Board of Governors Meeting
via Teleconference/Webinar

June 17, 2014
12:00-1:30 p.m. ET

Patient-Centered Outcomes Research Institute

Welcome and Introductions

Grayson Norquist, MD, MSPH

Chair, Board of Governors

Joe Selby, MD, MPH

Executive Director

Agenda

Time	Agenda Item
12:00 – 12:05 p.m.	Call to Order and Welcome
12:05– 12:10 p.m.	Consent Agenda • Minutes of May 5, 2014 Board Meeting • Naming the Chairs and Co-Chairs of the Clinical Trials and Rare Disease Advisory Panels • Naming Methodology Committee Member Steve Goodman to the Research Transformation Committee
12:10 – 1:00 p.m.	Overview of the Clinical Effectiveness Research Program
1:00 – 1:20 p.m.	Portfolio Analysis
1:20 – 1:25 p.m.	Update on Topic Selection Process
1:25 – 1:30 p.m.	Wrap Up and Adjournment

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Patient-Centered Outcomes Research Institute

Clinical Trials Advisory Panel & Rare Disease Advisory Panel Leadership

Bryan Luce, PhD, MBA
Chief Science Officer, PCORI

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Advisory Panel on Clinical Trials Proposed Leadership

CHAIR: Elizabeth Stuart, PhD, AM

Researcher (Biostatistician)

Associate Professor of Mental Health and Biostatistics, The Johns Hopkins Bloomberg School of Public Health

American Statistical Association (nomination)

Academic statistician with expertise in randomized trials, including handling complexities such as missing data, clustering, mediation analysis, and noncompliance. Previously, as a researcher at Mathematica Policy Research, she worked on a number of large-scale randomized trials of social interventions.

CO-CHAIR: John D. Lantos, MD

Expert in the Ethical Dimensions of Clinical Trials

Children's Mercy Hospital (Kansas City, MO)

Pediatrician, bioethicist, and leader in academic medicine. After 20 years on the faculty of The University of Chicago's Pritzker School of Medicine, he moved to Kansas City to create and direct a pediatric bioethics center at Children's Mercy Hospital. His research focuses on the ethics of clinical trials, and he has analyzed the ethical issues in neonatology, cancer chemotherapy, renal dialysis, cardiac assist devices, and primary care pediatrics. His research has been funded by National Institutes of Health, Robert Wood Johnson Foundation, and The John Templeton Foundation.

Advisory Panel on Rare Disease Proposed Leadership

CHAIR: Marshall L. Summar, MD

Clinician

Children's National Medical Center

Geneticist and pediatrician with expertise in rare diseases. His laboratory works on devices and treatments for patients with genetic diseases and adapting knowledge from rare diseases to mainstream medicine, and his research team is best known for its work in rare diseases affecting nitrogen and ammonia metabolism. He also works on newborn screening issues, developing testing and follow-up systems.

CO-CHAIR: Vincent Del Gaizo

Caregiver

Friends of Childhood Arthritis & Rheumatology Research Alliance (CARRA)

Father of a systematic-onset juvenile idiopathic arthritis patient and actively involved in the field of pediatric rheumatology research. For the past 12 years, he has been in the field of pediatric rheumatology research, directly contributing the patient voice and perspective to research initiatives in these rare diseases. He is a founding member and current Chair of Friends of CARRA, a nonprofit organization established to promote needed research and awareness of pediatric rheumatic conditions.

Board Vote: Consent Agenda

Item 1:	• **Approve** May 5, 2014 Board meeting minutes
Item 2:	• **Approve** the nominated chairs and co-chairs: Clinical Trials Advisory Panel Chair, Elizabeth Stuart and Co-Chair, John Lantos Rare Disease Advisory Panel Chair, Marshall Summar and Co-Chair, Vincent Del Gaizo
Item 3:	• **Approve** the appointment of Steve Goodman to the Research Transformation Committee as a Methodology Committee member
Vote:	• Vote to **approve the items** listed on the Consent Agenda

Clinical Effectiveness Research Program Portfolio

David Hickam, MD, MPH

Director, Clinical Effectiveness Research Program

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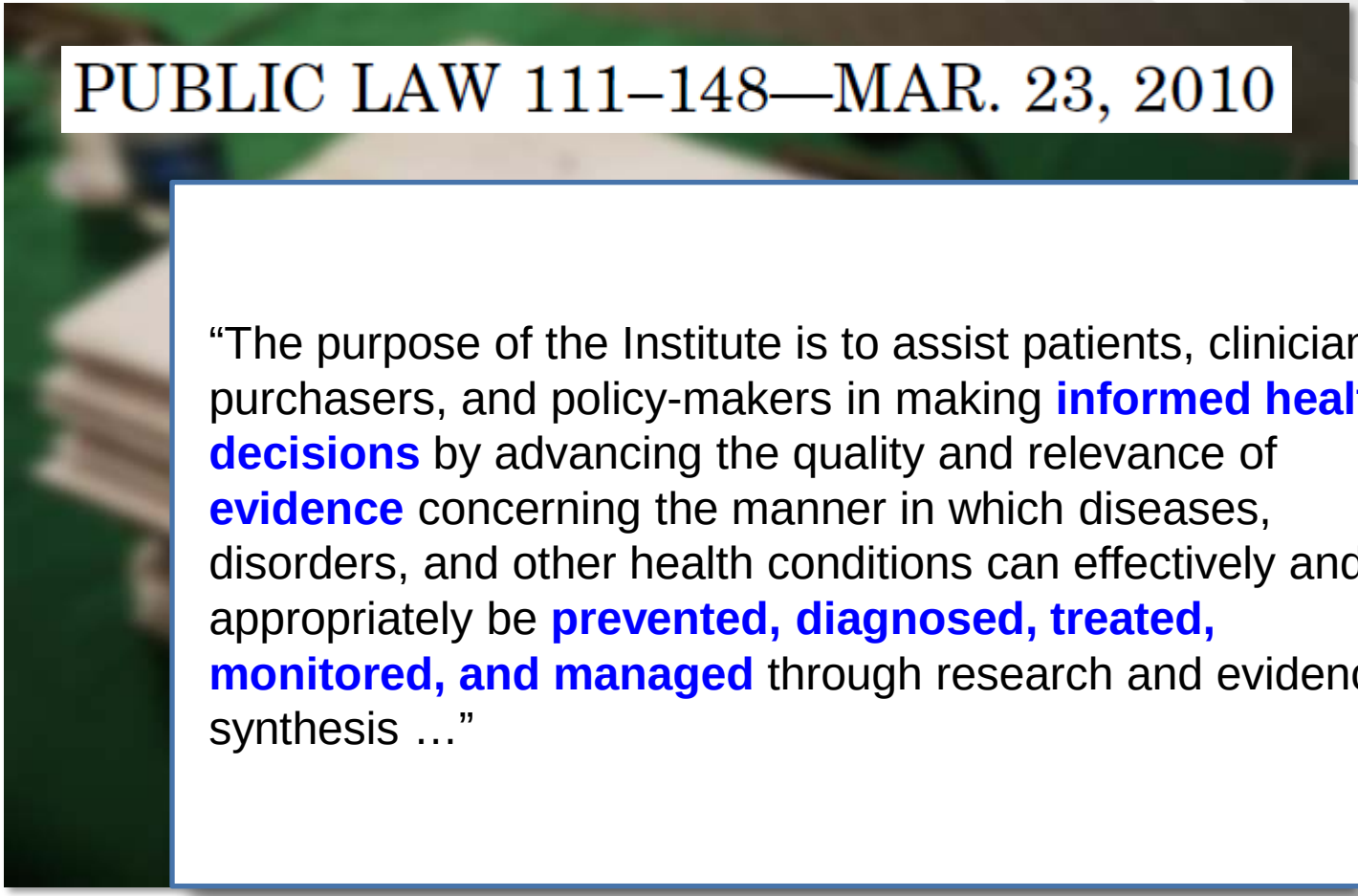
Agenda

- Program perspectives and goals
- Development of the current portfolio of research projects
- Plans for the next 12 months

PCORI's Mandate



"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 …"

Starting Point: The PCORI Methodology Standards

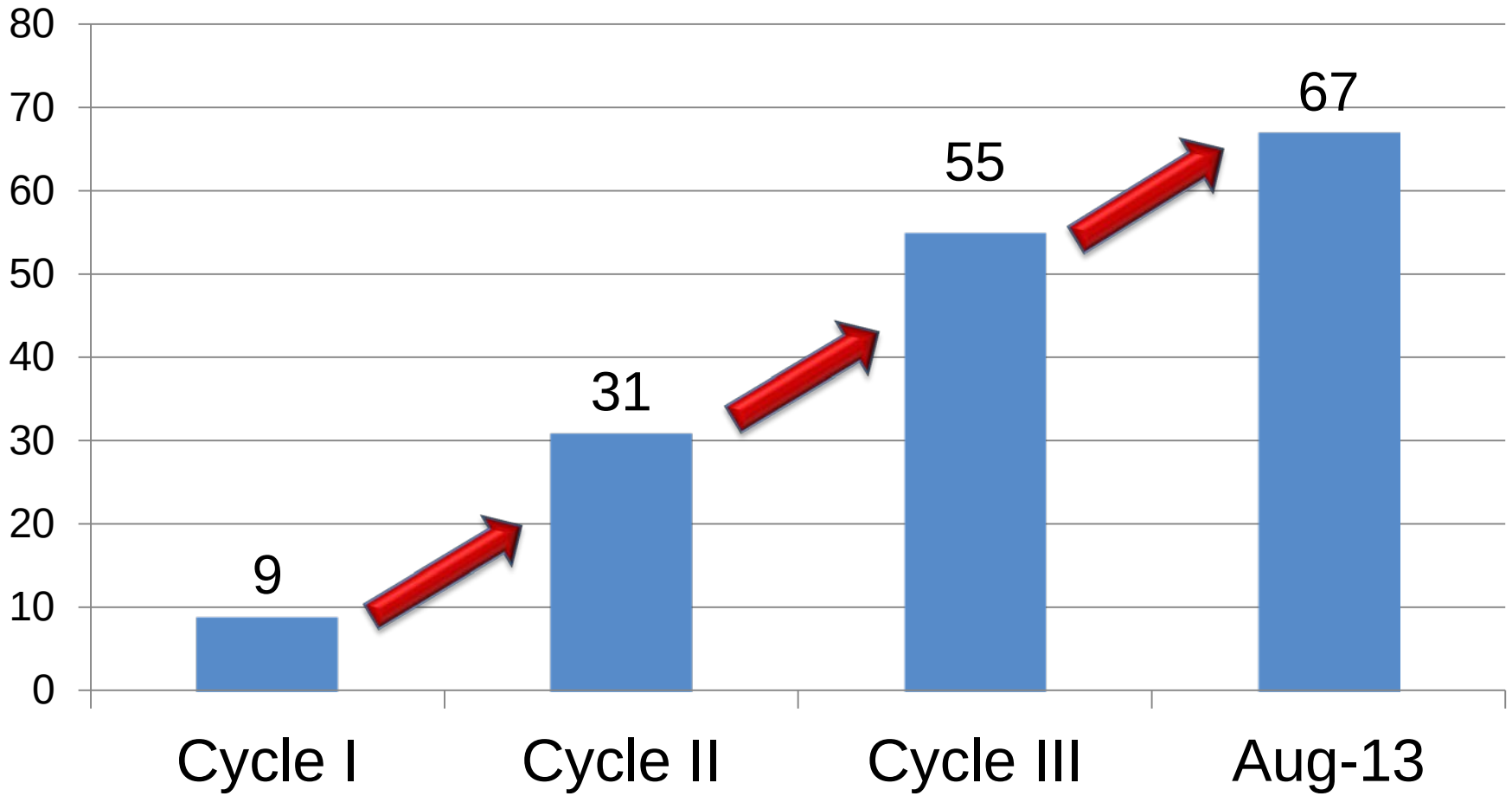
- Identify gaps in evidence (RQ-1)
- Identify specific populations and health decisions (RQ-3)
- Select appropriate interventions and comparators (RQ-5)
- Measure outcomes that people notice and care about (RQ-6)

RQ="Standards for Formulating Research Questions"

Developing the Broad Research Program: Assessment of Prevention, Diagnosis and Treatment Options

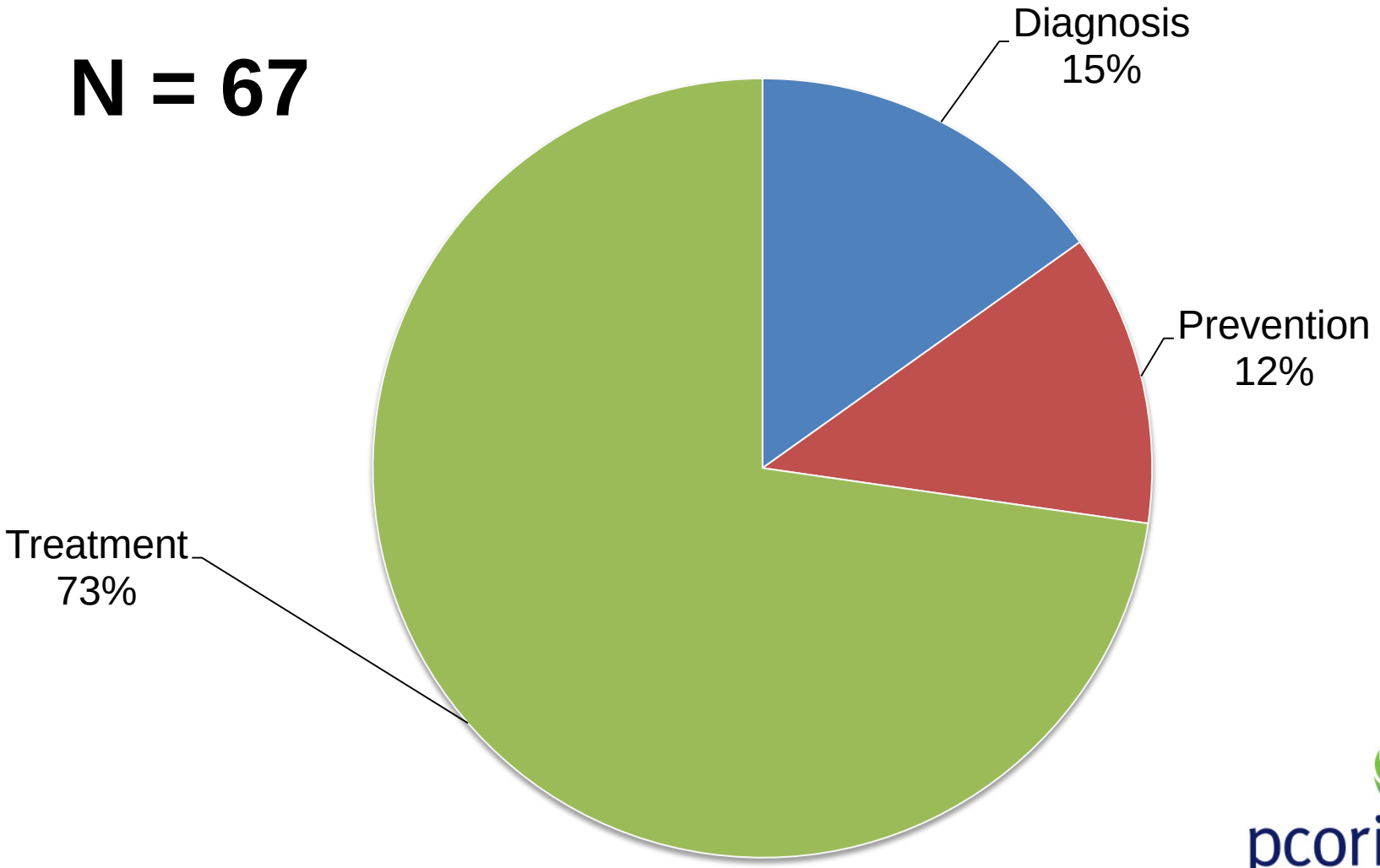
- Investigator-initiated research contracts
  - Projects must be comparative
  - No specified priority clinical topics
- Broad PCORI Funding Announcement (PFA) first released in summer 2012
- Completed 4 funding cycles in first 18 months
- Limited size of projects
  - Budget no greater than $1.5 million in direct costs
  - Project duration no greater than 3 years
- Required adherence to PCORI Methodology Standards
- Continuous improvement of merit review process

Assessment of Options Program: Cumulative number of funded awards ($115.5M)

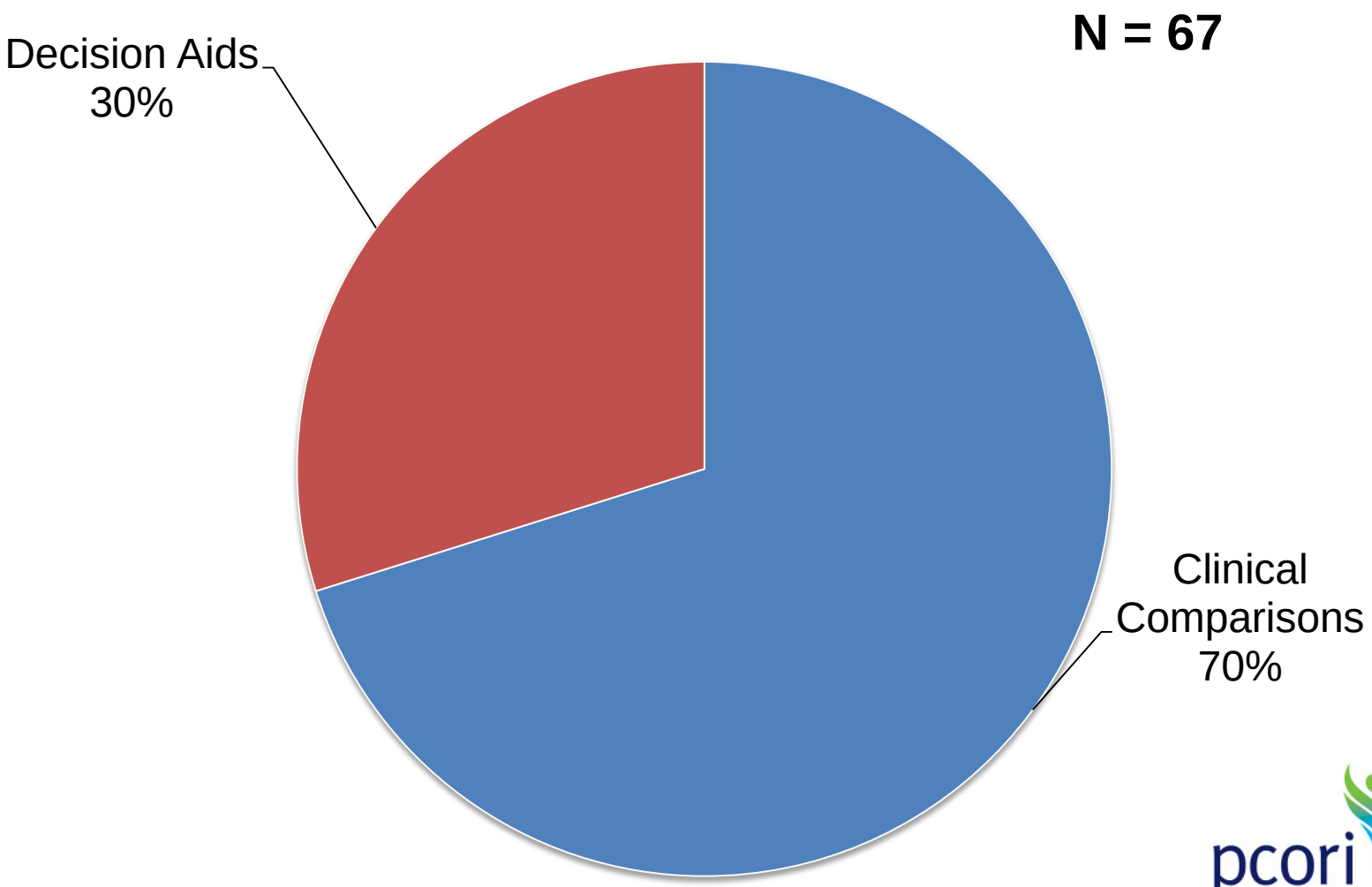


Clinical Focus of Funded Projects

N = 67



Types of Interventions



Sample Sizes for Clinical Trials

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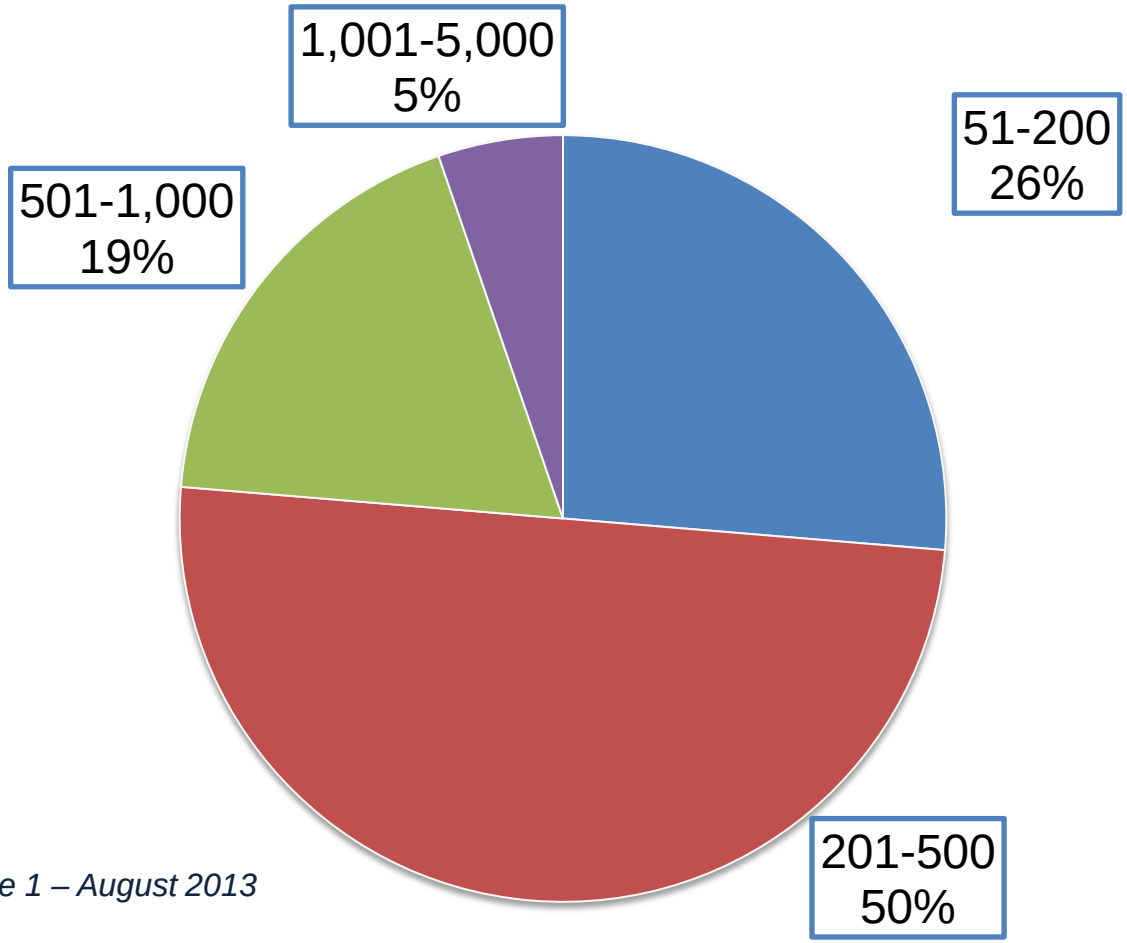

Cycles funded: Cycle 1 – August 2013

Sample Sizes for Observational Studies

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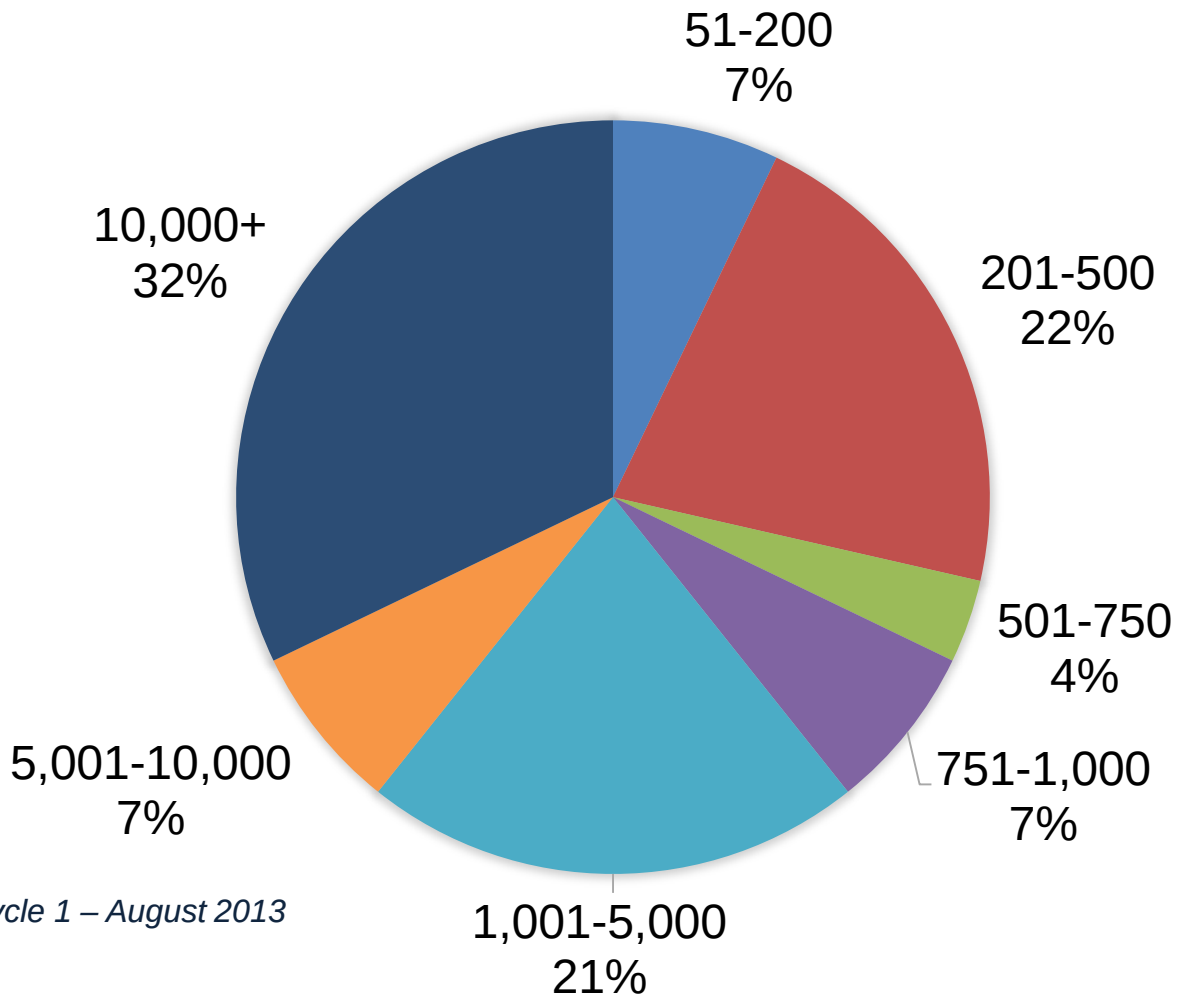


Cycles funded: Cycle 1 – August 2013

Study on a Tool to Promote Lung Cancer Screening among Tobacco Users

- **Promoting Informed Decisions about Lung Cancer Screening**
  - PI – Robert Volk, PhD
  - University of Texas MD Anderson Cancer Center
- Compare two strategies for providing information to help people decide whether to pursue screening via CT scanning
- Will assess changes in intentions and receipt of screening

Study on Diagnostic Methods for Detecting Recurrence among Women Previously Treated for Breast Cancer

- **Comparative Effectiveness of Surveillance Imaging Modalities in Breast Cancer Survivors**
  - PI – Karen Wernli, PhD, MS
  - Group Health Cooperative, Seattle, WA
- Data on 36,000 women diagnosed with breast cancer from 2005-2012 in whom either surveillance mammograms or breast MRI examinations have been obtained in 7 diverse regions across the U.S.
- Will assess breast cancer mortality, cancers missed, false positive rates, and biopsy rates

Adaptive Clinical Trial to Compare Medications for Painful Peripheral Neuropathy

- **Patient Assisted Intervention for Neuropathy: Comparison of Treatment in Real Life Situations**
  - PI – Richard Barohn, MD
  - University of Kansas Medical Center
- Will enroll 400 patients in a prospective adaptive trial and will compare four medications: adaptive randomization will help to increase power to detect differences among the drugs
- Primary outcome is pain relief

Characteristics of the Clinical Trials

- 39 projects as of May 2014
- Moderate size
  - Usually single-center trials
  - More participants when cluster designs are used
  - Limited power for sub-group comparisons
  - Accrual of participants is highly important
- Interventions are diverse:
  - Medications
  - Surgical interventions
  - Decision aids
  - Self-care tools

Trials Comparing Clinical Therapies

- Drugs for childhood epilepsy
- Drugs for neuropathic pain
- Surgical techniques for cervical disk disorders
- Manipulative and non-manipulative treatment for back pain
- Physical therapy regimens for knee arthritis
- Nicotine replacement regimens
- Weight loss programs
- Treatments to prevent dementia
- Counseling interventions in mental health (three trials)

Trials of Interventions to Promote Self-Care

- Management of symptoms in cancer patients
- Pain management
- Exercise in older adults
- Mobilization after back surgery
- Cardiovascular risk reduction
- Medication adherence
- Home oxygen adherence
- Home glucose monitoring

Trials of Interventions for Caregivers

- Caregivers of patients receiving allogeneic stem cell transplants
- Caregivers of elderly patients with dementia
- Parents of children with severe injuries or critical illness (three trials)

Trials to Assess the Impact of Decision Aids

- Decision to obtain screening for lung cancer
- Treatment options for appendicitis
- Choosing methods for contraception
- Choosing treatments for back pain
- Treatment options for lupus
- Cancer treatment choices (four trials)
- Guidance for use of diagnostic tests (three trials)

New Initiatives Derived from Stakeholder-Based Clinical Priorities

- Opportunity to identify important evidence gaps
  - Nomination of clinical topics
  - Advisory panel
- Targeted initiative on treatment options for uterine fibroids (AHRQ partnership)
- Pragmatic Studies Announcement
  - PFA first released in February 2014
  - Competitive Letters of Interest (LOIs)
  - Larger budgets and longer project durations
  - Collaboration with Addressing Disparities and Improving Health Systems Programs

Priority Topics for the First Pragmatic Studies Announcement

- Treatments to prevent the transition from episodic to chronic migraine
- Smoking cessation therapies in high risk persons
- Treatments to prevent the transition from episodic to chronic low back pain
- Diagnosis and management of bipolar disorder in children and adolescents
- Treatment strategies for osteoarthritis
- Management of ductal carcinoma *in situ*
- Strategy for follow-up of incidentally discovered pulmonary nodules

Priority Topics for the First Pragmatic Studies Announcement

- Treatments for multiple sclerosis
- Treatment strategies for autism spectrum disorder
- Proton therapy for breast, prostate, and lung cancer
- Treatment of opioid substance abuse
- Biological agents in Crohn's Disease
- Hemodialysis vs. peritoneal dialysis

Conclusions

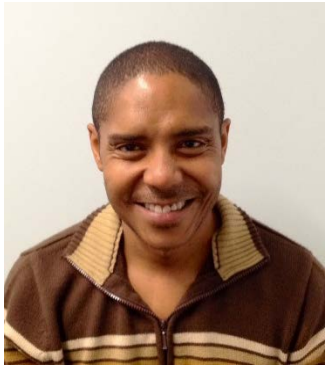
- PCORI has successfully developed a portfolio that includes a wide variety of studies that compare clinical options across a broad range of clinical questions
- The studies are aligned with the PCORI Methodology Standards
- This program is unique among U.S. research funding organizations
  - Emphasis on comparative effectiveness
  - Will require ongoing evaluation regarding usefulness
- Future initiatives will increasingly be developed from clinical priority topics that are based on patient and stakeholder input

The Clinical Effectiveness Research Program Team

Diane Bild, MD, MPH
Senior Program Officer

Jana-Lynn Louis, MPH
Program Associate

David Hickam, MD, MPH
Program Director



Raymond Lockett
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Julie McCormack, MA
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Senior Program Officer

Hal Sox, MD
Consultant

Portfolio Analysis

Christine Goertz, DC, PhD
Chair, Science Oversight Committee

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Update on Topic Selection Process

Christine Goertz, DC, PhD
Chair, Science Oversight Committee

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Wrap-up and Adjournment

Grayson Norquist, MD, MSPH
Chair, Board of Governors

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