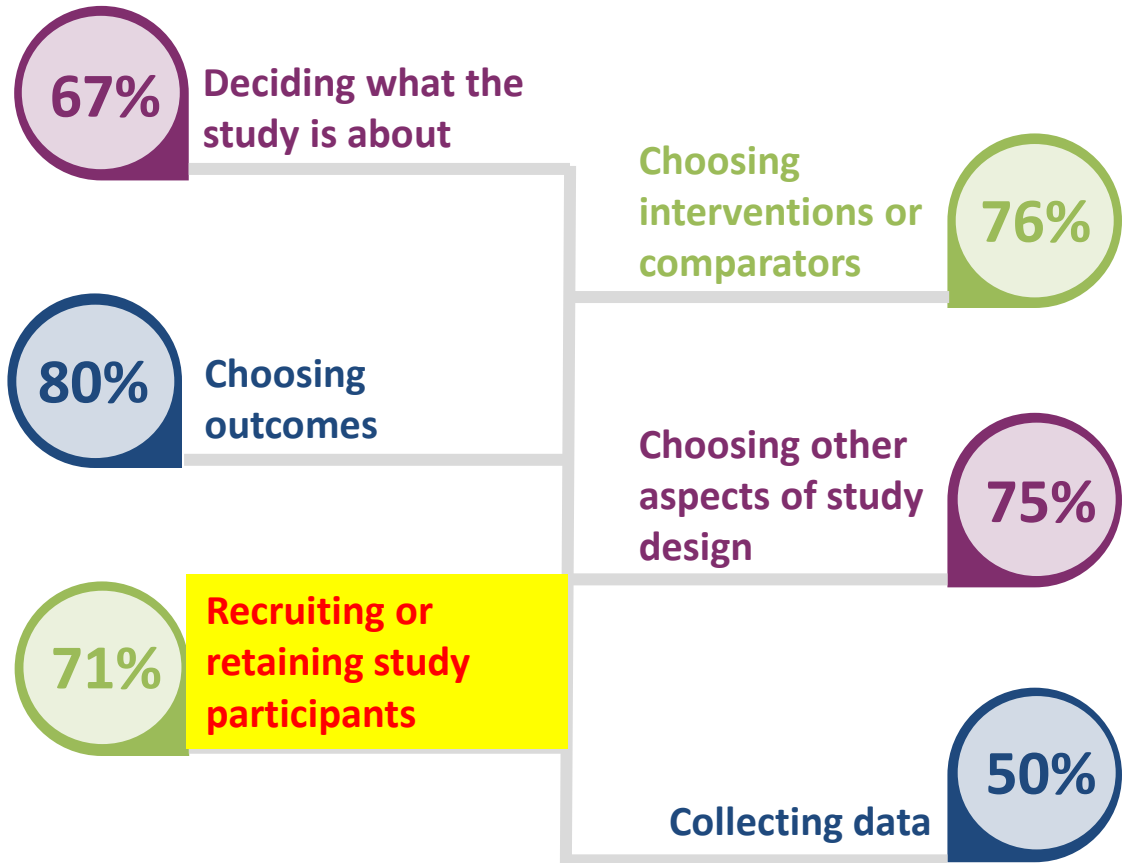


# PCORI projects engage stakeholders throughout the entire research process

## 3 out of 5

At least two-thirds of PCORI projects engage stakeholders in each early phase of research so that the studies matter to patients

### Research Phases Engaging Stakeholders: (by percent of projects)

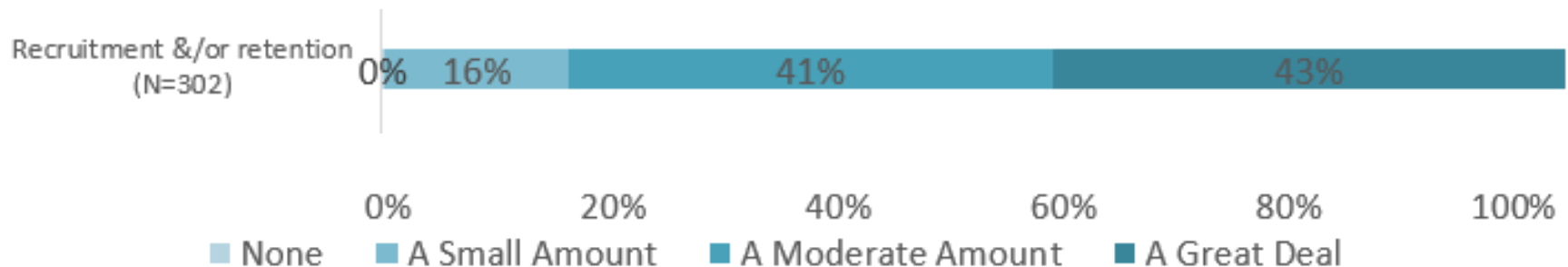


Note: Data from annual awardee reports collected through 6/30/17; N=305 awardees: 70 projects at project year 1, 116 projects at project year 2, 119 projects at project year 3.



# Nearly all researchers believe that stakeholders influence each part of the research process

- When PCORI researchers engage stakeholders in the research process, they feel it has an influence
- Specifically in recruitment and retention, 84% report a moderate to great deal of influence



*Note: Data from annual awardee report.*

*N indicates number of awardee respondents who reported engaging partners in that particular study phase and who answered corresponding influence question*



# Capturing the patient perspective in PCORI studies: recruitment

- Strategies for recruitment of patients with the specific condition, refine messaging
- Help developing outreach materials/screening tools
- Liaison between researchers and patient groups being recruited
- On-the-ground recruiting of study participants, practices, and partner organizations, raising awareness

“...helped researchers understand potential barriers to enrollment, particularly for minority candidates, and identified responses to these barriers.”

“We’ve had only one participant decline to participate since discussing recruitment with patients.”

“Stakeholder Co-I's relationship with individuals similar to those being recruited allowed her to provide insights on this population that is often difficult to recruit”



# Most common research partner engagement activities and effects on study recruitment, enrollment, and retention

## Activities

- Guidance and feedback:
  - Recruitment materials
  - Approaches to recruitment
  - Approaches to ensuring ongoing data collection of enrolled study participants
- Partners recruited study participants

## Effects

- More patient-centered
  - Recruitment procedures
  - Recruitment materials
  - Retention procedures
- More robust enrollment and retention



# Results of Engagement in Research: Stakeholder Involvement Led to Improved Enrollment

Minneci PC, Nacion KM, Lodwick DL, Cooper JN, Deans KJ. **Improving Surgical Research by Involving Stakeholders.** *JAMA Surgery.* Feb 2016.

- Awarded 2013, Assessment of Prevention, Diagnosis, and Treatment Options project
- Principal Investigator: Katherine Deans, MD, Nationwide Children's Hospital

In this PCORI-funded study of a patient activation tool (part of a larger comparison of surgery vs. antibiotics to treat pediatric appendicitis), **stakeholders provided suggestions help improve enrollment and retention rates**, including making the enrollment script more patient- and family-centered and offering an online option for follow-up.

These changes **increased enrollment in the trial from 65% to 95% and increased retention from 58% to 85%.**



In the **Top 5%** of research outputs scored by Altmetric



*These are tangible statistics that show that this process of involving the stakeholders can improve the study.*

Dr. Minneci, Co-Investigator



*This is why we have our stakeholder group, so that we can incorporate their input into all phases of the study. In this situation, it was critical to the success of our project.*

Dr. Deans, Principal Investigator



# Results of Engagement in Research: PCORI Studies of Patients with Kidney Diseases

Cukor D, Cohen LM, et al. **Patient and Other Stakeholder Engagement in Patient-Centered Outcomes Research Institute Funded Studies of Patients with Kidney Diseases.** *Clin J Amer Soc Nephrol.* May 2016.

Co-authored by researchers, patients, and stakeholders from 7 PCORI-funded studies focused on Kidney Disease, including a Patient-Powered Research Network focused on nephrotic syndrome, the article identified 3 early examples where engagement made a difference in the study:

1. Stakeholder advocacy motivated a major dialysis provider organization to find creative solutions to accommodate novel treatment delivery options in order to participate in the study (PI: Mehrotra)
2. Patient advisory panel feedback on the development of a decision aide helped ensure it was appropriate for the target audience of predialysis CKD patients and their caregivers (PI: Tentori)
3. Community engagement with the Zuni people helped identify psychologic and structural barriers that could be a challenge in a population with the highest prevalence of dialysis-requiring kidney diseases (PI: Shah)



*...The nephrology research community could serve as a model for implementing the ideals of community-based participatory research and patient-centered methodologies.*

#### **PCORI Projects with contributing authors:**

- PI: Lewis Cohen
- PI: Elizabeth Cope
- PI: Nashrollah Ghahramani
- PI: Denise Hynes
- PI: Rajnish Mehrotra
- PI: Vallabh Shah
- PI: Francesca Tentori



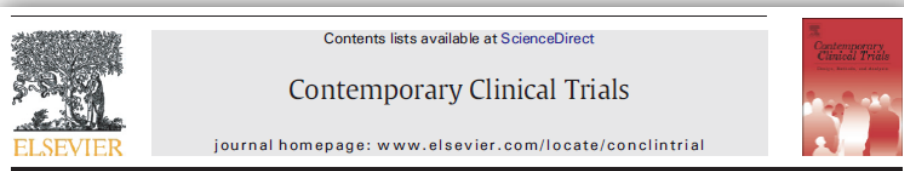
# Recruitment & Retention of Underserved Populations



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# What does the literature tell us?



Review  
Strategies addressing barriers to clinical trial enrollment of underrepresented populations: A systematic review  
Caren Heller<sup>a</sup>, Joyce E. Balls-Berry<sup>b,c,\*</sup>, Jill Dumbauld Nery<sup>d</sup>, Patricia J. Erwin<sup>e</sup>, Dawn Littleton<sup>e</sup>, Mimi Kim<sup>f,g</sup>, Winston P. Kuo<sup>h,i</sup>



## Enrolling African-American and Latino patients with asthma in comparative effectiveness research: Lessons learned from 8 patient-centered studies



C. Bradley Kramer, MPA,<sup>a</sup> Lisa LeRoy, MBA, PhD,<sup>b</sup> Sara Donahue, DrPH, MPH,<sup>b</sup> Andrea J. Apter, MD,<sup>c</sup> Tyra Bryant-Stephens, MD,<sup>d</sup> John P. Elder, MD,<sup>e</sup> Winifred J. Hamilton, MD,<sup>f</sup> Jerry A. Krishnan, MD, PhD,<sup>g</sup> Deborah Q. Shelef, MPH,<sup>h</sup> James W. Stout, MD, MPH,<sup>i</sup> Kaharu Sumino, MD, MPH,<sup>j</sup> Stephen J. Teach, MD, MPH,<sup>k</sup> and Alex D. Federman, MD, MPH<sup>l</sup>  
*Seattle, Wash, Cambridge, Mass, Philadelphia, Pa, San Diego, Calif, Houston, Tex, Chicago, Ill, Washington, DC, St Louis, Mo, and New York, NY*

Pediatr Blood Cancer 2013;60:1333–1337

## Exploring Barriers and Facilitators to Clinical Trial Enrollment in the Context of Sickle Cell Anemia and Hydroxyurea

Jeffrey D. Lebensburger, DO,<sup>1,\*</sup> Robert F. Sidonio, MD,<sup>2</sup> Michael R. DeBaun, MD, MPH,<sup>2</sup> Monika M. Safford, MD,<sup>3</sup> Thomas H. Howard, MD,<sup>1</sup> and Isabel C. Scarinci, PhD<sup>3</sup>

Journal of Surgery (2012) 203, 415–422

The American Journal of Surgery

Association of Black Academic Surgeons

## Increasing minority patient participation in cancer clinical trials using oncology nurse navigation

Dennis Ricky Holmes, M.D., F.A.C.S.<sup>a,\*</sup>, Jacquelyn Major, R.N., O.C.N.<sup>a</sup>, Doris Efosi Lyonga, M.D.<sup>b</sup>, Rebecca Simone Alleyne, M.D.<sup>c</sup>, Sheilah Marie Clayton, M.D., F.A.C.S.<sup>d</sup>



# What do PCORI studies tell us?

In Fall 2016, PCORI's Addressing Disparities team conducted a qualitative analysis of a sample of studies that did well with recruitment, identifying strategies that led to success, and general challenges encountered around recruitment and retention in underserved populations:

Recruitment Barriers	Recruitment Facilitators
<ul style="list-style-type: none"><li>• Conflict of time: work schedules, childcare, and personal time</li><li>• Transportation barriers</li><li>• Participation in research conducted at academic settings (i.e. feelings of intimidation and feeling out of place)</li><li>• Historical mistrust of institutions and research personnel</li></ul>	<ul style="list-style-type: none"><li>• Researchers should have a standing relationship with the community and the target population</li><li>• Key stakeholders should be included in all aspects of the research process, from the community needs and assets assessment to dissemination of results</li></ul>

\*Results of a recent survey on recruitment successes and challenges administered to a small sample of PIs will be available by January 2018.



# Current initiatives at PCORI

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- To further develop our understanding of facilitators and barriers to recruitment, enrollment and retention (RER), with targeted focus on understanding the unique challenges faced for underserved populations:
  - Use PCORI awardee-authored articles to build a comprehensive conceptual model regarding the relationship between engagement and RER outcomes
  - Use completed PCORI awards to test and analyze by sub-group through a multi-part analysis over the next 2 years
  - Administer a survey focused on recruitment successes and challenges to a sample of PIs with analysis completed by January 2018



# Thank You

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## **Engagement Resources:**

- [Engagement Rubric](#)
- [Budgeting for Engagement Activities](#)
- [Compensation Framework for Engaged Research Partners](#)
- [Sample Engagement Plans](#)
- [Methodology Report](#)
- [PCORI in Practice Webinar Series](#)

