

Board of Governors Meeting via Teleconference/Webinar

December 7, 2015

10:10 a.m. - 5:30 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



Board Vote

Call for a Motion to:

- **Approve** the meeting minutes from the November 17, 2015 board meeting

Call for the Motion to Be Seconded:

- **Second** the Motion
 - If further discussion, may propose an **Amendment** to the Motion or an **Alternative Motion**

Voice Vote:

- Vote to **Approve** the **Final** Motion
- Ask for votes in favor, opposed, and abstentions



Engagement in PCORnet

Sue Sheridan, MBA, MIM

Director, Patient Engagement

Sharon Terry, MA

CEO, PXE International

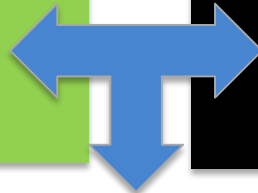
President & CEO, Genetic Alliance



Engagement as a Tool for Transformation

**PCORI is about
Research
Done Differently**

**PCORnet is about
Research Infrastructure
Done Differently**



**Engaging patients and
stakeholders is the
cornerstone**



PCORI Engagement Priorities and Long Term Goals

Develop Community Skilled in PCOR

to successfully establish an infrastructure for patients, caregivers, and other stakeholders to increase information and engage them in research, dissemination and evaluation



Engage Community in Research Process...

to influence research to be patient-centered, relevant, and useful,
to establish trust and a sense of legitimacy in research findings and
to encourage successful uptake and use of research results

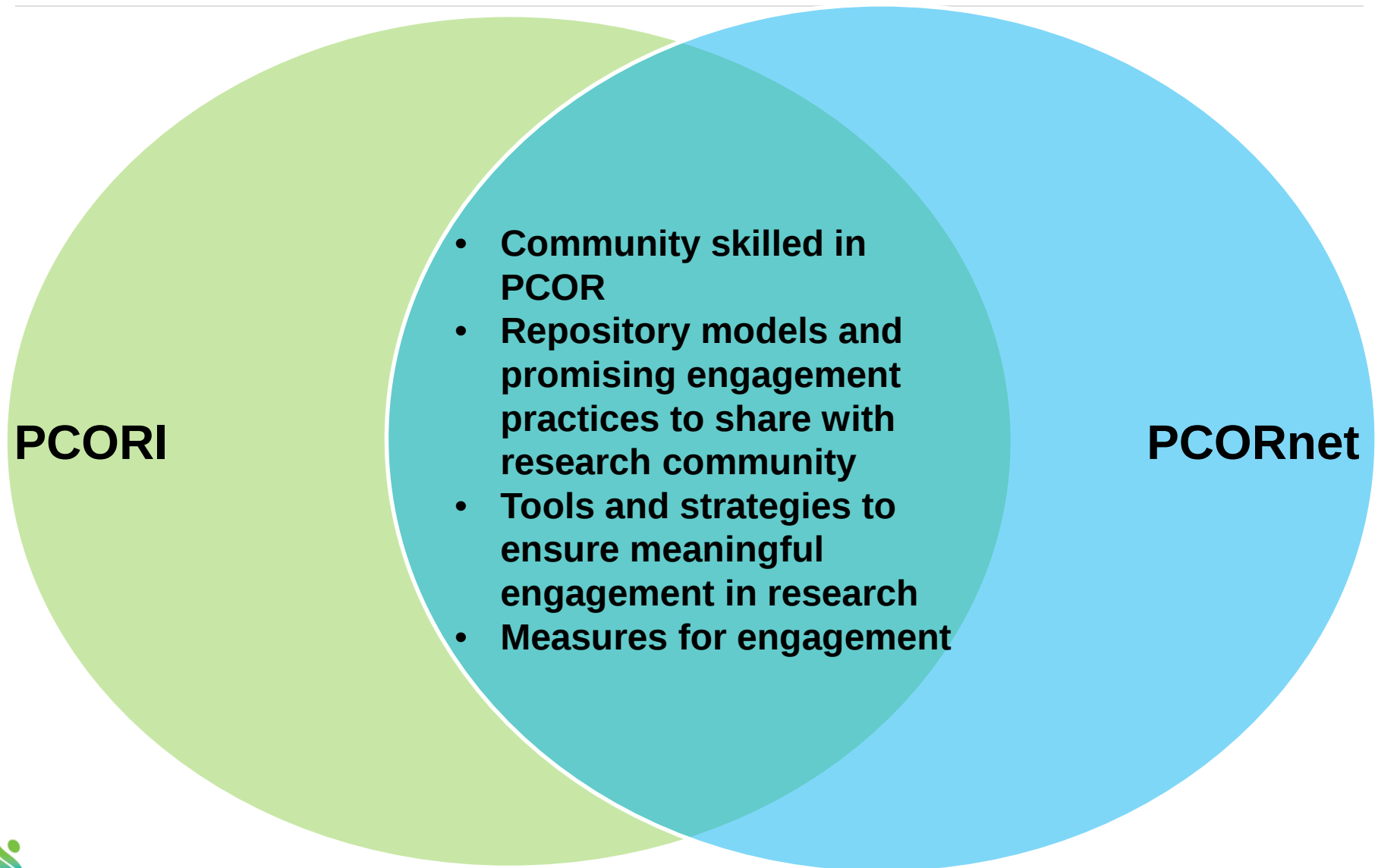


Promote Dissemination and Implementation...

so that patients, caregivers and other stakeholders have information they
can use to make decisions that reflect their desired health outcomes and to
speed implementation of our findings



Engagement in PCORI and PCORnet: Common Facilitators for Success



PCORI Engagement Tools and Resources to Support PCORnet Engagement in Phase II

Tools and strategies to ensure meaningful engagement in research:

- Engagement Rubric
- Patient Engagement Framework for Patient-Centered Outcomes Research (PCOR) in Infrastructure Development
- Compensation Framework

Tools and strategies to evaluate engagement:

- Examples of metrics and measures of engagement

Why develop a rubric?

What is the rubric?

How will the rubric be used?



PCORI Engagement Tools and Resources to Support PCORnet Engagement in Phase II

Training, capacity building tools and programs to develop community skilled in PCOR

- Eugene Washington Engagement Awards
- PCOR/CER training
- Ambassadors
- Team Science curriculum
- Rubric training video



BeTTER SAID (Bringing Stakeholders Together for Engagement in Research for the Selection of Arthroplasty Implant Devices)

Project Aims

- Prepare arthroplasty patients and other stakeholders to participate collaboratively in patient-centered CER through **Interactive Training Webinars**.
- Train patients and other stakeholders to be successful partners in research on the selection process of arthroplasty implant devices in a **Series of Roundtable Discussions**
- Prepare patients and other stakeholders to be informed ambassadors on arthroplasty implant research in a **summarizing Dissemination Webinar**

This new BeTTER SAID network capacity will be embedded within the existing infrastructure of the CAPriCORN CDRN, the NYC-CDRN, and the AR-PoWER PPRN and its online CreakyJoints patient community. The online community engagement and data collection platforms of AR-PoWER PPRN and CreakyJoints permit ongoing engagement of BeTTER SAID patients as research partners and as informed decision makers and consumers of health care.

*Global Healthy Living Foundation, Inc.
New York, NY*

*Eugene Washington PCORI Engagement Awards,
Awarded October 2015*



Big Data for Patients (BD4P)

Potential Impact

- BD4P will develop a community of informed and empowered advocates who understand what Big Data is, how it is being used in research and medicine, its promises and limitations, the challenges, the impact on patients, and how they can use this knowledge

Engagement

- Patients will be a part of the organizing committee, program team, and workshop leadership. They will inform the needs assessment, curriculum development, workshop content and community of practice

BD4P will empower patients to participate more fully—and effectively—in health research involving data science. BD4P will foster the development of a community of patients who understand:

- The key language and scientific concepts underlying Big Data
- How Big Data is being used in research and medicine
- The promise, limitations, and potential impacts on patients
- How patients can impact Big Data research, policy and funding decisions

*Reagan-Udall Foundation for the FDA
Washington, DC*

*Eugene Washington PCORI Engagement Awards,
Awarded June 2015*



PCORnet Engagement

Sharon F. Terry, MA

CEO, PXE International

President & CEO, Genetic Alliance

PI, Community Engaged Network for All (PPRN)

Chair, Engagement Committee

Member of the Executive Committee

Co-PI, Coordinating Center



Engagement Goal for PCORnet

Create a truly participant/patient-centered research network by systematizing overall PCORnet engagement, governance, leadership, and research practices; implement a strong process for measurable engagement strategies, and ensure that all PCORnet activities engage all “participant” types. This will be executed on two levels:

1. PCORnet enterprise-level
2. Individual network-level and will focus engagement on several stakeholder types
 - a) Patient/participant
 - b) Clinician/provider
 - c) Investigator/researcher
 - d) Community/systems leader



Engagement Workgroup

- Sharon Terry: CENA PPRN and Coordinating Center
- Rachael Fleurence: PCORI
- Sue Sheridan: PCORI
- Bray Patrick-Lake: Coordinating Center
- Rebekah Angove: REACHnet (formerly LaCDRN)
- Bill Clark: COPD PPRN
- Lucinda Jewell: MOOD PPRN
- Holly Peay: DuchenneConnect PPRN
- Nicholas Uzl: CCFA PPRN
- Neely Williams, Mid-South CDRN



Engagement Committee

All engagement strategies, activities, and products will be monitored by the PCORnet Executive Committee (EC) and the PCORnet Council.



Observations

- PCORnet has begun to recognize participant/patient-led research in the form of the PPRNs; needs more
- Engagement is valuable and must assess needs; understand experience, including utilization and access to services; create meaningful and effective tools; and, amplify the voice of patients and families
- Engagement requires deep and authentic interactions across and within all of the networks, and beyond to the participants/patients served inside and outside PCORnet
- PCORnet needs coordination and systematization of engagement activities.
- PCORnet engagement must inherently be stakeholder driven, assisted centrally, and conducted throughout the network in a federated model
- PCORnet has the opportunity to improve population health—through meaningful stakeholder engagement—by addressing factors that impact health outcomes at the individual, interpersonal, and community levels



Strategic Activities

- Analysis of all engagement products, tools, and activities
- Framework for engagement activities over the three years of Phase II
- Development of criteria and measures for engagement
- Implement an improved process for systematic engagement strategies throughout
- Recommend engagement tactics within PCORnet
- Identify a process for ongoing coordination with PCORI to ensure that other activities, studies, and resources are being leveraged
- Outline a routine process for sharing and systematizing successful practices and tools across the Network and into individual networks and their institutions



PCORnet Engagement Key Driver Diagram

Goal

Create an authentic participant-centered research network by systematizing engagement of patients, clinicians, researchers, and community leaders

Drivers

Governance and leadership

Stakeholder involvement in all aspects of PCORnet studies

Develop and share models and best practices

Educate and activate the public about PCORnet practices.

Interventions

- Representation on PCORnet Council
- Transparent dashboard
- Incentive system for proper patient compensation

- Monitor participant-centricity
- Engagement checklist to ensure clinician involvement
- Monitor study relevance
- Provide peer-to-peer mentor system.

- Provide technical assistance, learning opportunities.
- Develop a clear public call to action.
- Ongoing external advisory group.



Building Trustworthiness in PCORnet

January 19 – 20, 2016

Workshop Objectives:

- Describe the characteristics of trustworthy engagement
- Examine successes and failures in building trustworthiness in research initiatives
- Begin a robust dialogue around trustworthiness within PCORnet
- Create recommendations for PCORnet and its stakeholders
- Create recommendations for the networks and stakeholders that comprise PCORnet

We do not ask: how do we get people to trust us?

We ask: how are we trustworthy and not trustworthy?

The onus is on us.



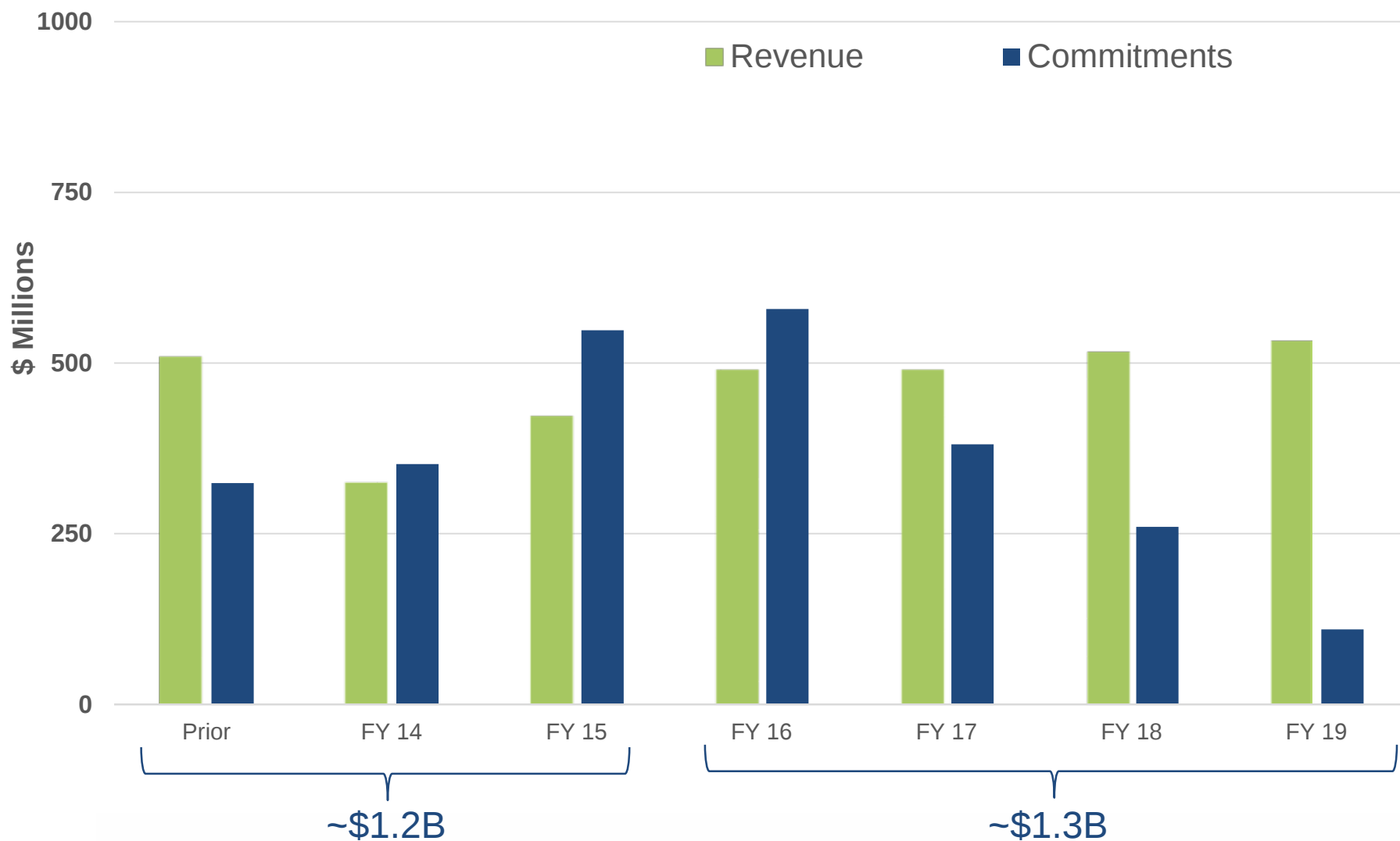
Executive Director's Report

Joe Selby, MD, MPH

Executive Director



PCORI Revenue and Commitments



A National Evidence-Generation Network



U.S. Food and Drug Administration
Protecting and Promoting Public Health

www.fda.gov

Medical Product Safety Surveillance

FDA



Sentinel
Coordinating
Center

Coordinating
Center(s)



Sponsor(s)

Medical Product Safety

Coordinating
Center(s)



Sponsor(s)

Clinical Research

Comparative Effectiveness Research

Sponsor(s)

Quality of Care

Sponsor(s)



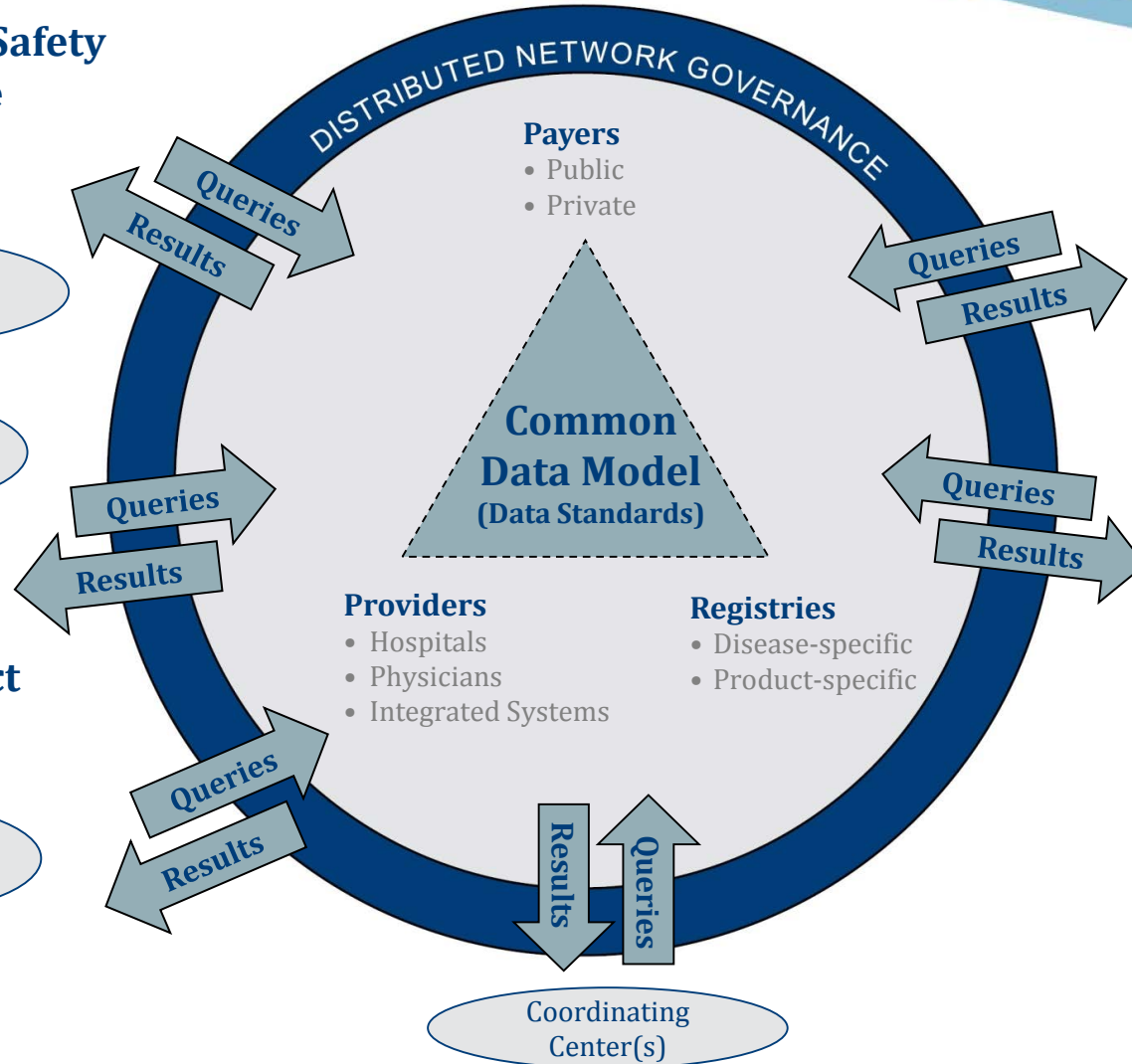
Coordinating
Center(s)

Coordinating
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Sponsor(s)

Public Health Surveillance



A National Evidence-Generation Network



U.S. Food and Drug Administration
Protecting and Promoting Public Health

www.fda.gov

Medical Product Safety Surveillance

FDA



Sentinel
Coordinating
Center

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Center(s)



Sponsor(s)

Medical Product Safety

Coordinating
Center(s)



Sponsor(s)

Clinical Research

Comparative Effectiveness Research

Sponsor(s)

Quality of Care

Sponsor(s)



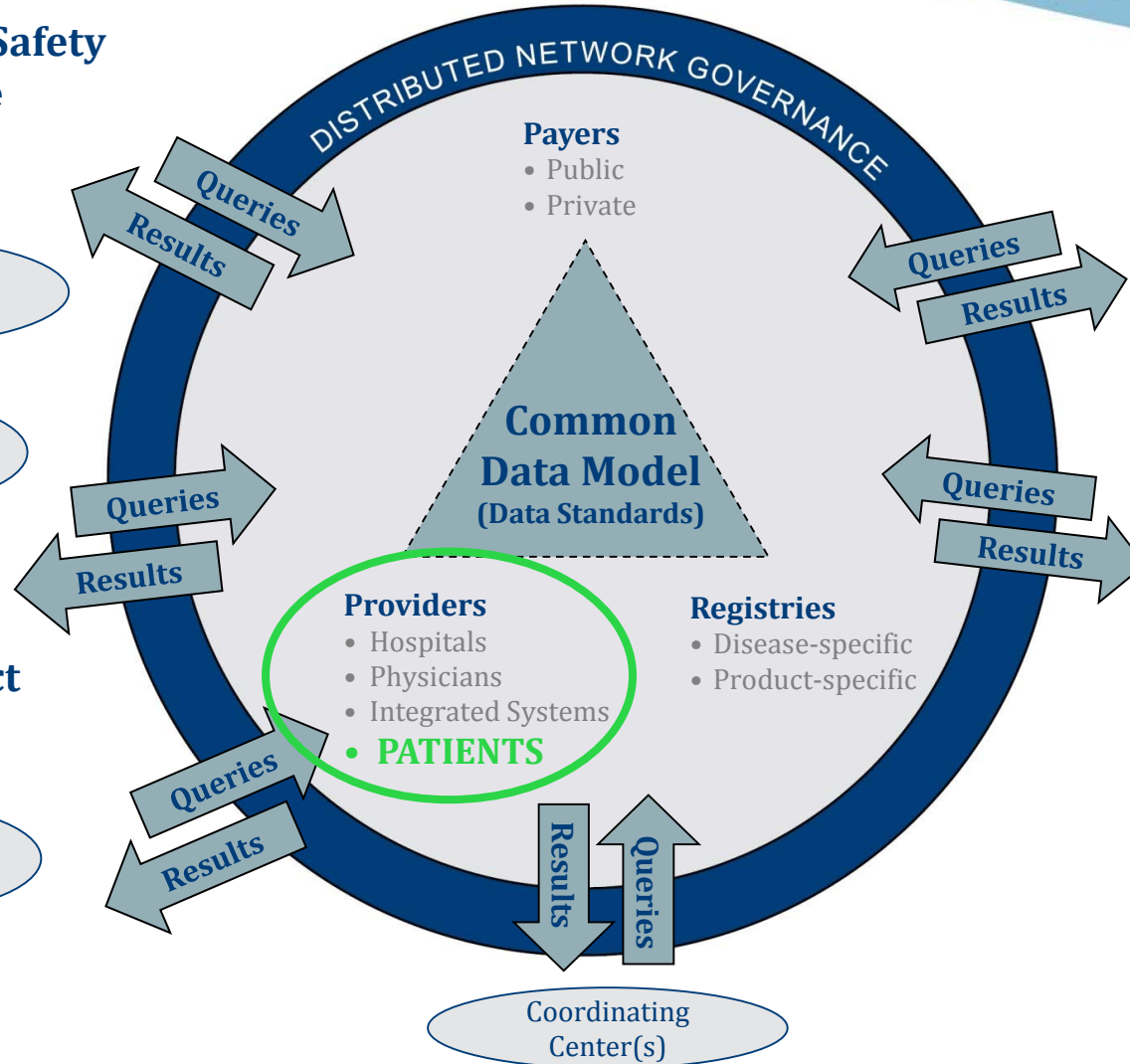
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Medical Product Safety Surveillance

FDA



Sentinel
Coordinating
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Center(s)



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Coordinating
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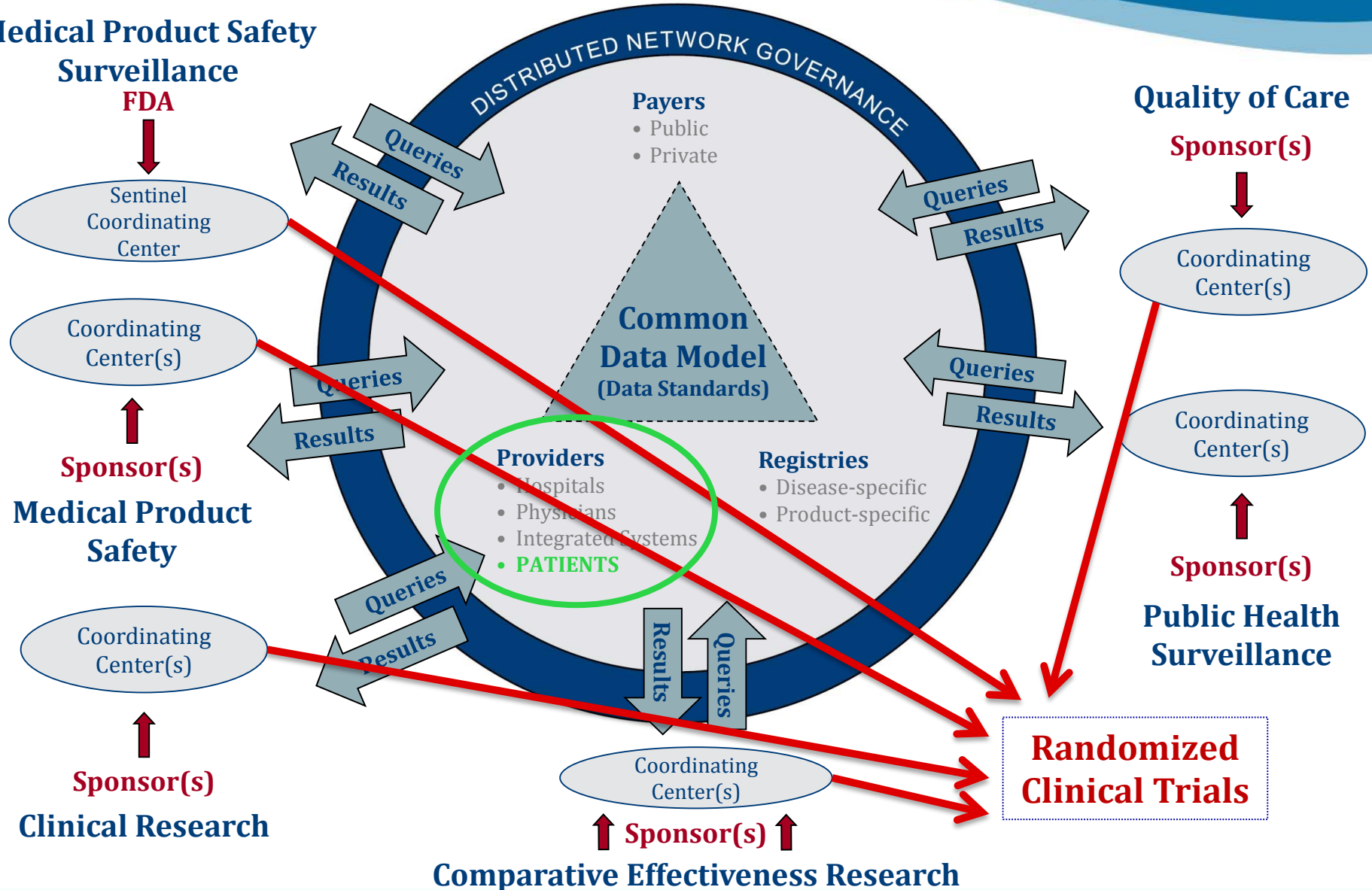
Coordinating
Center(s)



Sponsor(s)

Public Health Surveillance

**Randomized
Clinical Trials**



Important Upcoming Meetings:

- **Data Quality and Missing Data in Patient-Centered Outcomes Research using EMR/Claims Data:** Methodology Meeting with PCORnet and others, invitation only (December 7, 2015)
- **Back Pain** – This expert stakeholder working group will consider the subgroups of back pain patients that should be studied in a CER study of surgery vs. non-surgical care (January 7, 2016)
- **PCORnet Trustworthiness Meeting** – PCORnet hosts an open meeting with patients involved in PCORnet networks regarding the uses of personal health information for clinical research (January 19-20, 2016)
- **Accelerating Clinical Knowledge Generation and Use** – PCORI, PCORnet and National Academy of Medicine host Health system and health plan CEOs, many from PCORnet sites, to discuss the value of embedded research (January 20-21, 2016)



Involving Patients as Equal Partners in Research

- https://www.youtube.com/watch?v=XC_I2go1EIU&feature=youtu.be



Time	Agenda Item
10:10-10:15	Welcome, Call to Order, and Approve November 17, 2015 Board Meeting Minutes
10:15-11:00	Engagement in PCORnet
11:00-11:40	Executive Director’s Report and End-of-year Dashboard Review
11:40-12:15	Portfolio Portrait/Analyses
12:15-1:15	Break
1:15-2:15	Stakeholder Perspectives: Patients <i>Moderator: Gail Hunt, Board Member</i> • <i>Marc Boutin, JD, CEO, National Health Council</i> • <i>Donna Cryer, JD, President & CEO, Global Liver Institute</i> • <i>Celeste Castillo Lee, Institute for Patient and Family-Centered Care</i>
2:15-3:00	Consider for Approval: Release Revised and New Standards for Public Comment
3:00-3:15	Consider for Approval: Revised Selection Committee Charter
3:15-3:30	Break
3:30-4:00	Evaluation Update: Merit Review Score Analysis
4:00-4:30	Workforce Training Proposal
4:30-5:00	Progress on Open Science
5:00-5:30	Public Comment
5:30	Wrap Up and Adjournment

Dashboard Review

End of FY 2015

Joe Selby, MD, MPH

Executive Director

Michele Orza, ScD

Senior Advisor to the Executive Director

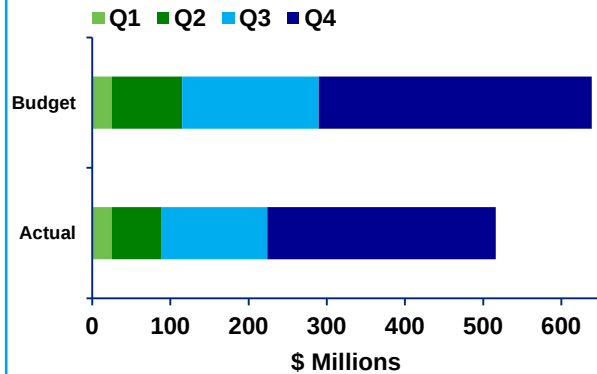


Discussion Questions

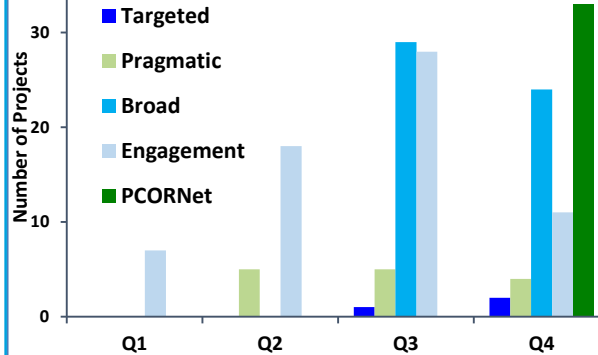
- What is your view of our *performance in FY 2015 and our status* as of the end of FY 2015?
- What are your thoughts about our *funding commitments over the next four years*?
- Is our *approach to targeted topics* optimal?



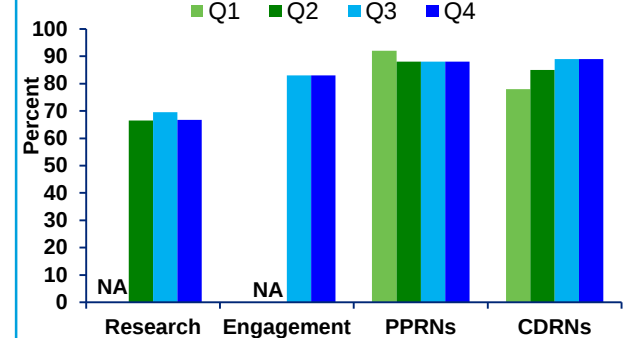
Funds Committed to Research – up to \$640M



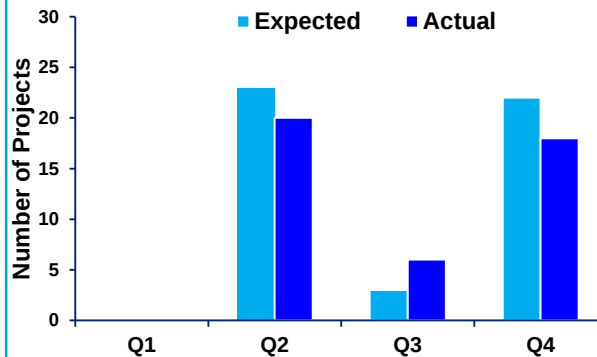
Projects Awarded



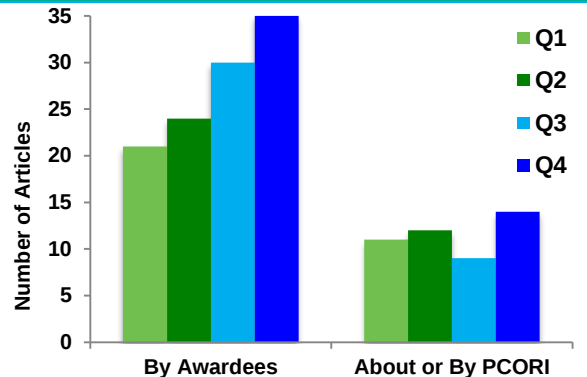
Percent of Projects on Track



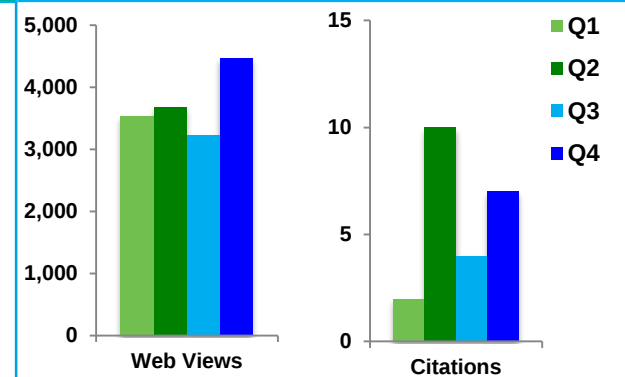
Projects Completed as Expected



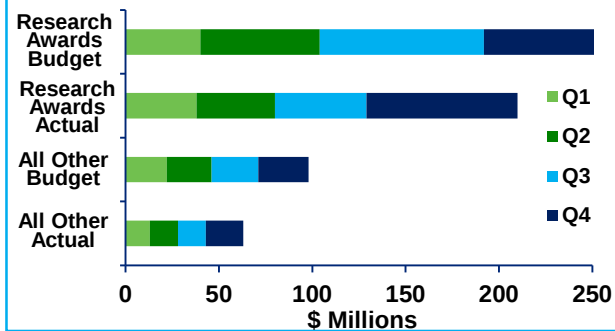
Journal Articles Published



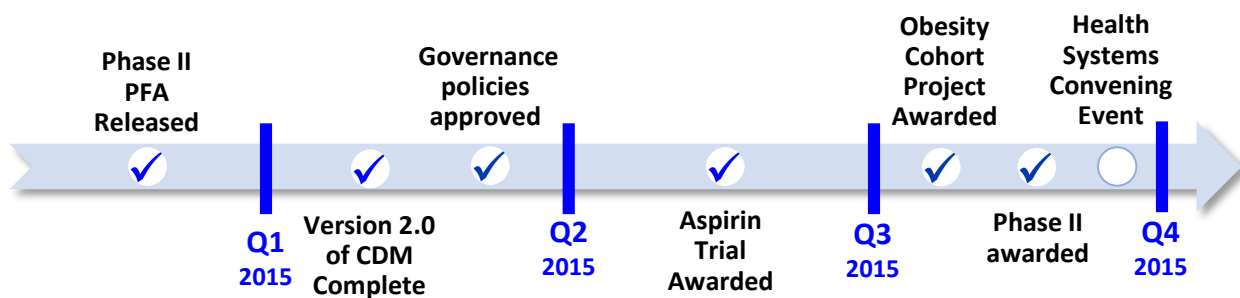
Uptake of Methodology Standards



Expenditures – Total Budget, up to \$362M



Progress of PCORNet – Completion of Phase I



Goal 3 Results: PCORI Influence on Curriculum

Influencing Research

A new graduate level Health Psychology course at Tulane University is modeled on PCORI's National Priorities for Research

Michael Hoerger, PhD

Assistant Professor of Psychology and Psychiatry
Tulane University

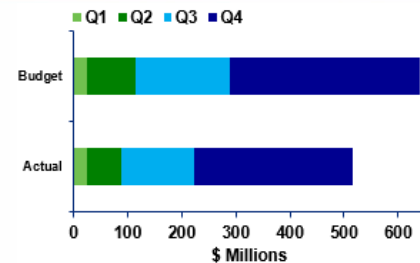
Designed a new Health Psychology course for the Masters Program in Behavioral Health based on PCORI's National Priorities for Research

- Course objective: prepare students for doctoral training in psychology so that they can ultimately pursue careers in clinical practice, research, and policy
- 10 modules, with 6 focused on PCORI
- Hoerger, M. (2015). Educating the Psychology Workforce in the Age of the Affordable Care Act: A Graduate Course Modeled After the Priorities of the Patient-Centered Outcomes Research Institute (PCORI). *Training and Education in Professional Psychology*. E-pub ahead of print.

“PCORI’s five national priorities are noteworthy for psychologists and psychology trainees in terms of suggesting potential funding areas and clarifying trends in US healthcare for psychologists.”

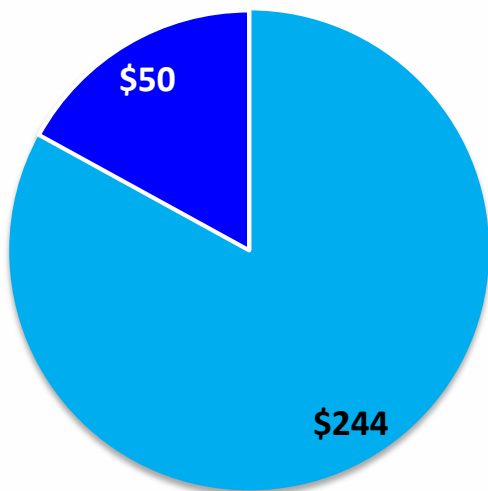


We are making progress on our Strategic Priority to “*Increase the proportion of research funding going to focused and targeted topics*”

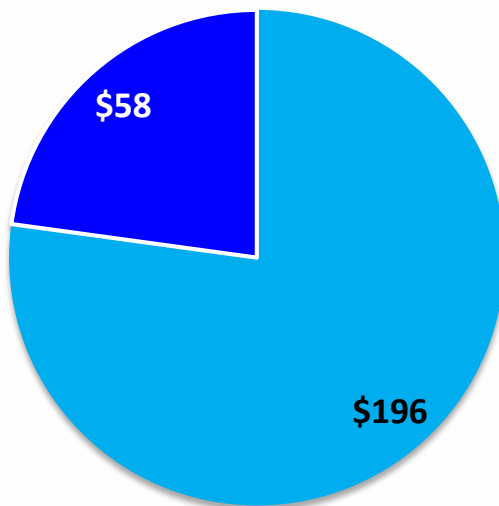


Funds Committed in Each Fiscal Year by Type of PFA

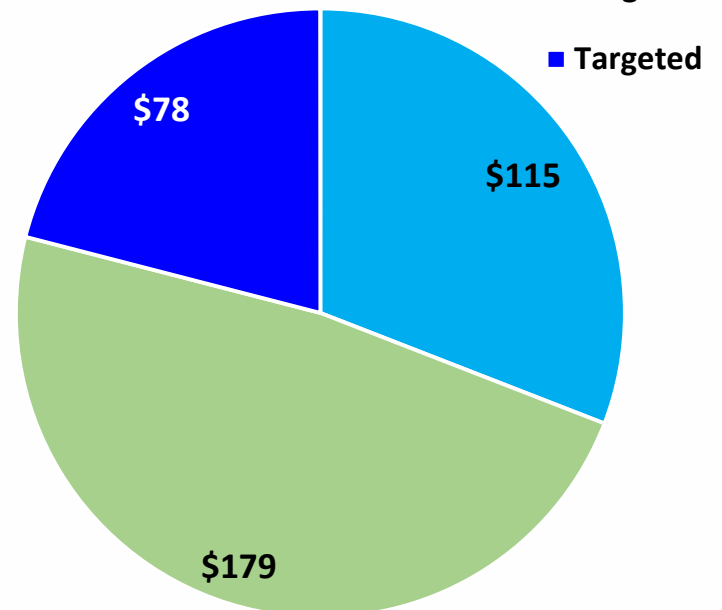
FY 2013
Total=\$294M



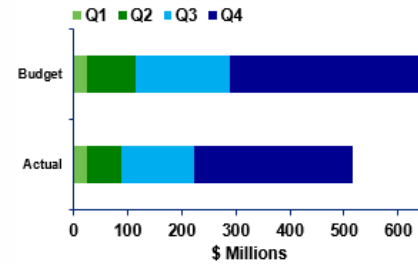
FY 2014
Total=\$254M



FY 2015
Total=\$372M

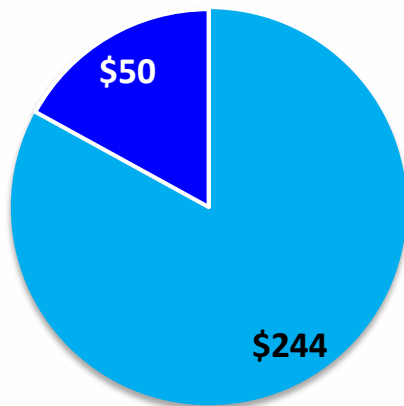


And the result is a substantial shift in the distribution of our portfolio

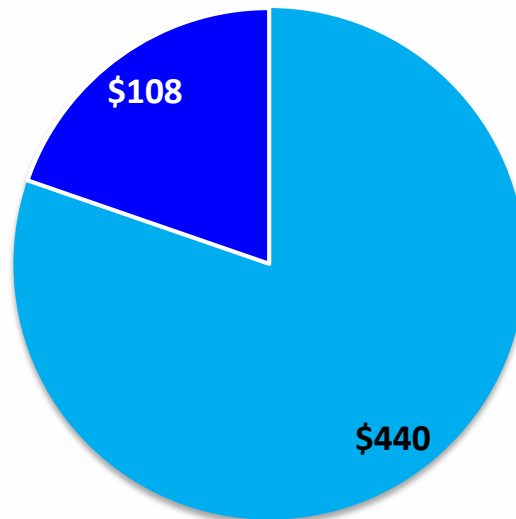


Funds Committed by Type of PFA, Cumulative Total by Fiscal Year

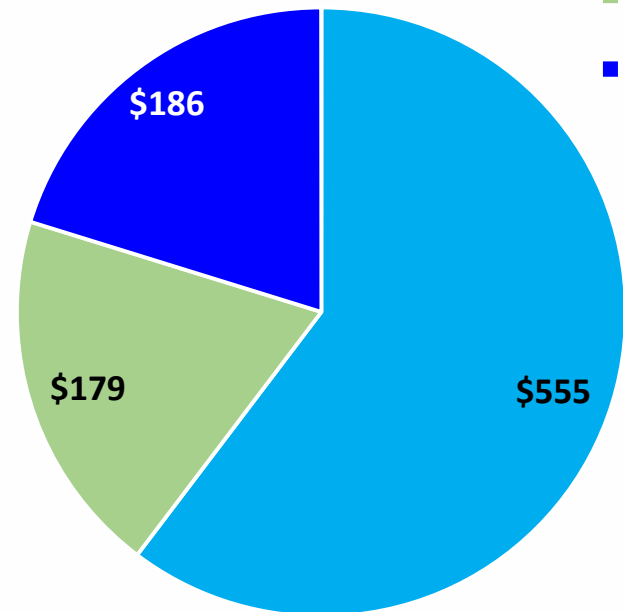
End of FY 2013
Total=\$294M



End of FY 2014
Total=\$548M



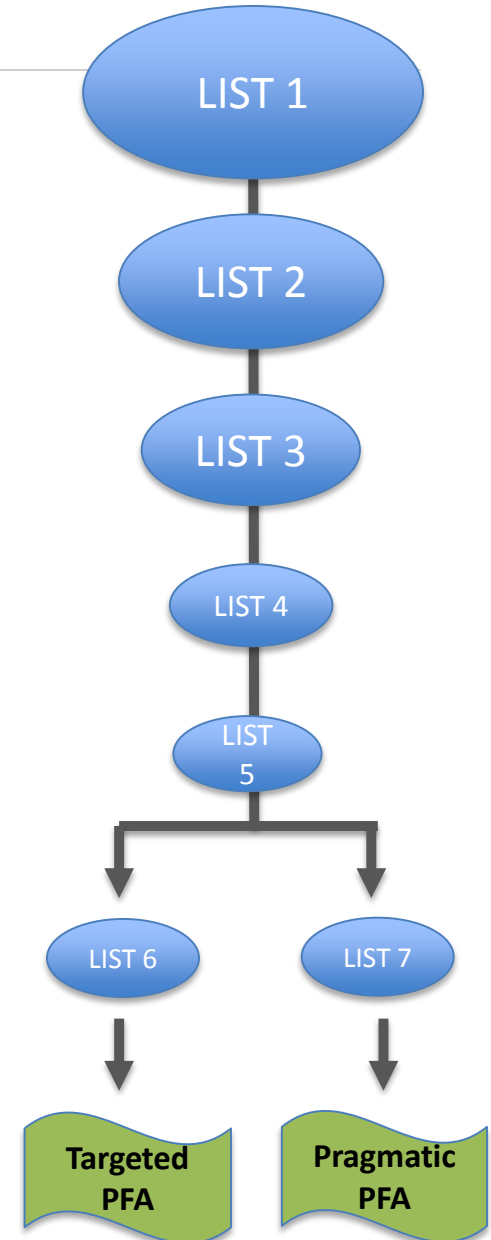
End of FY 2015
Total=\$920M



■ Broad
■ Pragmatic
■ Targeted



Topic Prioritization Pathway



Topic Prioritization Pathway: FY15 Q4 Snapshot

Under Consideration

SOC Endorsed for Refinement (List 5)

- Asthma
- Chronic Low Back Pain
- Diabetes
- Mental Health Integration
- Palliative Care
- Sickle Cell Disease

Reviewed by Advisory Panels (List 4)

- Antimicrobial Resistance
- Autism Spectrum Disorders- risk assessment
- NOACs for stroke prevention
- Care coordination
- Cognitive Impairment
- Communication
- Coronary Artery Disease- Statins
- Dementia
- Genetic Testing for Rare Disease
- Glaucoma
- Health IT & Evidence-Based Treatment
- High Cholesterol
- Implantable Cardiac Defibrillators
- Links btw Providers & Community
- Neck Pain
- Orthopedic Surgery
- Pneumonia
- Post-Acute Care Transitions
- Role of Spacers in Asthma
- Substance Abuse- Adolescents/Alcohol
- Weight Maintenance & Reduction

Approved Topics

For Targeted PFAs (List 6)

- Chronic Pain/Long Term Opioid Therapy
- Multiple Sclerosis
- NOACs for blood clots
- Treatment-Resistant Depression

For Pragmatic Studies PFAs (List 7)

- Autism Spectrum Disorders- behavioral analysis
- Cardiovascular Disease
- Caregiver Engagement & Support
- Carotid Artery Disease
- Dental Caries
- Diabetes
- End Stage Renal Disease
- Insurance Design
- Medication Management
- Migraine
- Musculoskeletal Pain
- Opioid Abuse Treatment
- Osteoarthritis
- Pelvic Floor Dysfunction
- Pre-term & Low Birth Weight
- Suicide Prevention
- Tobacco Use Prevention & Cessation
- Traumatic Brain Injury

Funded Topics

Funded via Targeted PFAs

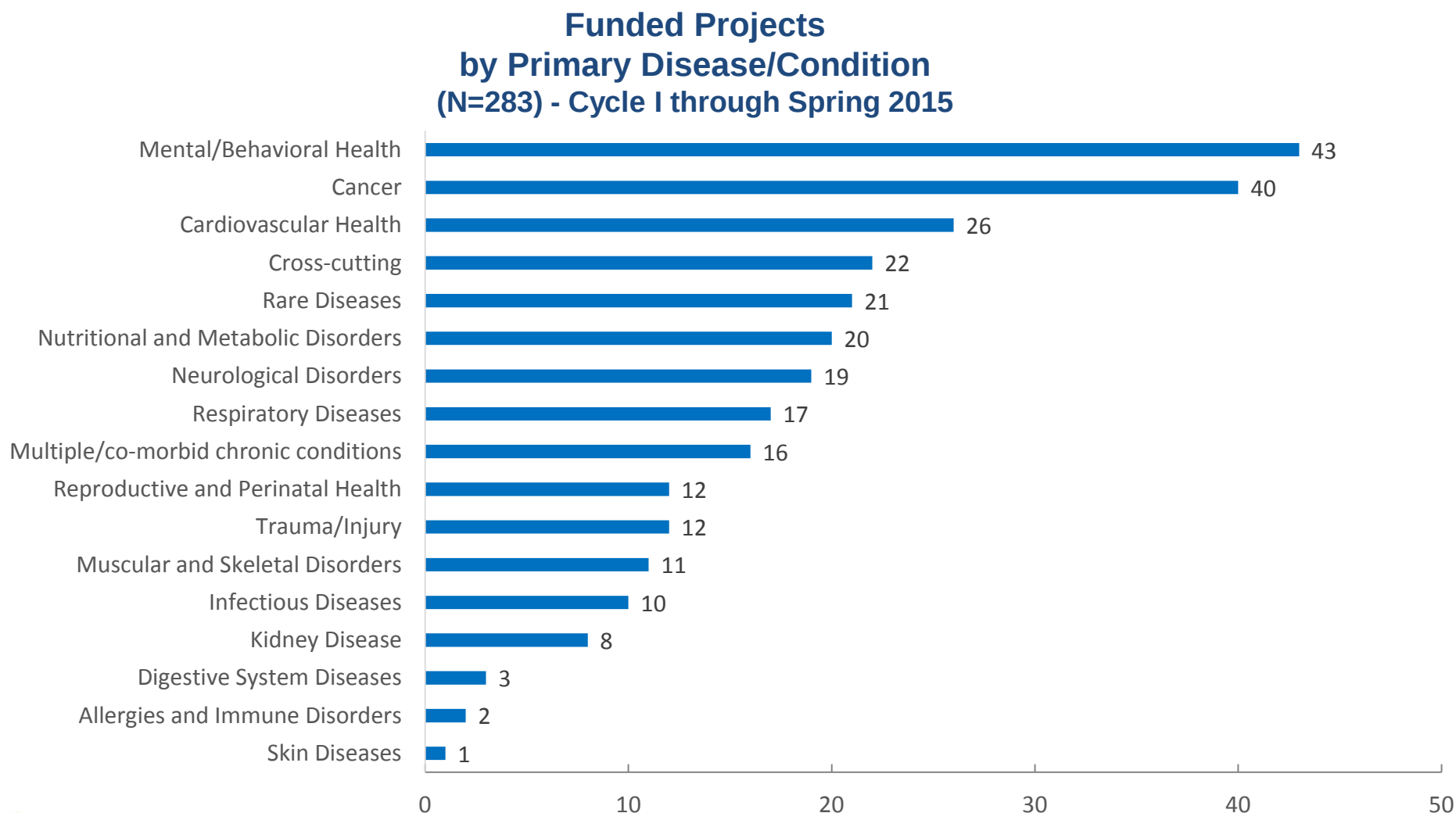
- CAD-Aspirin Dose
- Care Transitions
- Falls in Elderly
- Hepatitis C- New Therapies
- Hypertension
- Obesity in Diverse Populations
- Severe Asthma in African Americans & Hispanics
- Uterine Fibroids

Funded via Pragmatic PFAs

- Bipolar Disorder
- Breast Ductal Carcinoma in Situ
- Crohn's Disease- Biologics
- Chronic Back Pain
- Hip Fractures
- Mental Health & Primary Care
- Particle Beam Therapy
- Pulmonary Nodules & CT Surveillance



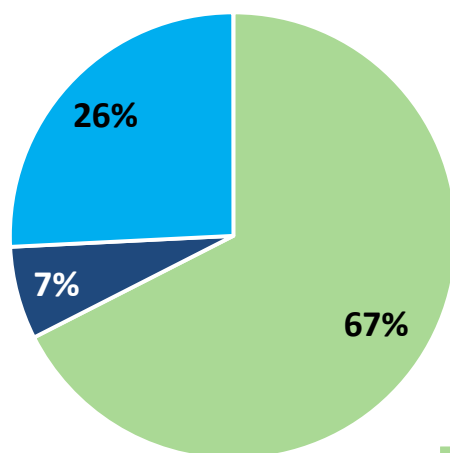
We have projects focusing on a wide range of conditions, with the largest concentrations in mental/behavior health and cancer



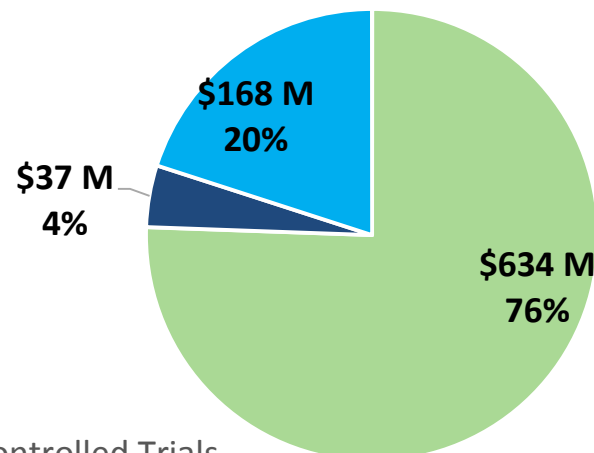
The majority of studies in our portfolio are clinical trials

Funded Portfolio by Study Design Cycle I through Spring 2015

Study Design by
Number of Awards
(N=283)



Study Design by
Award Amount (\$)
Total: \$838M

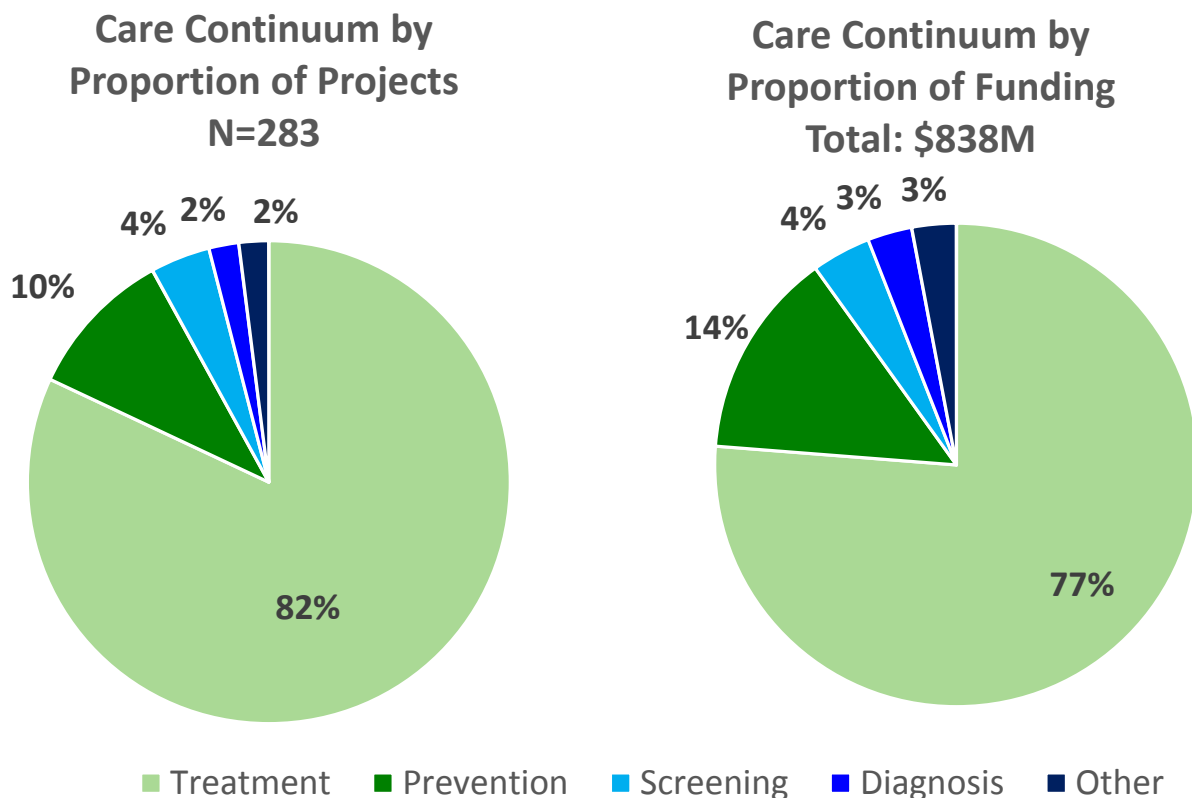


- Randomized Controlled Trials
- Secondary Data Analysis
- Observational (Prospective)



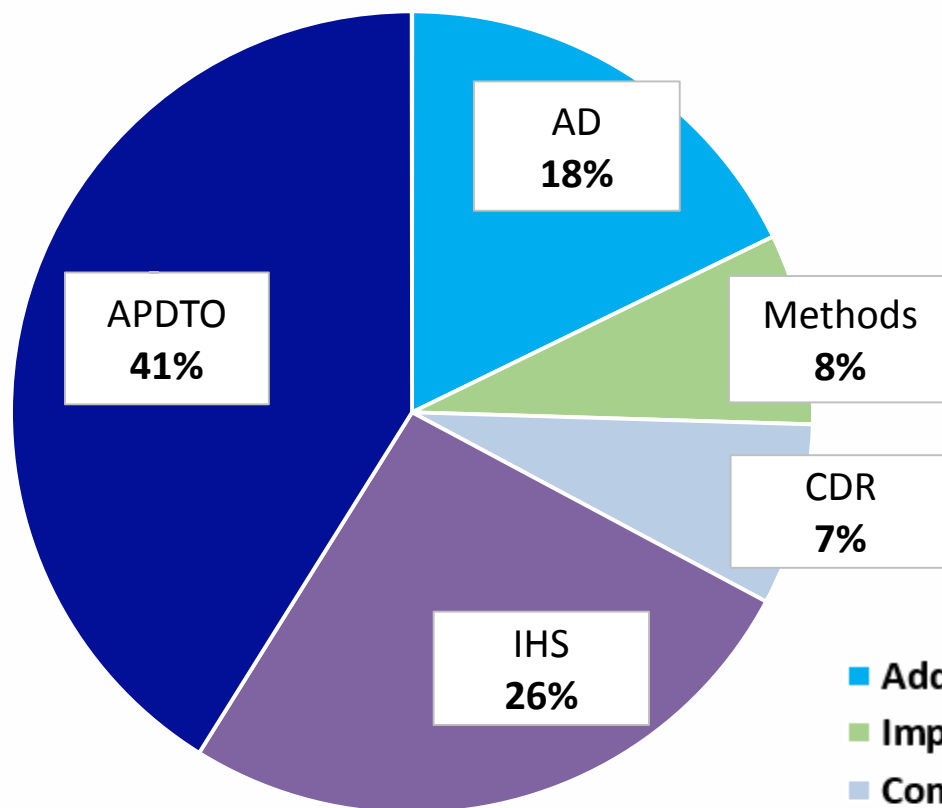
The majority of our studies focus on treatment and 18% of our funding is in screening and prevention studies

Funded Portfolio by Care Continuum Cycle 1 through Spring 2015

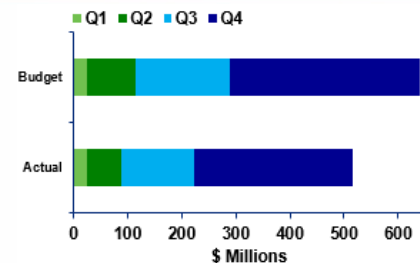


The majority of our research funding has gone to our APTDO and IHS portfolios

Total= 355 Projects, \$920M



- Addressing Disparities
- Improving Methods for Conducting PCOR
- Communication and Dissemination Research
- Improving Healthcare Systems
- Assessment of Prevention, Diagnosis, and Treatment Options



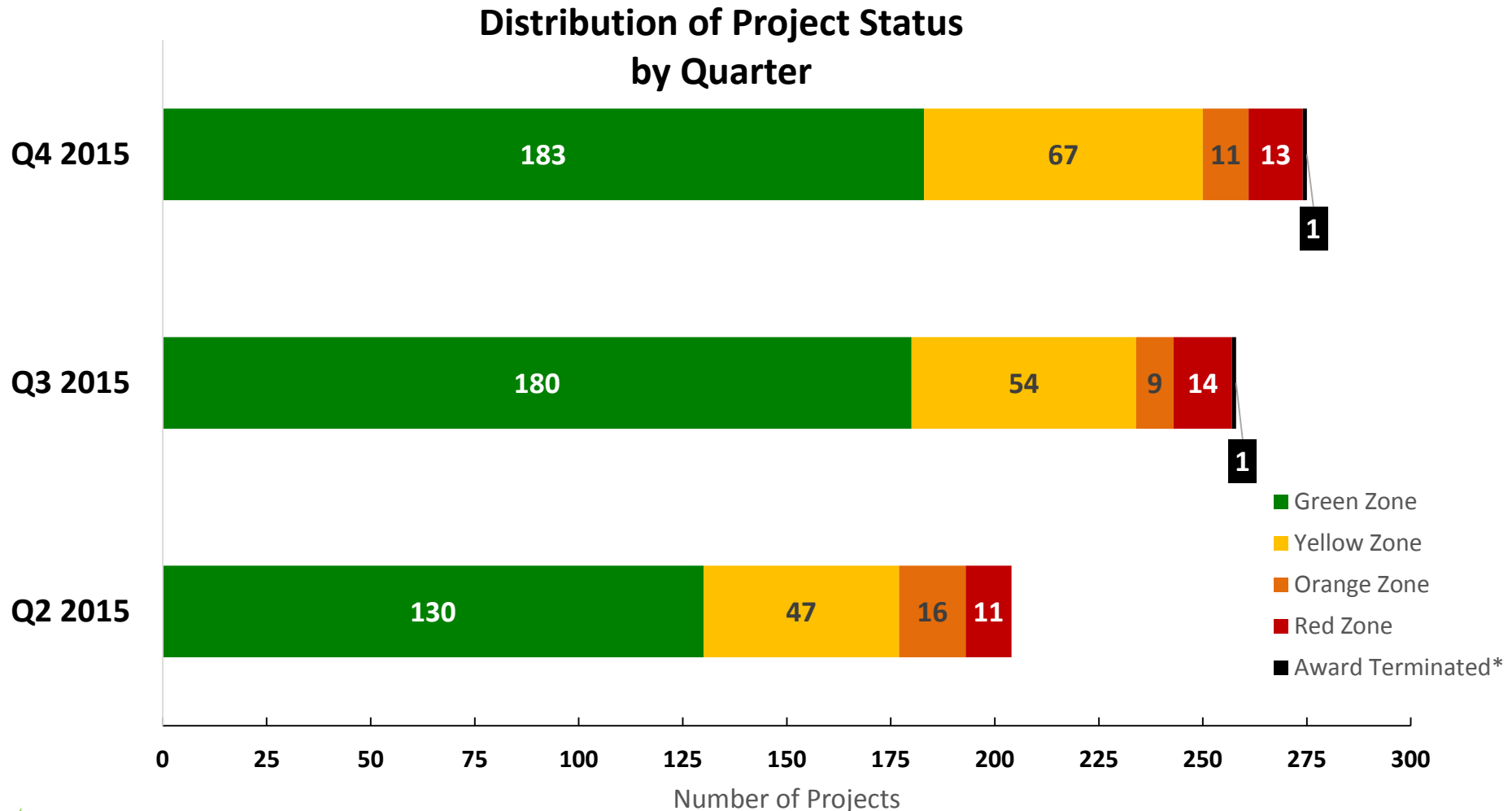
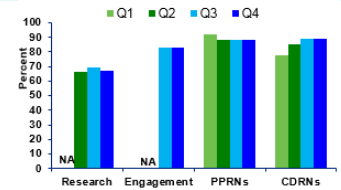
We actively monitor our projects, support them to be successful, and classify their progress as shown below

GREEN	YELLOW	ORANGE	RED
Project is On Track ≥ 75% of Recruitment -AND- ≥ 85% Milestones on Time -AND- PO has confidence in project.	Does not meet Green criteria -AND- <75% and ≥50% Recruitment -OR- <85% and ≥65% Milestones -OR- PO has some concerns about project.	Does not meet Yellow criteria -AND- <50% of Recruitment -OR- <65% and ≥50% Milestones -OR- PO has serious concerns; Modifications to the Milestone Schedule and/or project plan are likely required.	Does not meet Orange criteria -AND- <50% Recruitment, persistently -OR- <50% All Milestones -OR- PO has significant concerns; Major modifications to Milestone Schedule are required.
Next Steps			
Continue monitoring	Increased communication	Place project under review; Pursue Modifications	Draft Project Remediation Plan

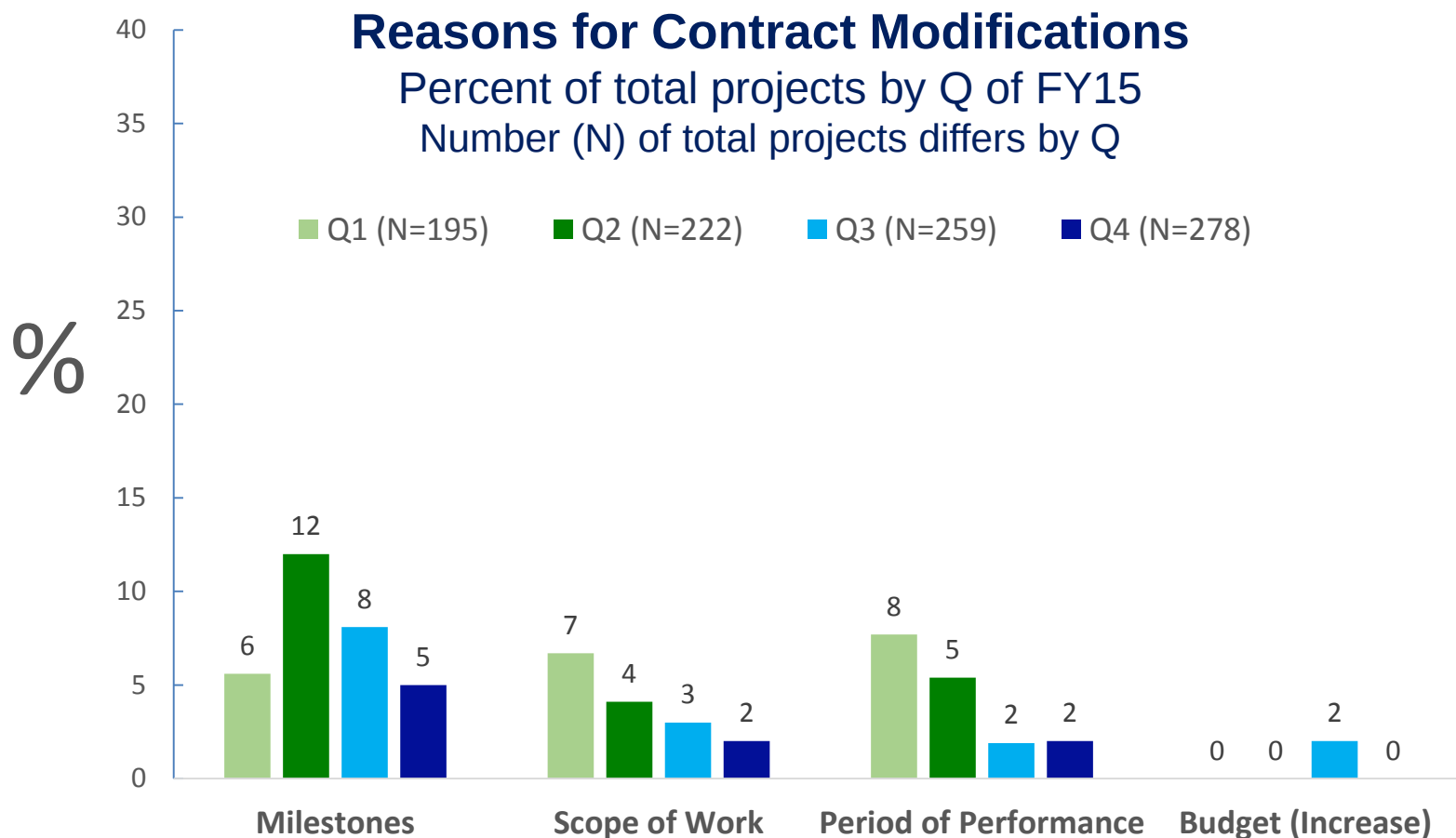
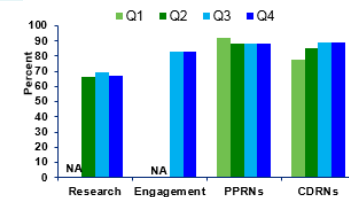
The “Percent of Projects on Track” shown on the Dashboard is the percent of projects in the green zone



The majority of our projects are on track and we are giving additional attention to those that are not



Some projects require contract modifications in order to be successfully completed

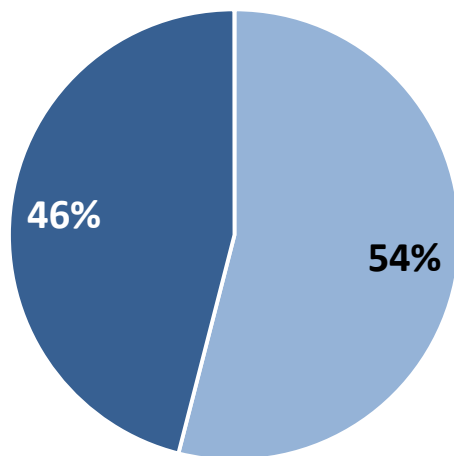


- Includes all research projects with at least one progress report due
- Does not include infrastructure or pilot projects

Almost half of our first completed cohort of studies (the Pilot Projects, average length 26 months) required an extension (5 months on average) and 88% were then completed within the contract period

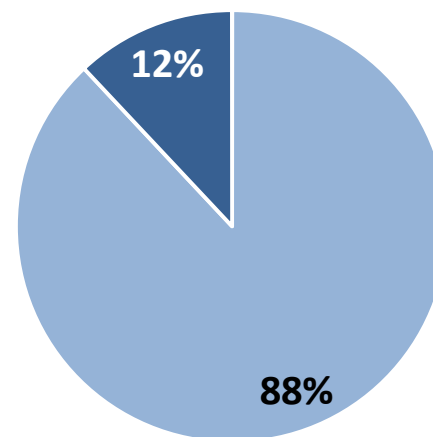
Our First Completed Cohort of Projects

PILOTS
Contract Extensions
(N=50)



- No Extension
- Required Contract Extension

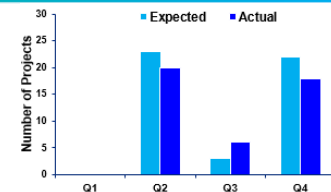
PILOTS
Completed Project within
Contract Period (N=50)



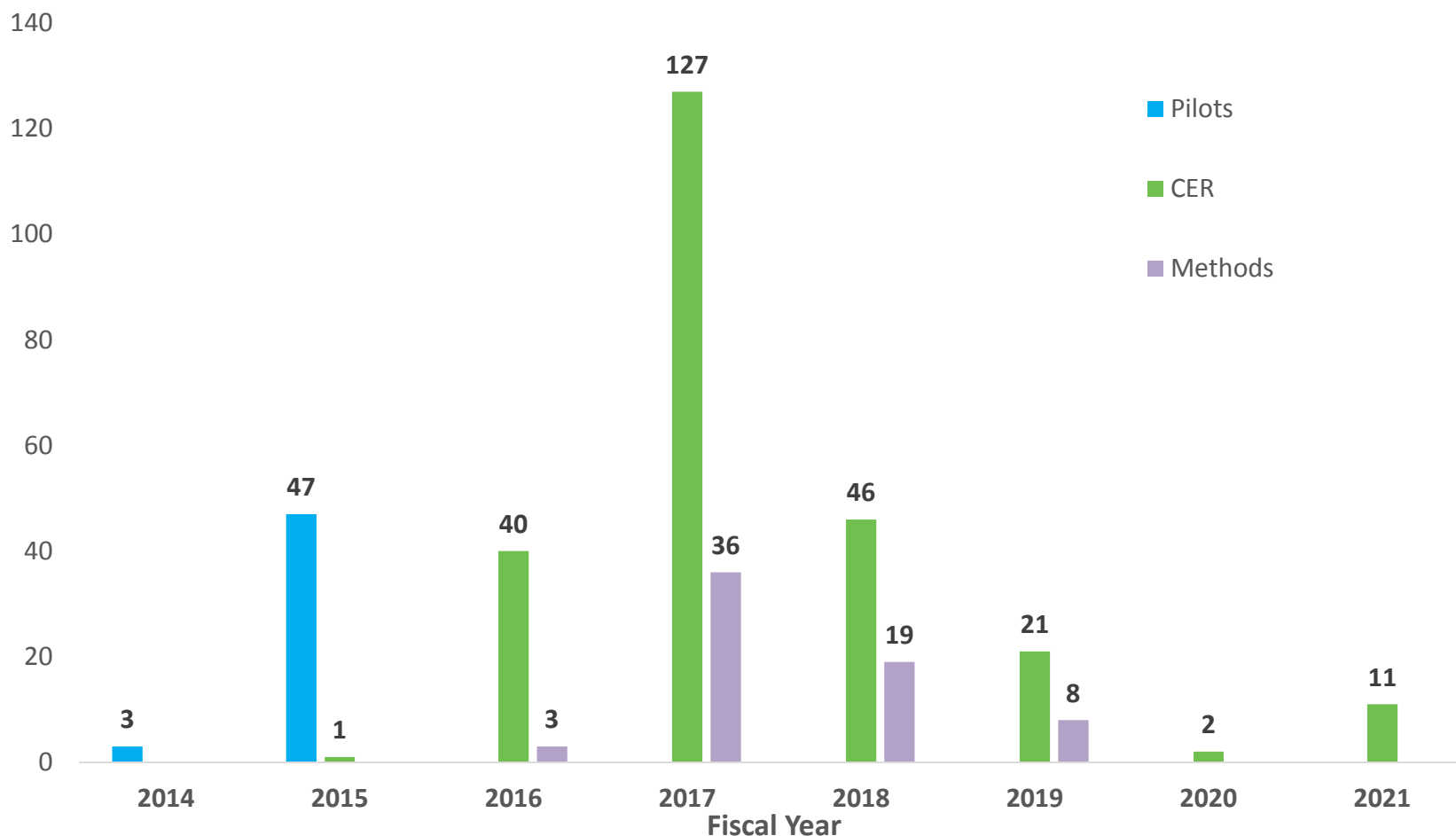
- Completed in Contract Period (includes modified contracts)
- Not Completed within Contract Period (Late)



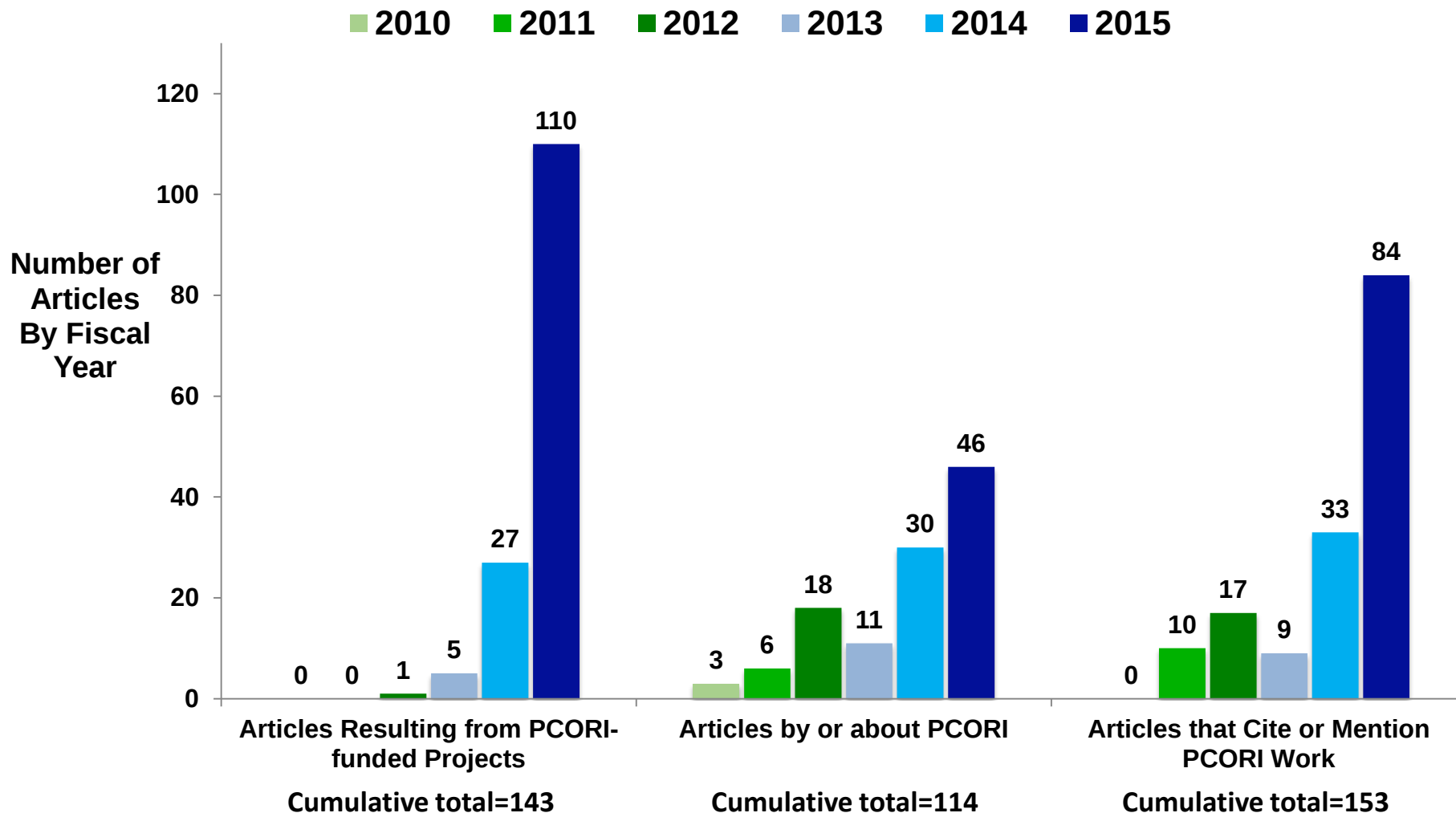
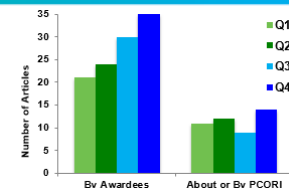
We currently expect the contracts for our first cohort of CER studies to end late in FY 2016



Expected Number of Contracts Ending by Fiscal Year



The number of journal articles concerning PCORI increased in FY2015



Three Published Protocols for CER Studies

Field CA, et al. Cultural adaptation of a brief motivational intervention for heavy drinking among Hispanics in a medical setting. BMC Public Health. 2015 Jul 30;15:724.

Culturally-adapted therapy vs non-adapted therapy for heavy drinking among Latino men

Taveras EM, et al. Connect for health: Design of a clinical-community childhood obesity intervention testing best practices of positive outliers. Contemp Clin Trials. 2015 Sep 28.

Contextually-tailored health coaching vs non-tailored coaching for weight loss in obese children ages 2-12 years

Williams QI, et al. Physical therapy vs. internet-based exercise training (PATH-IN) for patients with knee osteoarthritis: study protocol of a randomized controlled trial. BMC Musculoskelet Disord. 2015 Sep 28;16(1):264.

Standard physical therapy vs internet-based training for largely uninsured patients with knee osteoarthritis

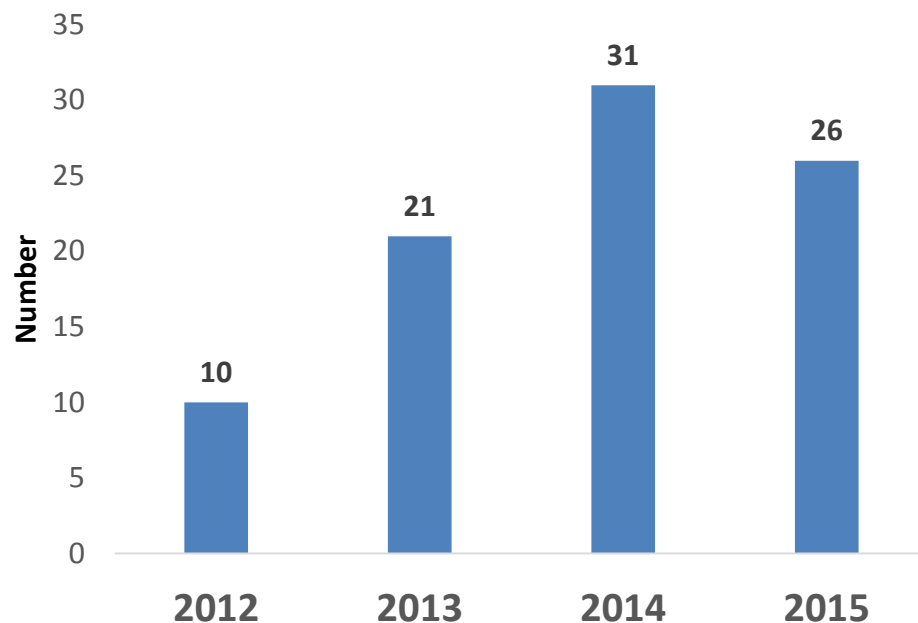


Measures of Uptake of Our Methodology Standards: We are tracking citations and other early indicators

Example – Citations, Page-views, and Downloads of:

Methodology Committee of the Patient-Centered Outcomes Research Institute (PCORI). **Methodological standards and patient-centeredness in comparative effectiveness research: the PCORI perspective.** *JAMA*. 2012 Apr 18;307(15):1636-40.

Citations of Methodology Standards
By Calendar Year



JAMA Statistics

Total Views	2,444 Pageviews
3,532	1,088 PDF Downloads



Measures of Uptake of Our Methodology Standards: In FY2016, we will be tracking PCORI-Funded CME/CE

Our CME/CE activity is designed to introduce PCORI's Methodology Standards, review the development and types of standards, and provide context and guidance for adherence relevant to the use and application of these standards in research and in responding to PCORI funding announcements

PCORI Methodology Standards Series with 6 Modules

Released Sept 30, 2015

Module 1

Basic Context, Role, and Development of Standards in CER

Module 2

Role of Standards in PCORI Funding Announcements & the Application Process

Module 3

Formulating Research Questions and Patient Centeredness

Module 4

Data Integrity, Preventing and Handling Missing Data, and Heterogeneity of Treatment Effects

Module 5

Data Registries, Data Networks, and Causal Inference

Module 6

Standards for Specific Study Designs- Adaptive and Bayesian Trial Designs and Diagnostic Tests



Measures of Uptake of Our Methodology Standards: Incorporation into academic curriculum – Example

Joel M. Gelfand, MD, MSCE

Center for Clinical Epidemiology & Biostatistics

Perelman School of Medicine

University of Pennsylvania

MSCE with Concentration in Patient-Centered Outcomes Research (PCOR)

- Students explore methods spanning qualitative research, clinical epidemiology, molecular epidemiology, and clinical trials
- Unifying theme is generation of data that helps patients, their care givers, providers, and health care delivery systems make informed health care decisions that improve outcomes that matter most to patients
- Students expected to pursue a thesis relevant to patient-centered outcomes research

EPID 624: Methods in Patient-Centered Outcomes and Effectiveness Research

Class	Topic
1	Overview of PCOR: PCORI Methodology Report
2	Formulating Research Questions, Identifying Evidence Gaps
3	Patient Centeredness: Stakeholder Engagement
4	Patient Centeredness: Methods to Increase Patient Enrollment
5	Patient Centeredness: Selection of Outcomes, PROs
6	Sensitivity Analyses
7	Missing Data
8	Data Registries & Automated Databases for CER
9	Pragmatic Trials & Bayesian Designs



Discussion Questions

- What is your view of our *performance in FY 2015 and our status* as of the end of FY 2015?
- What are your thoughts about our *funding commitments over the next four years*?
- Is our *approach to targeted topics* optimal?



PCORI Portfolio Portrait

Lori Frank, PhD

Program Director, Evaluation and Analysis

Heather Edwards, PhD, MPH, MBA

Program Officer, Strategic Portfolio Analysis

Vadim Gershteyn, MPH

Program Associate, Evaluation and Analysis

Rachel Witsaman, MPH, PMP

Senior Program Associate, Evaluation and Analysis



Agenda

- Describe the coding taxonomy development
- Present the new taxonomy structure
- Provide examples of portfolio views



Taxonomy Goals

- Support PCORI project portfolio reporting and information sharing
- Support understanding and review of contents of PCORI's funded project portfolio, applications, and stakeholder-submitted topics



Taxonomy Development

Use of existing taxonomies where appropriate

- Checked for term variations in the Unified Medical Language System (UMLS) Thesaurus
- Used primarily Medical Subject Headings (MeSH), especially for disease states
- Applied Census definitions to population categories
- Based outcomes categorization on Cochrane Consumers and Communication Review Group taxonomy
- Mapped to NIH Research, Condition, and Disease Categorization (RCDC)



Taxonomy Development

Hierarchical categories for content

- Theme (e.g., Population)
- Code (e.g., Age Groups)
- Sub-code (e.g., Adults, excluding elderly)

Building from

- Medical Subject Heading (MeSH)
- Unified Medical Language System (UMLS) Concept Unique Identifier (CUI)



PCORI Portfolio Taxonomy

Addresses PCORI's unique information needs

- Highlights populations of interest (caregivers, Medicaid, rare diseases, etc.)
- Classifies comparators
- Lists Level and Reporter associated with each outcome
- Codes for Stakeholder Engagement in research



STUDY

Condition

- Allergies & Immune
- Birth & Developmental
- Blood
- Cancer
- Cardio-vascular
- Dental Care
- Ear, Nose, Throat
- Eye
- Functional Limitations
- Gastro-intestinal
- Genetic/ Rare
- Infectious
- Kidney
- Liver
- ...Others not listed

Study Population

- Age
- Race
- Ethnicity
- Sex
- Sexual Orientation
- Income
- Population Density
- Disability
- Educational Attainment
- Employment Status
- Provider Populations
- Literacy
- Language
- Oversample
- Special Populations

Care Continuum

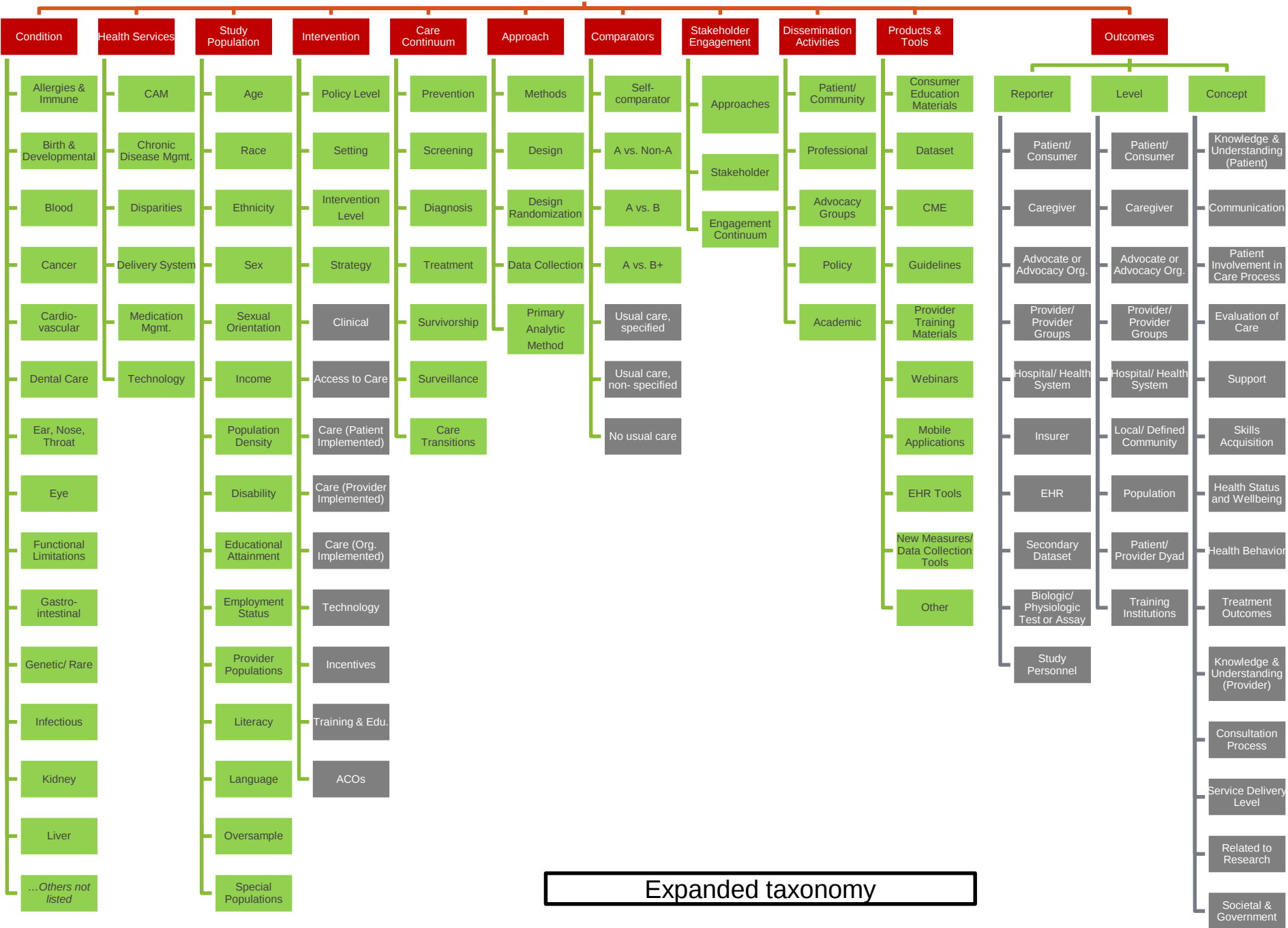
- Prevention
- Screening
- Diagnosis
- Treatment
- Survivorship
- Surveillance
- Care Transitions

Comparators

- Self-comparator
- A vs. Non-A
- A vs. B
- A vs. B+
- Usual care, specified
- Usual care, non- specified
- No usual care

Previous coding categories

STUDY



STUDY

Approach

Methods

Design

Design
Randomization

Data Collection

Primary Analytic
Method



STUDY

Approach

Methods

Design

Design
Randomization

Data Collection

Primary Analytic
Method



STUDY

Approach

Methods

Design

Design
Randomization

Data Collection

Primary Analytic
Method

Observational

Interventional



STUDY

Approach

Methods

Design

Design
Randomization

Data Collection

Primary Analytic
Method

Observational

Case control

Cross
sectional

Prospective
cohort

Retrospective
cohort

Interventional

Bayesian

Cluster
randomized

Interrupted
time series

Large sample

Multi-level
nested

Pilot

Pragmatic

Pre-post

Quasi-
experimental

RCT

Stepped
wedge



Views from the PCORI Portfolio

- Study populations
- Study approach
- Interventions
- Comparators
- Stakeholder engagement



Views from the PCORI Portfolio

- PCORI funded a total of 252 projects through April 2015 (Fall 2014 cycle):

Program	Projects
APDTO	100
IHS	62
AD	55
CDR	35

- Research plans for these 252 projects were coded using the taxonomy
- Coding was inclusive - all applicable categories and subcategories were marked for each project



Taxonomy Structure: Study Population

Age

Race

Ethnicity

Sex

Sexual
Orientation

Income

Population
Density

Disability

Educational
Attainment

Employment
Status

Provider
Populations

Literacy

Language

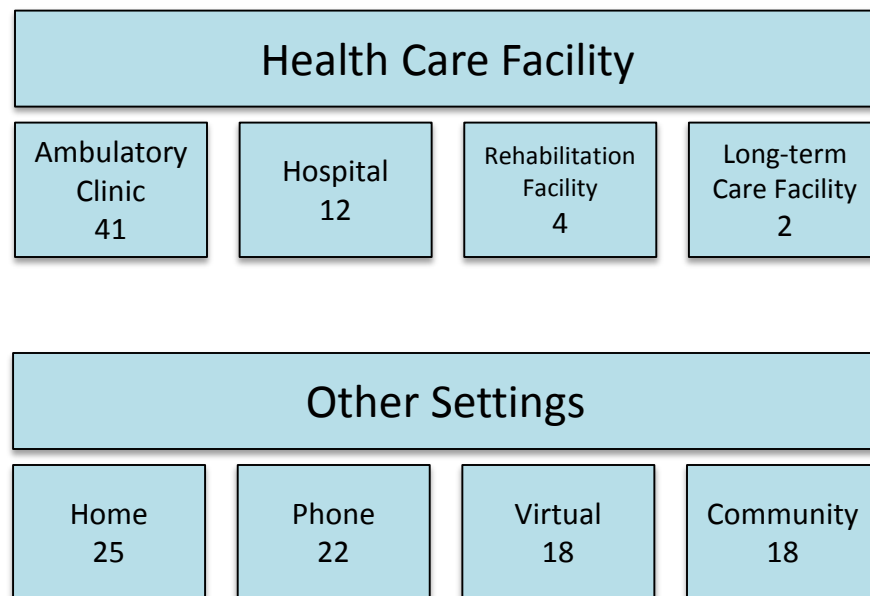
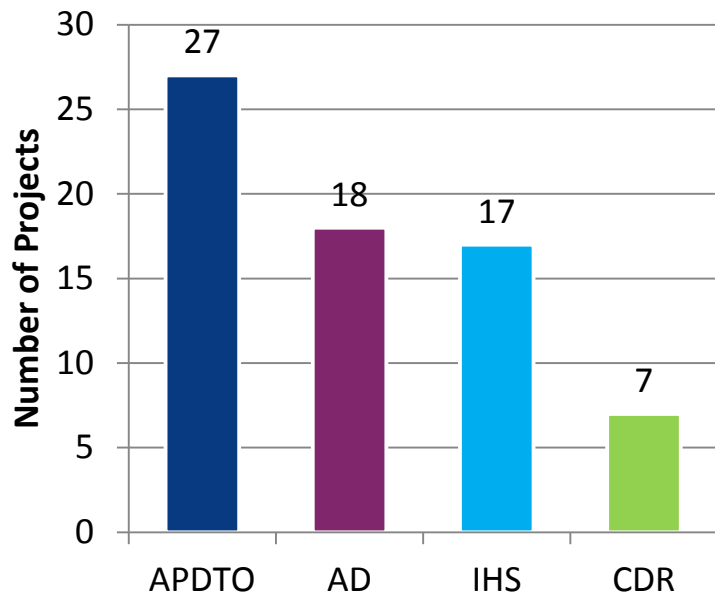
Oversample

Special
Populations



Study Populations: Older Adults

Sixty-nine projects (27%) focus on older adult populations

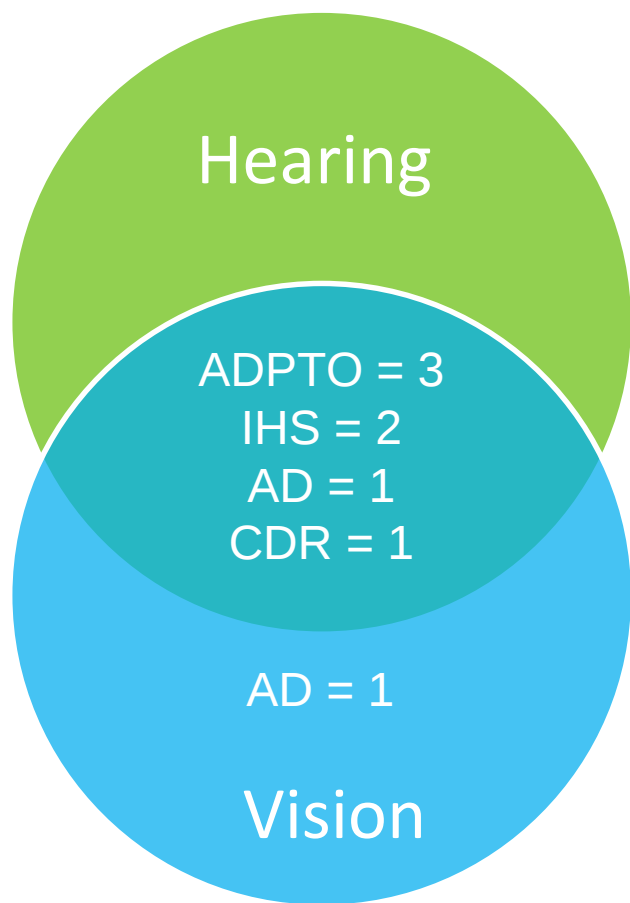


Not applicable: 3



Study Populations: Vision/Hearing Loss

Eight projects (3%) include populations with vision or hearing loss



Intervention Strategy

Projects

Provider or Organization-
Implemented Care Interventions

5

Access Interventions

3

Training and Education

3

Technology Interventions

2

Accountable Care Organizations

1

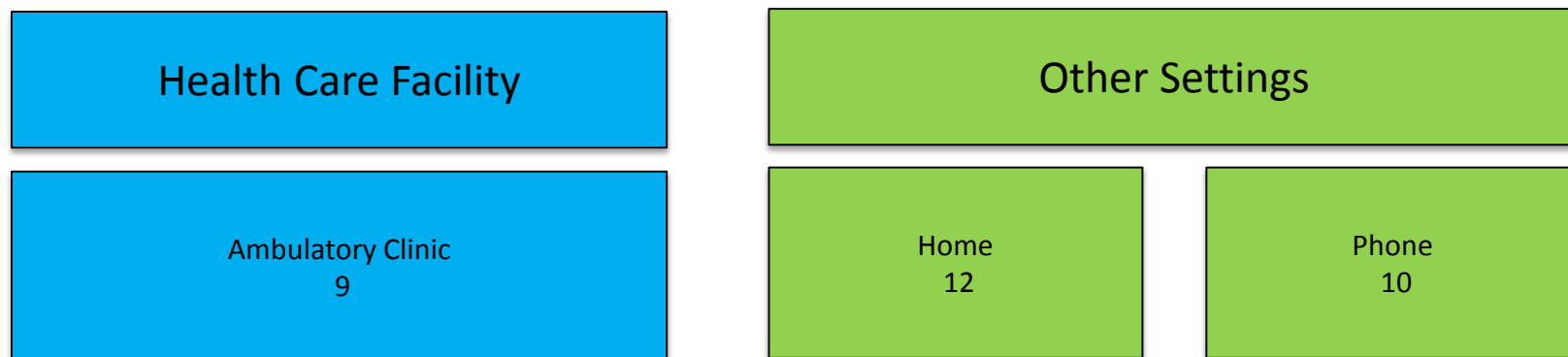
Clinical Interventions

1



Study Populations: Community Health Workers

Fifteen projects (6%) have a focus on community health workers



Note: Continuum not applicable to one project



Taxonomy Structure: Approach

Methods

Design

Design
Randomization

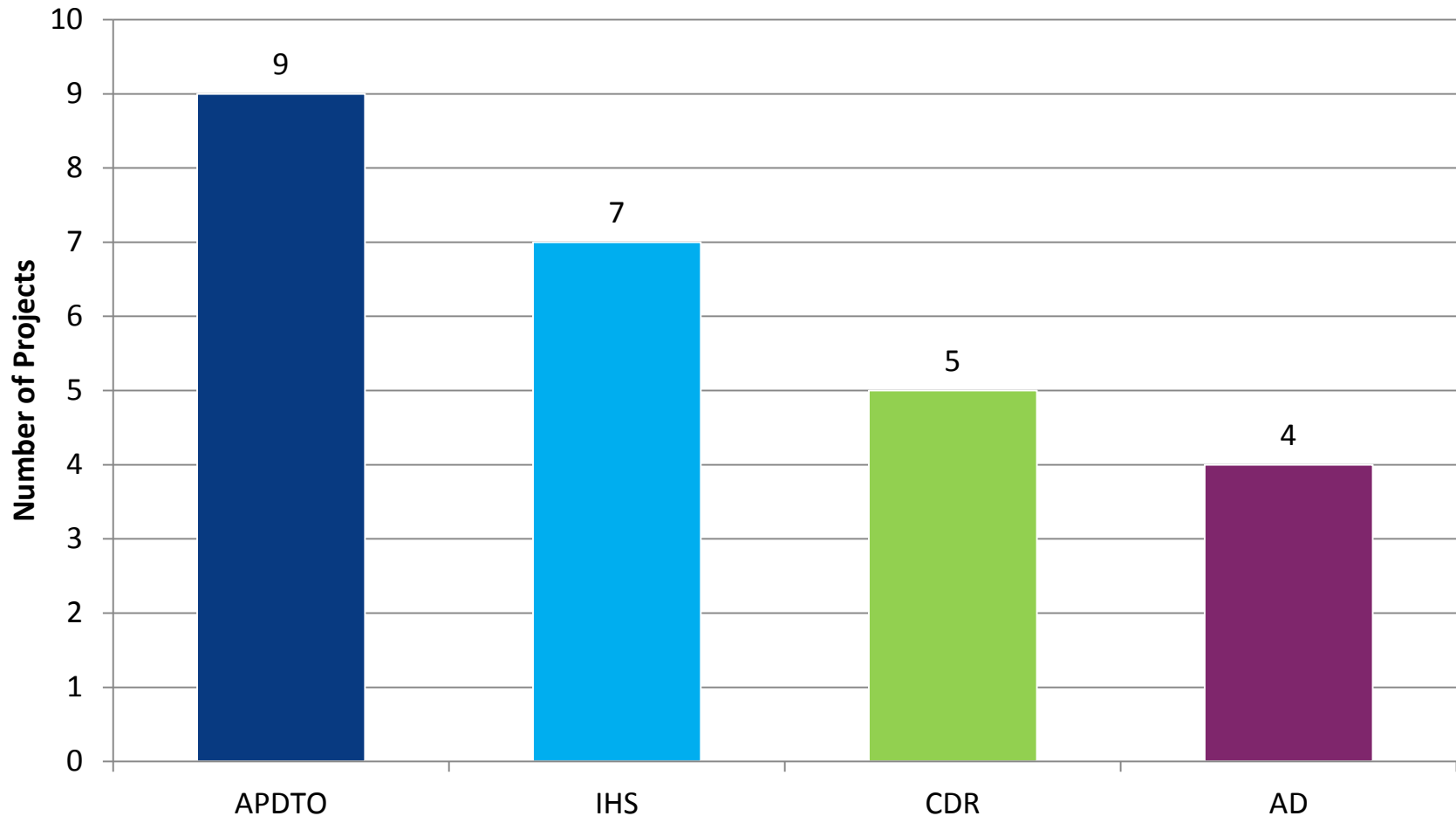
Data Collection

Primary
Analytic Method



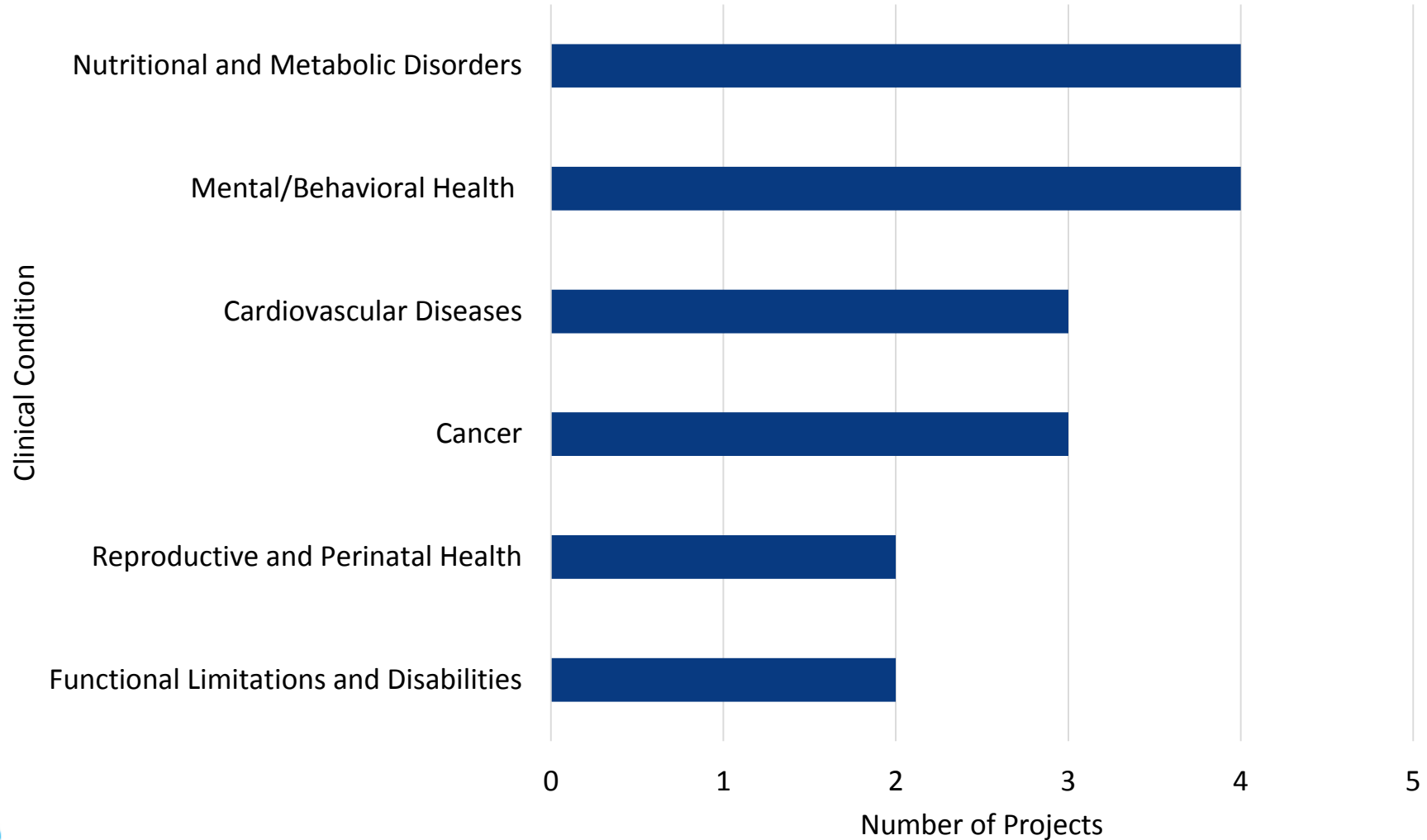
Approach: Design

Twenty-five projects (10%) use cluster randomized designs



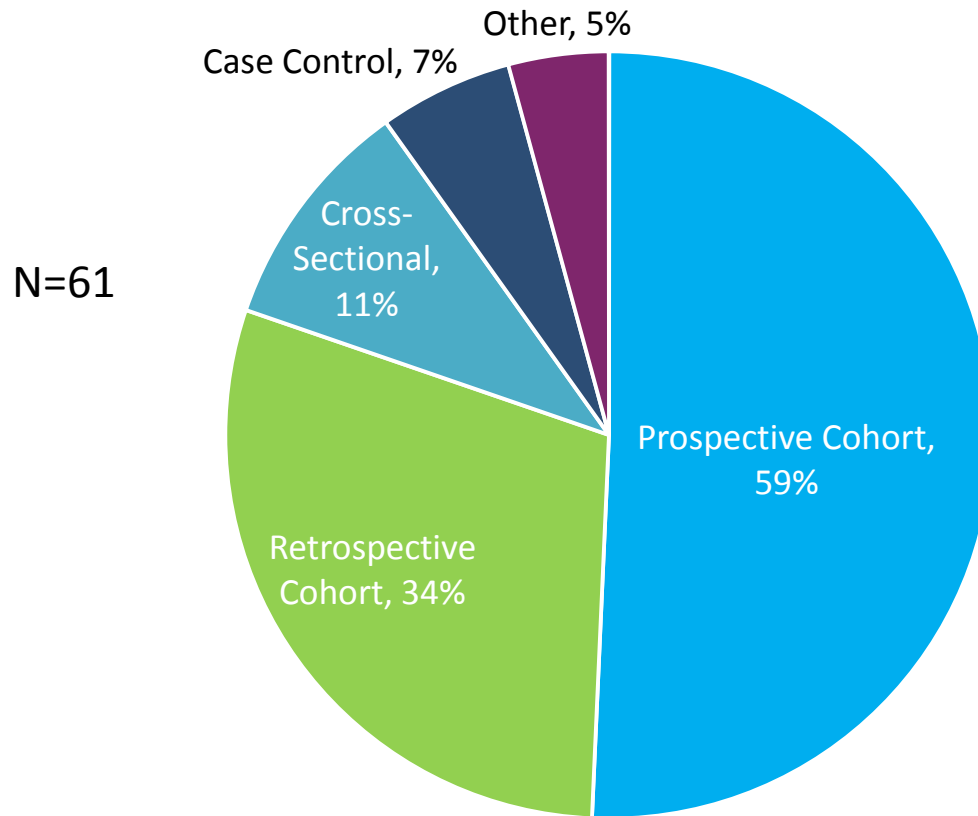
Approach: Design

Cluster Randomized Projects (N = 25) by Clinical Condition

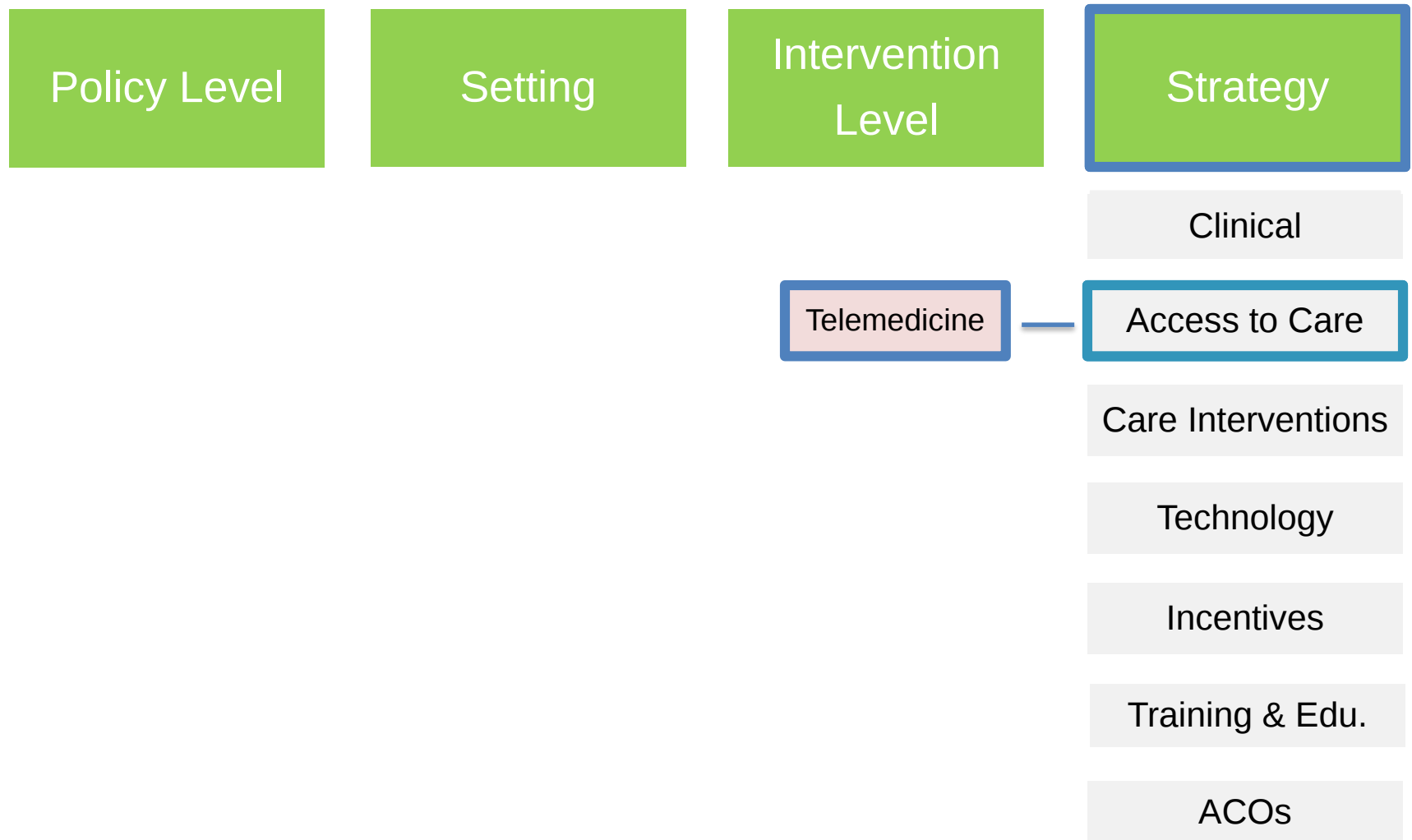


Approach: Design

Sixty-one (24%) projects use observational study designs



Taxonomy Structure: Intervention



Taxonomy Structure: Comparators

A vs. B

A vs. B+

Usual care



Comparators: Examples from the PCORI Portfolio

2 arms

Anti-TNF Monotherapy versus Combination Therapy with Low Dose Methotrexate in Pediatric Crohn's Disease PI: M. Kappelman, PhD, University of North Carolina

Compares the effectiveness of low-dose oral methotrexate in combination with anti-TNF biological therapy or anti-TNF alone on PROs and adverse effects for children with moderate-to-severe Crohn's disease.

3 arms

Comparative Effectiveness of CARRA Treatment Strategies for Polyarticular Juvenile Idiopathic Arthritis. PI: Y. Kimura, MD, Hackensack University Medical Center

Compares three treatment strategies for juvenile idiopathic arthritis: 1) Step-Up: disease modifying anti-rheumatic drug (DMARD) monotherapy + biologic medication if needed; 2) Early Combination: DMARD + biologic medication at treatment onset; and 3) Biologic First: biologic medication monotherapy at treatment onset.

4 arms

Randomized Trial to Increase Adherence to Cervical Cancer Screening Guidelines for Young Women PI: A. Moscicki, MD, University of California, San Francisco

Compares the effectiveness of 1) provider-based feedback intervention (PBF) , 2) mHealth application plus PBF, and 3) patient-centered approach intervention plus PBF on adherence to cervical cancer screening, compared to a control arm.

Taxonomy Structure: Outcomes

Reporter

- Patient/ Consumer
- Caregiver
- Advocate or Advocacy Org
- Provider/Provider Groups
- Hospital/Health System
- Insurer
- EHR
- Secondary Dataset
- Biologic/ Physiologic Test or Assay
- Study Personnel

Level

- Patient/ Consumer
- Caregiver
- Advocate or Advocacy Org
- Provider/Provider Groups
- Hospital/Health System
- Locally/Defined Community
- Population
- Patient/Provider Dyad
- Training Institution

Concept

- Knowledge & Understanding (Patient)
- Communication
- Patient Involvement in Care Process
- Evaluation of Care
- Support
- Skills Acquisition
- Health Status and Wellbeing
- Health Behavior
- Treatment Options
- Knowledge and Understanding (provider)
- Consultation Process
- Service Delivery Level
- Related to Research
- Societal and Government



Taxonomy Structure: Stakeholder Engagement

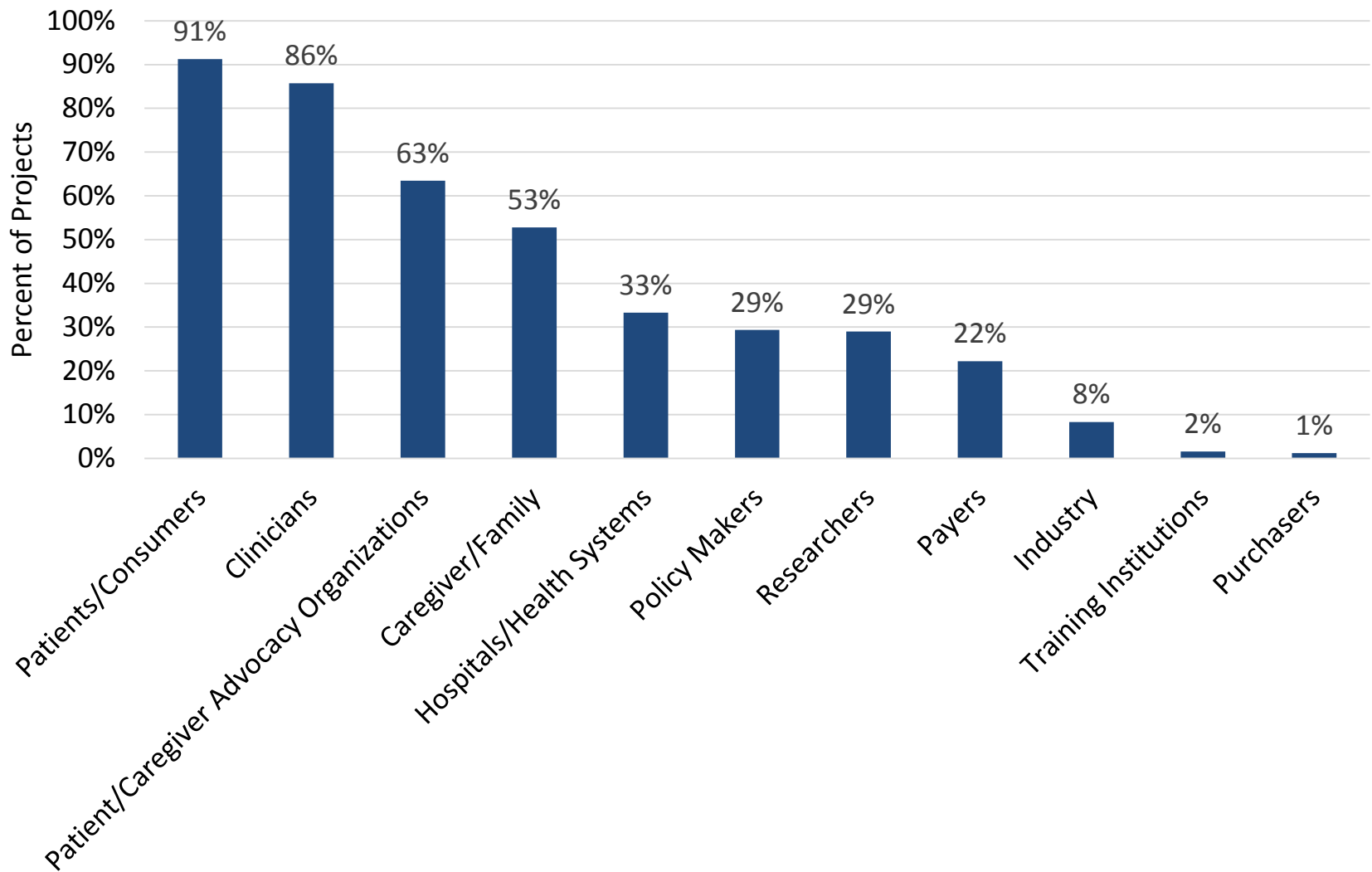
Approaches

Stakeholder

Engagement
Continuum



Engagement: Stakeholder Types



Elizabeth Harrison

1978-2014



Acknowledgements

PCORI:

Mary Jon Barrineau

Lauren Fayish

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Amy Grossman

Mary Kay Margolis

Krista Woodward

Ohio State University:

Ann Scheck McAlearney

Timothy Huerta

Alison Aldrich

Barbara Barash

Jennifer Hefner

Tyler Johnson

Cynthia Sieck

Lindsey Sova



Appendix: Detailed Taxonomy Categories



Taxonomy Structure: Conditions

Allergies & Immune	Birth & Developmental	Blood	Cancer	Cardio-vascular	Dental Care	Ear, Nose, Throat
Eye	Functional Limitations	Gastro-intestinal	Genetic/ Rare	Infectious	Kidney	Liver
Mental/ Behavioral Health	Multiple/ Comorbid Chronic Conditions	Muscular and Skeletal	Neuro-logical	Nutritional and Metabolic	Rare Disease	Reproductive and Perinatal
Respiratory	Skin	Systemic	Toxin	Trauma/ Injury	Urinary	Wellness



Taxonomy Structure: Health Services

CAM

Chronic
Disease
Management

Disparities

Delivery
System

Medication
Management

Technology



Taxonomy Structure: Study Population

Age

Race

Ethnicity

Sex

Sexual
Orientation

Income

Population
Density

Disability

Educational
Attainment

Employment
Status

Provider
Populations

Literacy

Language

Oversample

Special
Populations



Taxonomy Structure: Policy Intervention

Policy Level	Setting	Intervention Level	Strategy
			Clinical
			Access to Care
			Care Interventions
			Technology
			Incentives
			Training & Edu.
			ACOs



Taxonomy Structure: Care Continuum

Prevention

Screening

Diagnosis

Treatment

Survivorship

Surveillance

Care
Transitions



Taxonomy Structure: Approach

Methods

Design

Design
Randomization

Data Collection

Primary
Analytic Method



Taxonomy Structure: Comparators

Self-
comparator

A vs. Non-A

A vs. B

A vs. B+

Usual care,
specified

Usual care,
non- specified

No usual care



Taxonomy Structure: Stakeholder Engagement

Approaches

Stakeholder

Engagement
Continuum



Taxonomy Structure: Dissemination Activities

Patient/
Community

Professional

Advocacy
Groups

Policy

Academic



Taxonomy Structure: Products & Tools

Consumer
Education
Materials

Dataset

CME

Guidelines

Provider
Training
Materials

Webinars

Mobile
Applications

EHR Tools

New Measures/
Data Collection
Tools

Other



Break

We will return at 1:15 pm ET



Join the conversation on Twitter via
#PCORI



Stakeholder Perspective Panel: Patients

Gail Hunt

Moderator, Board Member

Marc Boutin, JD

CEO, National Health Council

Donna Cryer, JD

President & CEO, Global Liver Institute

Celeste Castillo Lee

Institute for Patient and Family-Centered Care



Methodology Committee Update

Robin Newhouse, PhD, RN

Chair, PCORI Methodology Committee

Steve Goodman, MD, MHS, PhD

Vice-chair, PCORI Methodology Committee



Methodology Committee Members

- Robin Newhouse, Chair
- Steven Goodman, Vice Chair
- Naomi Aronson
- Ethan Basch
- David Flum
- Cindy Girman
- Mark Helfand
- Bob Kaplan
- Michael Lauer
- David Meltzer
- Brian Mittman
- Sally Morton
- Neil Powe
- Sebastian Schneeweiss
- Mary Tinetti
- Adam Wilcox
- Clyde Yancy



Session Topics and Objectives

What are we going to cover today?

Methodology Standards Revisions

- Review process
- Overview of revisions

New Methodology Standards: Designs Using Clusters

- Review process
- Overview of the standards

Next Steps

- For both revised and new Methodology Standards

Board Vote

- Vote to approve release of revised and new Methodology Standards for public comment



Methodology Standards Review: Review Process

1. Convened workgroups of MC members and PCORI staff to review each category of the original standards and propose possible revisions
2. The proposed revisions were shared with the MC at biweekly meetings, sometimes through repeated iterations
3. The MC made further revisions at the October 29th in-person meeting and held a vote to approve all proposed changes
4. Standards needing further revisions after October 29th were revised and approved by the MC on November 9th



Methodology Standards Review: Overview

The results of the review of the existing 47 Methodology Standards were:

- 25 Standards revised
- 12 Standards combined in groups of two or three to form 6 revised Standards
- 1 Standard deleted
- 9 Standards unchanged
- 3 new Standards added to the existing Standards categories
- 5 new Designs Using Clusters Standards

General rationales for the revisions included:

- To streamline and clarify language
- To ensure alignment of the Standards and other PCORI guidance
- To enhance wording to reflect advances in methodology
- To synchronize concepts that are addressed in more than one Standard



Example of a New Standard that Resulted from the Standards Review Process

CI-1: CI-Model: Specify the causal model underlying the research question

Researchers should describe the causal model relevant to the research question, which should be informed by the PICOTS framework: populations, interventions, comparators, outcomes, timing, and settings. The causal model represents the key variables; the known or hypothesized relationships among them, including the potential mechanisms of effect; and the conditions under which the hypotheses are to be tested.

Researchers should use the causal model to determine whether and how the study can handle bias and confounding and the extent to which valid estimates of the effects of an intervention can be generated based on the particular data source, hypothesis, and study design.



New Standards on Designs Using Clusters: Development Process

1. PCORI staff developed an early draft of standards, based on the scientific literature in this area
2. A group of experts gave feedback and developed a second draft of the standards
3. These proposed Standards were presented to the MC
4. Additional expert feedback was solicited for further clarification and editing
5. The MC made further revisions at the October 29th in-person meeting and voted to approve the final draft



New Designs Using Clusters Standards: Overview

RC-1: Specify whether the study objectives, the interventions, and the primary outcomes pertain to the cluster level or individual level

RC-2: Justify the choice of cluster randomization

RC-3: Power and sample size estimates must use appropriate methods to account for the dependence of observations within clusters, and the degrees of freedom available at the cluster level

RC-4: Data analyses must account for the dependence of observations within clusters regardless of its magnitude

RC-5: Because cluster randomization trials often involve a limited number of groups or clusters, stratified randomization is recommended



Next Steps

- Seek Board approval to make the 48 revised, new and unchanged Methodology Standards available for public comment
- Read, process, and categorize public comments
- If indicated, revise the Methodology Standards based on public comments
- Revise the Methodology Report based on revisions and additions to the Standards
- Seek Board approval to publish the updated Standards and the accompanying Methodology Report
- Expected timeline: January through June 2016



Board Vote

Call for a Motion to:

- **Approve** release for public comment of the proposed new and revised Methodology Standards

Call for the Motion to Be Seconded:

- **Second** the Motion
 - If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion

Voice Vote:

- Vote to **Approve** the **Final** Motion
- Ask for votes in favor, opposed, and abstentions



Other MC Initiatives & Workgroups

- PCORI Network Research Methods Work Group
 - Data Quality Expert Meeting on December 10th
- Evaluating Multi-component, Multi-level Interventions
 - New Standards on Complex Interventions
- Patient-Centered Measurement Work group
- Usual Care Work group
- Methodology Standards dissemination activities
 - Methodology Standards Academic Curriculum
 - Baylor College of Medicine CME/CE Initiative



Revised Selection Committee Charter

Christine Goertz, DC, PhD

Chair, Science Oversight Committee

Chair, Standing Selection Committee



Background

- Charter approved by Board in July 2014 has provided sound framework
- Over time, recognition that Charter should be updated:
 - Retain core principles
 - Make more flexible to respond to changing needs and cycles
 - Reflect responsibilities more clearly



Unchanged Core Principles

- A Standing Selection Committee is appointed; additional Special Selection Committees may be appointed
- Selection Committees appointed by Board Chairperson, in consultation with Chair of Science Oversight Committee (SOC) and Executive Director
- Selection Committees composed of Board members and Methodology Committee members



Summary of Revisions

- Selection Committee framework more flexible
 - No longer must have exactly 6 members
 - Must have at least 3 members
 - Methodology Committee members may be appointed; Board members are a majority
 - SOC Chair no longer required to serve on Standing Selection Committee
- Responsibilities section clarified
 - Confirms that Selection Committee considers several factors, including merit review panels, scientific program staff recommendations, programmatic fit, portfolio balance, duplication of funded research, and methodological concerns



Board Vote

Call for a Motion to:

- **Approve** the revised Selection Committee Charter

Call for the Motion to Be Seconded:

- **Second** the Motion
 - If further discussion, may propose an **Amendment** to the Motion or an **Alternative Motion**

Voice Vote:

- Vote to **Approve** the **Final** Motion
- Ask for votes in favor, opposed, and abstentions



Break

We will return at 3:30 pm ET



**Join the conversation on Twitter via
#PCORI**



Evaluation Update: Merit Review Score Analysis

Laura Forsythe, PhD, MPH

Associate Director, Evaluation and Analysis

Lori Frank, PhD

Program Director, Evaluation and Analysis



Agenda

- Background on evaluation of PCORI Merit Review
- Information on merit review scores to support ongoing assessment of PCORI's merit review process
 - Impact of in-person discussion
 - Prediction of funding decisions and final review scores



Engaging Patients and Stakeholders in Research Proposal Review: The Patient-Centered Outcomes Research Institute

Rachael L. Fleurence, PhD; Laura P. Forsythe, PhD, MPH; Michael Lauer, MD; Jason Rotter, MHS; John P.A. Ioannidis, DSc, MD; Anne Beal, MD, MPH; Lori Frank, PhD; and Joseph V. Selby, MD, MPH

The inaugural round of merit review for the Patient-Centered Outcomes Research Institute (PCORI) in November 2012 included patients and other stakeholders, as well as scientists. This article examines relationships among scores of the 3 reviewer types, changes in scoring after in-person discussion, and the effect of inclusion of patient and stakeholder reviewers on the review process. In the first phase, 363 scientists scored 480 applications. In the second phase, 59 scientists, 21 patients, and 31 stakeholders provided a “prediscussion” score and a final “postdiscussion” score after an in-person meeting for applications. Bland–Altman plots were used to characterize levels of agreement among and within reviewer types before and after discussion. Before discussion, there was little agreement among average scores given by the 4 lead scientific reviewers and patient and stakeholder reviewers. After discussion, the 4 primary

reviewers showed mild convergence in their scores, and the 21-member panel came to a much stronger agreement. Of the 25 awards with the best (and lowest) scores after phase 2, only 13 had ranked in the top 25 after the phase 1 review by scientists. Five percent of the 480 proposals submitted were funded. The authors conclude that patient and stakeholder reviewers brought different perspectives to the review process but that in-person discussion led to closer agreement among reviewer types. It is not yet known whether these conclusions are generalizable to future rounds of peer review. Future work would benefit from additional data collection for evaluation purposes and from long-term evaluation of the effect on the funded research.

Ann Intern Med. 2014;161:122–130. doi:10.7326/M13-2412

For author affiliations, see end of text.

www.annals.org



Acknowledgements

- Merit Review Score Analysis Workgroup
 - Christine Goertz (Chair, Scientific Oversight Committee)
 - Mike Lauer (Methodology Committee)
 - Yen-Pin Chiang (Associate Director, ADTPO)
 - Steve Clauser (Program Director, IHS)
 - PCORI Evaluation & Analysis staff
- Provide expert input on PCORI's evaluation of Merit Review, including analysis of score data and integration of these data with findings from other sources



How is PCORI Merit Review Unique?

- Brings together scientists, patients, and other stakeholders to review proposals
- Uses unique funding criteria
 - Criterion 1: Impact of the condition on the health of individuals and populations
 - **Criterion 2: Potential for the study to improve health care and outcomes**
 - Criterion 3: Technical merit
 - **Criterion 4: Patient-centeredness**
 - **Criterion 5: Patient and stakeholder engagement**



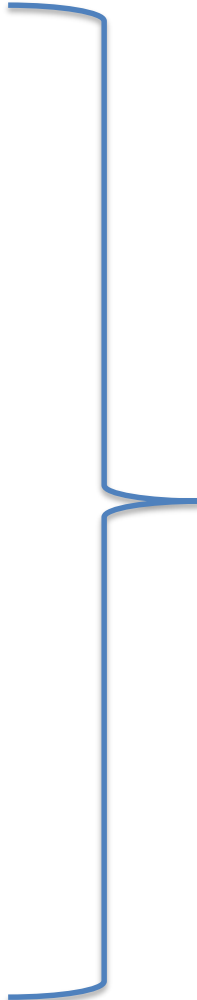
Score Analysis Questions

1. What is the impact of in-person discussion on
 - reviewer scores?
 - agreement between reviewers?
2. Which criteria contribute the most to prediction of
 - funding decisions?
 - final review scores?
3. What is the relationship between the merit review criteria scores?
4. What are reviewer impressions of PCORI Merit Review?



Information Sources

- **Merit Review score analyses**
- Reviewer survey
- Reviewer focus groups
- Applicant survey
- Analysis of applicant characteristics
- CER literature search
- PCORI Stakeholder surveys
- LOIs and application critique review
- Data collection on engagement



PCORI Staff & Committees



Dissemination of Score Analysis Findings

- Shared learnings with PCORI staff and committees
- Session at PCORI Annual Meeting on Evaluating Merit Review
- Scientific manuscripts in preparation



Information on Merit Review Scores



Key Takeaways (1 of 2)

- The in-person discussion has an impact on application review
- While PCORI Merit Review brings together reviewers from a variety of perspectives, they tend to agree about which projects best meet PCORI criteria



Key Takeaways (2 of 2)

- All reviewer impressions of the potential to improve health care and outcomes, as well as scientist reviewer views of the technical merit and patient-centeredness of the research, are critical to success in merit review
- All reviewer views influence funding decisions and each others' final review scores
 - Scientist reviewer ratings have a strong influence on scores
- Technical merit is important to all reviewer types



What is the impact of in-person discussion on reviewer scores? Agreement between reviewers?

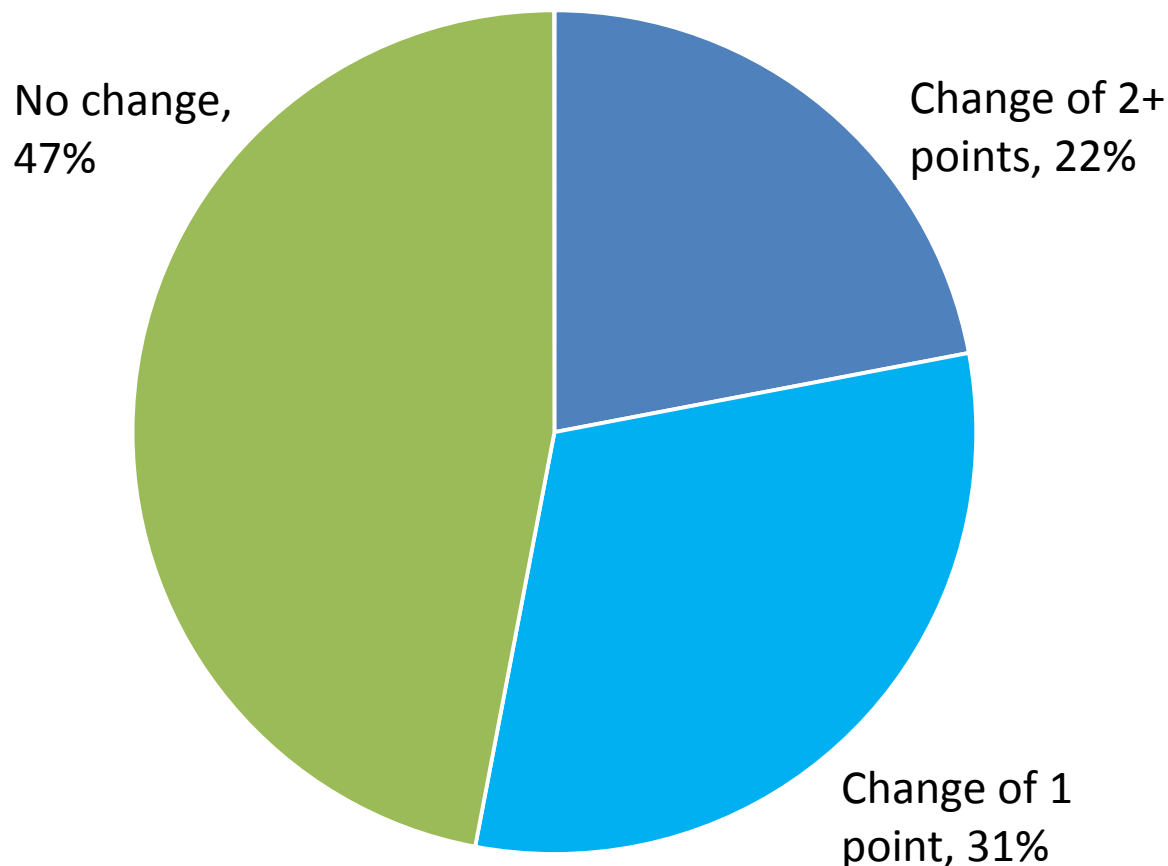


Agreement Before and After Panel Discussion

- Analyses of agreement show that agreement is greater after discussion across all reviewer types, particularly for proposals initially scored in the middle of the scoring range
 - Replicates findings from Fleurence et al., 2014 based on PCORI's first review cycle



Changes in Review Scores after Discussion



Scores change about half of the time, and of those, 81% of score changes are towards **poorer** scores



Changes in Review Scores after Discussion

	Scientists N (%)	Patients and Stakeholders N (%)
-7 to -2 (scores improved)	24 (1%)	60 (3%)
-1	156 (8%)	139 (7%)
0 (no change)	991 (50%)	836 (44%)
1	495 (25%)	408 (22%)
2 to 7 (scores worsened)	309 (16%)	453 (24%)
Mean [std] change	0.5 [1.1]	0.7 [1.4]*

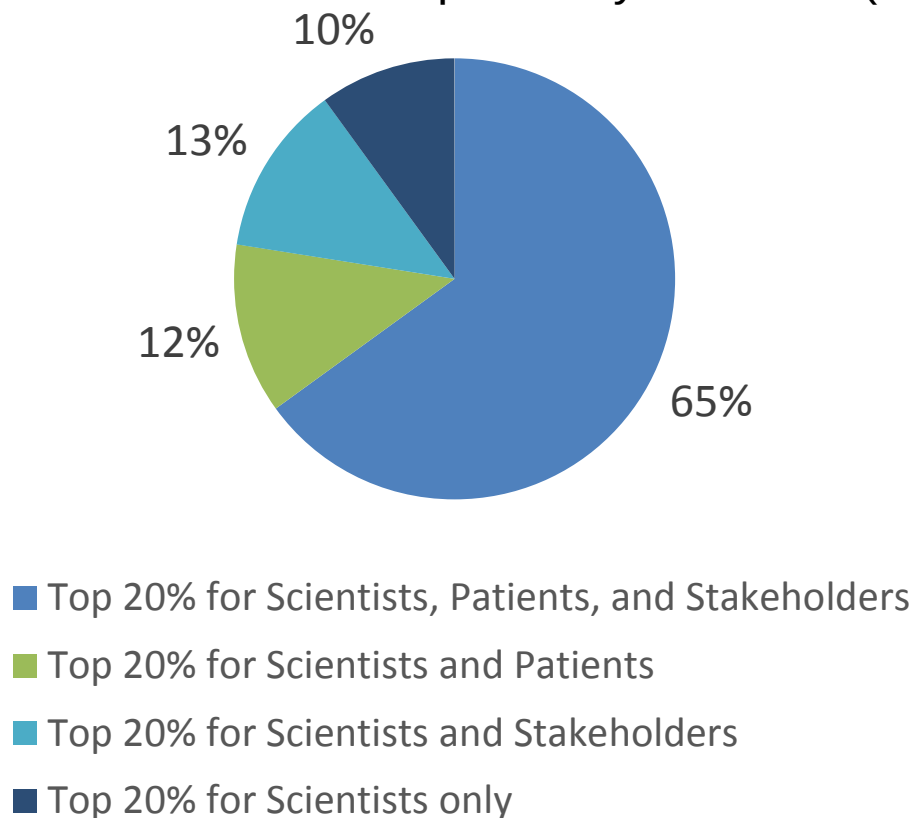
**Statistically significant difference between scientists and patients/stakeholders, $p < 0.001$*

All reviewer types change their scores after discussion. Patients and stakeholders showed larger changes in scores



Agreement on Applications Scored in the Top 20%

Proposals scored in the top 20% by scientists (N = 120)



Scientist, Patient, and Stakeholder reviewers tend to agree on the top applications.



Summary: Impact of In-Person Discussion

- The in-person discussion has an impact on application review
- While PCORI Merit Review brings together reviewers from a variety of perspectives, they tend to agree about which projects best meet PCORI criteria



Which criteria contribute the most to prediction of funding decisions? Of in-person review scores?



Review Criteria Scores and Funding Decisions

Online Scores

Impact of the Condition

Potential to Improve Health

Technical Merit

Patient Centeredness

Engagement

Potential to Improve Health

Patient Centeredness

Engagement

Potential to Improve Health

Patient Centeredness

Engagement

Scientists

Patients

Stakeholders

**Funded
Proposals**

Logistic regression model for
funding status (Yes/No)

N=1261 applications, includes
August 2013 to Fall 2014



Review Criteria Scores and Funding Decisions

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Review Criteria Scores and In-Person Scores

Online Scores

Final Scores

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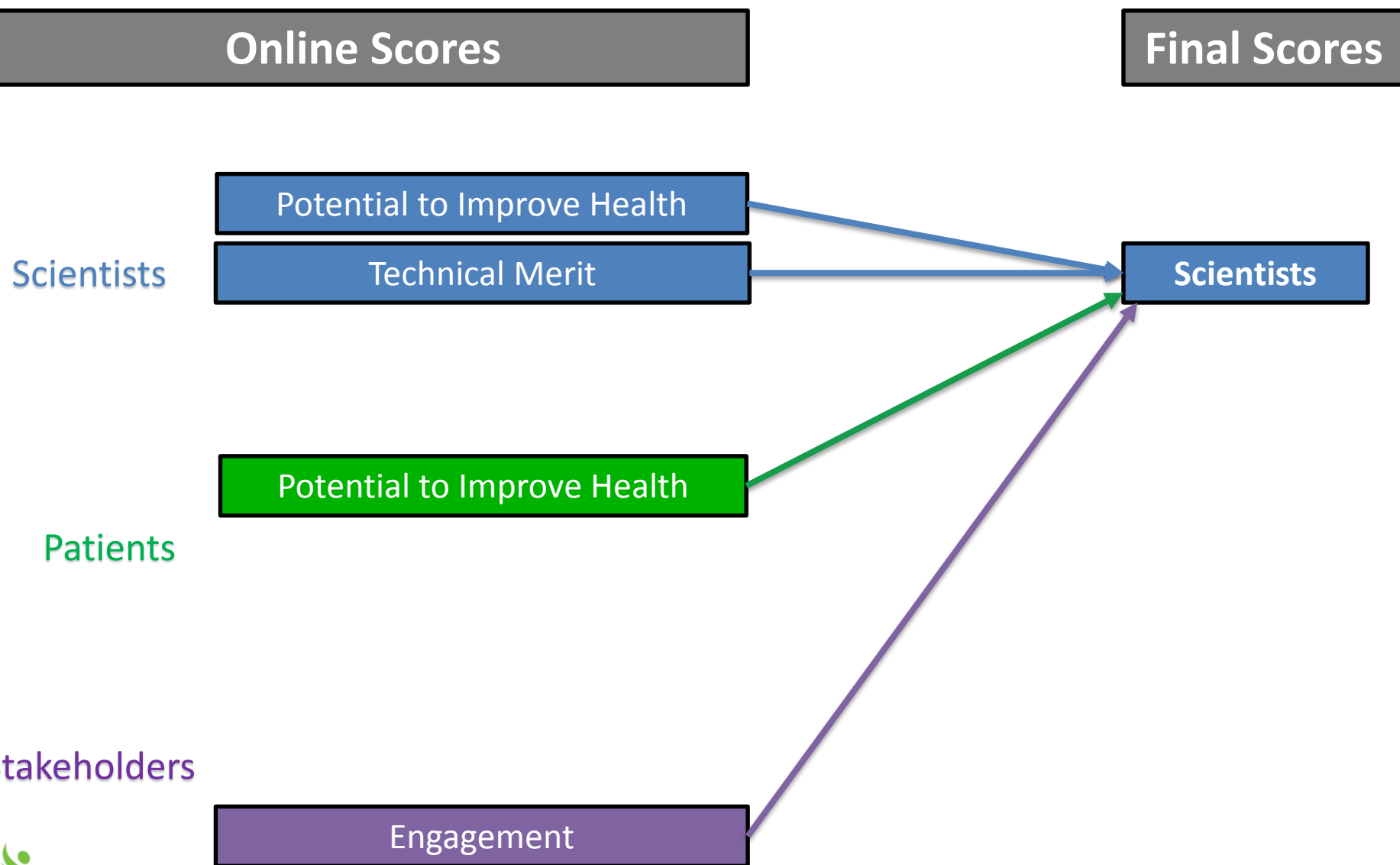
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Review Criteria Scores and In-Person Scores



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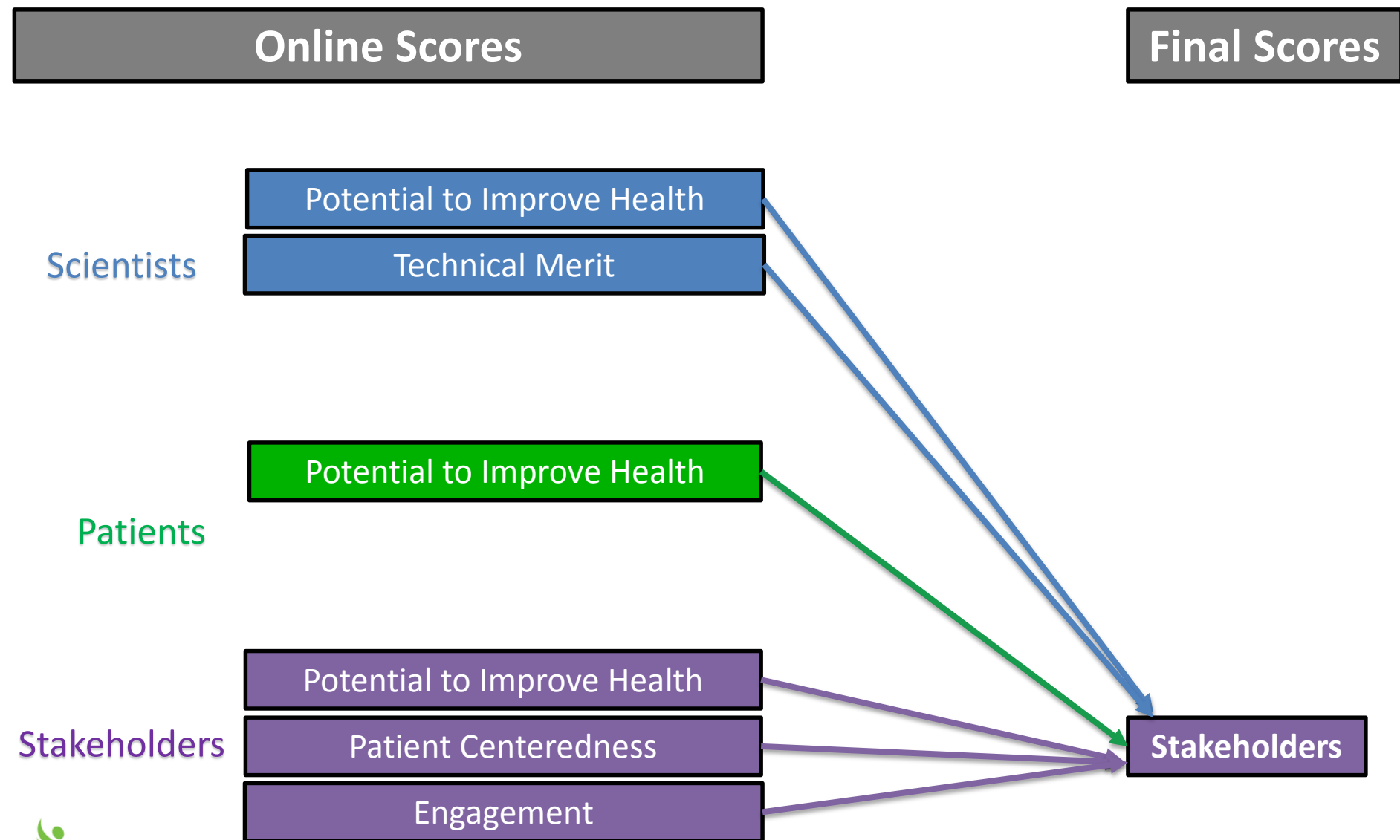
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Review Criteria Scores and In-Person Scores



Review Criteria Scores and In-Person Scores

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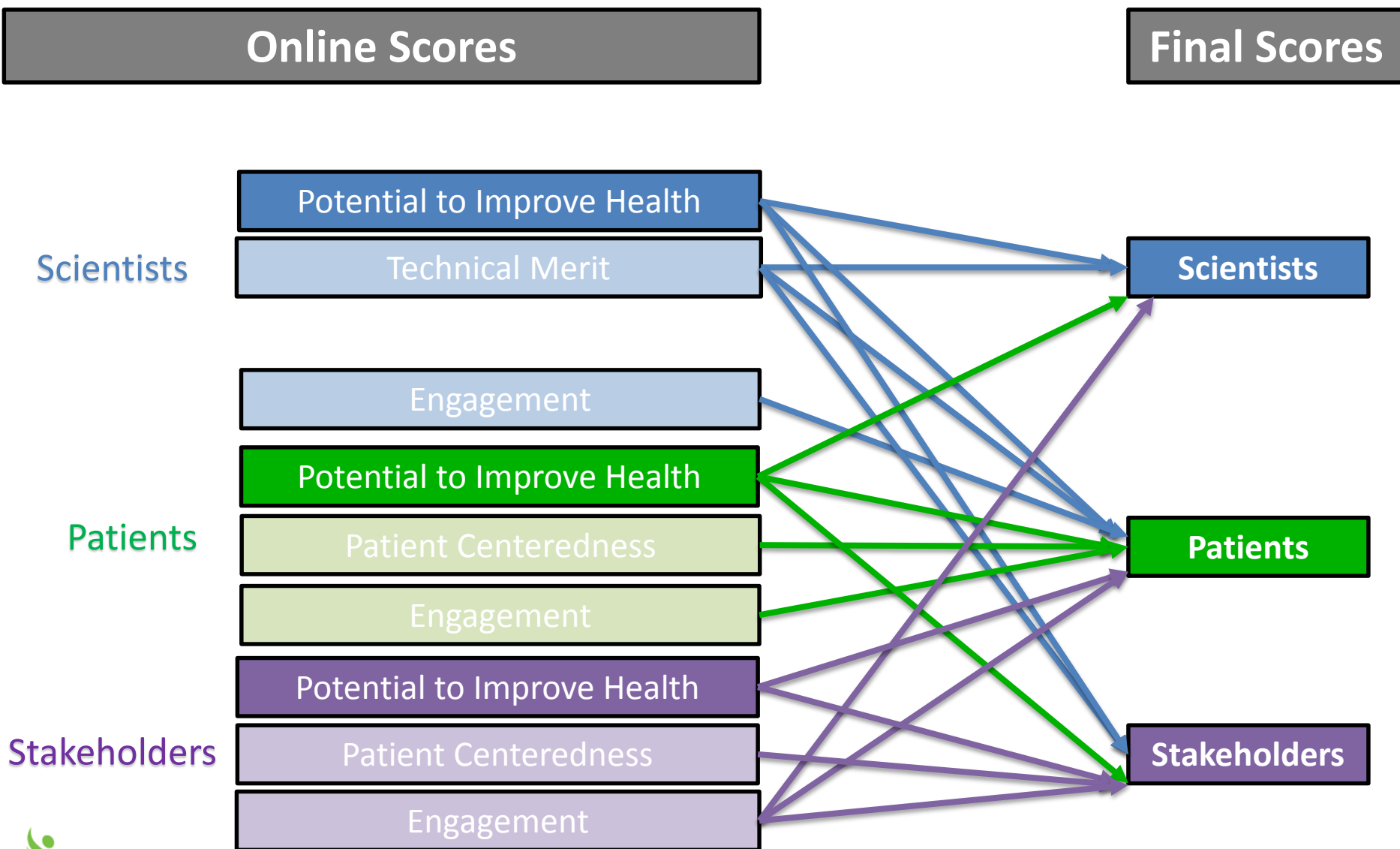
Stakeholders

Patients

Stakeholders



Review Criteria Scores and In-Person Scores



Review Criteria Scores and In-Person Scores

- For each reviewer type, final overall scores were related to criteria scores from all the other types of reviewers
- The strongest predictor of final overall scores for each type of reviewer were scientist ratings of technical merit
- Assessments of potential to improve health care and outcomes by all reviewer types were important



Summary: Funding Decisions and Review Scores

- All reviewers' impressions of the potential to improve health care and outcomes, as well as scientist reviewer views of the technical merit and patient-centeredness of the research, are critical to success in merit review
- Scientist, patient and stakeholders view influence funding decisions and each others' final review scores
 - Scientist reviewer ratings have a strong influence on scores
- Technical merit is important to all reviewer types



Discussion

- What other questions about Merit Review do you have that an analysis of scores could answer?
- What other ways of examining the influence of patient and stakeholder reviewers would you suggest?



A Possible Role for PCORI in Workforce Training

Joe Selby, MD, MPH

Executive Director

Richard Kronick, PhD

Director, AHRQ

Board of Governors and EDIC Member

Harlan Weisman, MD

Managing Director, And-One Consulting

Board of Governors and RTC Member



Questions for the Board

- Is Research Workforce Training an appropriate area of investment for PCORI?
- If so, is a focus on patient-centered research within learning health systems the best approach?
- Should all PCORI work on this topic be done through AHRQ?
- Does the timeline seem reasonable?



Overview

- PCORI—IOM meetings in 2014 established a need for trained researchers skilled at working *within learning health systems, with health systems data and with systems executives/leaders*
- AHRQ has strong interest in expanding its PCOR training programs to include a focus on the learning health system
- Ongoing discussions at the RTC and with AHRQ strengthen the thinking that training researchers in these settings would be valuable
- Efforts from AHRQ, AcademyHealth, and Robert Wood Johnson Foundation should be considered and could be opportunities for collaboration
- PCORI could bring a strong, unique focus and resources to workforce training needs in this area



AHRQ Collaboration

- Starting in January 2016, AHRQ plans to convene a panel of up to 15 experts to advise on workforce training roll-out
 - Panel will draw from learning health systems, health services research, and patient-centered outcomes research fields
- Expected Timeline: Six months for planning and evidence gathering followed by six months for training program implementation
- AHRQ has briefed the RTC on this program and PCORI is invited to participate

Goals of AHRQ Panel



Advantages of Collaborating with AHRQ

- Researcher training is already established at AHRQ, including training in PCOR, and workforce training is within AHRQ's scope of work in our authorizing legislation
- PCORI can leverage AHRQ's expertise in the area of researcher training
- AHRQ has already allocated funding dollars towards workforce training and there is opportunity for PCORI to supplement if endorsed by the Board of Governors



Other Interest and Potential Partners in Workforce Training:

- **AcademyHealth**
 - Launching the next round of its periodic reassessment into training needs in health services research in early 2016. PCORI is sponsoring and participating, along with AHRQ, the VA, and RWJF
- **Robert Wood Johnson Foundation (RWJF)**
 - Initiated the National Leadership Program Initiative (NLPI), which includes a “New Clinical Scholars” program
 - NLPI’s goal is building a ‘Culture of Health’ to attain the best health possible for society through interdisciplinary collaboration
 - Trains a variety of “clinicians,” not just physicians, to work within communities to improve health care and community health
 - Emphasis is not entirely on research, but on leadership



Continuation of RWJF Programs

- **National Leadership Program**

- Decentralized (not site-based) with a cohort of 50 trainees per year in each of 4 programs
- More focus on interdisciplinary collaboration and leadership training
- Engaging both individuals and small teams of trainees who are further along in their career

- **National Clinician Scholars Program (NCSP)**

- RWJF ended funding of its Clinical Scholars Program, so the four host institutions formed a consortium to continue their traineeships
- Directed to training physician and nurse scientists only with a yearly cohort of 20 trainees
- Remains site-based and will be more project-focused based on the host institution initiatives



PCORI's Novel Contributions

- PCORI can determine needed elements of training curricula to support the needs of the current PCOR landscape
 - Promote patient centeredness
 - Focus on learning health systems
 - Possible synergies with PCORnet



Points for Discussion (1 of 2)

1. Should PCORI further investigate possibilities to contribute to workforce training?
2. Should it do so in collaboration with AHRQ and after these two surveys are completed?
3. Does the Board have thoughts on who should be targeted for training (clinicians, non-clinicians scientists), important elements of curricula, and ideal settings to establish learning health systems?



Points for Discussion (2 of 2)

4. Funding could target individual traineeships, centers of excellence within institutions, or broader systems
 - Who are the optimal awardees: individual, academic centers, or health systems?
5. PCORnet's CDRNs would be a feasible starting point to implement a pilot workforce training program leveraging established PCORnet infrastructure
 - PCORnet institutions have an established dedication to PCOR and quality data



Appendix:

More information on:

***RWJF Clinical Scholars Program,
AHRQ Training Programs***

and

The National Leadership Program Initiative



RWJF Clinical Scholars Program

- The Clinical Scholars program operated from 1972-2015 and funded over 1,100 physician researchers
- The 2015 Clinical Scholars cohort of 31 physicians was announced August 5th of 2014. While this is the largest cohort in recent history, the program will be discontinued after the grantees' two year stint
- The program supports scholars (in various amounts) for master's degree level study and research in a post-residency training program. It allows two years for study and prolonged protected time for further research
 - Involves formal coursework, methods of healthcare research, mentorship and guidance in project development
- Operated at four institutions: UCLA, University of Michigan, University of Pennsylvania, and Yale University
- Fewer career prospects, dwindling funds, and fewer applications led to the phase-out of the program



Clinical Scholars Program Impact

- Legitimized health services research and health policy research as major disciplines, creating a base of physician leadership in this field and a new body of evidence
- Major focuses of the program included leadership development, community-based participatory research (CBPR) and policy change
- One metric for the program's success is the ascendancy of scholar alumni to leadership positions
 - Hospital administrators and CEOs
 - Academic professors, deans and chairs
 - AHRQ/CMS/other government leadership
- Other metrics include impact of Scholars on research, influence of Scholars on specialty areas of health care (particularly oncology, emergency medicine, and pediatrics), and the CBPR focus

<http://www.rwjf.org/en/library/research/2011/08/robert-wood-johnson-clinical-scholars-program.html>

http://www.rwjf.org/content/dam/farm/reports/program_results_reports/2013/rwjf70030



RWJF National Leadership Program

- **Goal of NLPI is building a ‘Culture of Health’ to attain the best health possible for society through interdisciplinary collaboration***
 - It is meant to build upon RWJ’s past clinical scholarships and general dedication to strengthening the US healthcare workforce
- The Foundation announced the four partnering institutions hosting new leadership initiatives on November 2nd, 2015
- Initial planning grant of up to \$750,000 for a three-month planning phase, in addition to an expectation of a longer term commitment to these programs beyond grant period
- Roll-out of programs planned to begin in September 2016

*Robert Wood Johnson, RWJF Announces National Program Centers for Major New Leadership Initiatives, www.rwjf.org.



NLPI Centers

1. **Diversity in Health Policy Research (DHPR):** Johns Hopkins University, Baltimore, MD
 - Provides training in health policy and population health to doctoral students from disadvantaged backgrounds
2. **Interdisciplinary Research Leaders (IRL):** University of Minnesota, Minneapolis, Minnesota
 - Develops groups of researchers and community members dedicated to producing evidence needed
3. **Multisector Leaders for Health (MLH):** National Collaborative for Health Equity, a project of New Venture Fund, Washington, D.C.
 - Unites diverse group of leaders representing key sectors of determinants of health (education, policy, business, urban planning, etc.) to grow influence
4. **New Clinical Scholars (NCS):** University of North Carolina at Chapel Hill, NC
 - Develops cohorts of clinicians to lead change in communities

<http://www.rwjf.org/en/library/articles-and-news/2015/11/major-new-leadership-initiatives.html>



AHRQ Workforce Development Initiatives

- Training dollars from the very beginning (PCOR Trust Fund)
 - Institutional training for PCOR (K12 awards) – centers of excellence
 - Individual awards (K awards)
 - Institutional capacity (R24 awards) – infrastructure development in PCOR; especially directed to institutions with a track record of community engagement
 - K99ROO awards – Supports transition from mentored to independent PCOR with a guaranteed tenure-track faculty position
 - K18 award – mid-career award for those who conduct research but do not yet know CER/PCOR
 - NRSA T32 awards – also have PCOR training in their programs (not PCOR Trust Fund)



AHRQ Workforce Development Initiatives (cont.)

- Adequacy of training for researchers
 - AHRQ has been able to fund at about the 30th percentile of applications
 - AHRQ has funded about 35 institutions, primarily on the East Coast and West Coast
 - Currently about 130 trainees at any point in time.
- AHRQ's interest in Training researchers to support the Learning Health System:
 - Training investigators, especially clinicians, to work more effectively
 - Training non-clinician researchers (sociologists, psychologists, economists) to become multidisciplinary researchers
 - Equipping all researchers with the knowledge and skills needed to collaborate with executives, operations leaders, and quality improvement leaders within delivery systems and health plans
 - Emphasis is on post-doctoral training of “purebreds” to broaden their skills and understanding to enable them to work and think in an interdisciplinary manner



Open Science Update

Jason Gerson, PhD

Associate Director, CER Methods



PCORI Open Science Working Group

- The Working Group was convened in the summer of 2015 to revise the Draft Policy on Open Science and to make recommendations for how to operationalize that policy.
- The Working Group is comprised of staff members from Science, Legal, and Information Technology (Aggarwal, Chiang, Convery, Evans, Gerson, Gurgol, Moscou-Jackson, Peters)
- Today, we will update you on our efforts and apprise you of our planned activities
 - PCORI's consultation with national experts
 - Recap of Annual Meeting Plenary Session regarding Open Science
 - Public release of draft Open Science policy
 - Future planned activities/action items



Consultation with National Experts

- Members of PCORI's Open Science Working Group have spoken with a number of leading national experts about some of the operational and technical challenges of implementing an Open Science policy. These conversations have addressed a number of critical considerations, including, but not limited to:
 - operational challenges of building and maintaining data repositories
 - making key decisions about centralized versus federated models
 - challenges regarding de-identification of data
 - the development and enforcement of data use agreements
 - issues of informed consent
 - ascertaining participant perspectives on data sharing



Annual Meeting: Open Science Plenary Session

- Keynote
 - Francis Collins, MD, PhD (NIH and PCORI board member) – historical context of open science with a focus on past and present NIH initiatives
- Panelists
 - Moderated by Austin Frakt, PhD (VA/BU)
 - Steve Goodman, MD, MHS, PhD (Stanford and PCORI Methodology Committee) – PCORI Open Science Framework and key decision points for implementation
 - Philip Bourne, PhD (NIH) – operational challenges of building and maintaining data repositories, NIH Commons Initiative
 - Bradley Malin, PhD (Vanderbilt) – challenges regarding de-identification of data, data use agreements
 - Michelle Mello, PhD, JD (Stanford) – informed consent; patient perspectives on open science



Annual Meeting: Open Science Plenary Session

- Key Takeaways:
 - Incremental progress is worthwhile and prudent
 - Infrastructure (technology, governance, staffing) to support data sharing is non-trivial
 - Identify collaborative opportunities with other funders and institutions
 - Building and maintaining trust in the organization managing data sharing is critical
 - Awardee and data user compliance with data sharing policy must be measured and evaluated



Draft PCORI Open Science Policy

- The Open Science Working Group has drawn on the insights gathered from our interviews and the plenary session, and developed a revised Policy for Open Science and Data Sharing
- The broad goal of the draft policy is to articulate PCORI's commitment and vision for open science and to signal expectations for applicants, awardees and other stakeholders
- The purpose of the policy is to: (a) facilitate reproduction of original analyses to increase the integrity of PCORI-funded research findings; and (b) promote data sharing to enable conduct of additional analyses using data from PCORI-funded studies, thereby augmenting the knowledge generated from the original study



Draft PCORI Open Science Policy (2)

- The draft policy is organized around three sets of requirements:
 - Applicants: must demonstrate a willingness to support open science and describe planned activities that will enable Data Sharing in their application
 - Awardees: must prepare for possible future requests for Data Sharing by developing a data management and data sharing plan in a manner consistent with applicable privacy, security and other legal requirements
 - Data Sharing Requests: such requests may originate from third party researchers and/or PCORI program staff
- The policy is drafted in a manner that will enable PCORI to incorporate additional operational details and procedures over time, based on learning from a planned pilot phase and decisions informed by experts
- The Research Transformation Committee reviewed and discussed the draft policy during their September 2015 Meeting
- The draft policy will be released for public comment in early 2016 after presentation to the Board for approval at a future meeting



Future Planned Activities

- The Open Science Work Group will undertake the following activities in the next 3 months:
 - Identify 2-3 projects in PCORI portfolio that are willing to serve as pilot studies for testing data sharing approaches
 - Identify 3-4 data repositories that may be acceptable for PCORI purposes
 - Develop a draft governance protocol for reviewing and evaluating data access requests
 - Convene 6-8 external experts that can serve as an advisory group for ongoing efforts



Discussion Questions

- What should be the criteria for selecting projects for the pilot exercise?
- At the conclusion of the pilot, what outcome measures should we use to evaluate it and help us determine success factors?



Public Comment Period

Sue Sheridan, MBA, MIM

Director, Patient Engagement



Wrap Up and Adjournment

Grayson Norquist, MD, MSPH

Chairperson, Board of Directors

