

# Advisory Panel on Clinical Trials

## Spring 2017 Meeting

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Washington, DC

March 30, 2017



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# Welcome and Goals for the Day

**Anne Trontell, MD, MPH**

Associate Director, Clinical Effectiveness and Decision Science, PCORI

**Elizabeth A. Stuart, PhD, AM (Chair)**

Associate Dean for Education & Professor of Mental Health,  
Biostatistics, and Health Policy and Management,

The Johns Hopkins Bloomberg School of Public Health

**John D. Lantos, MD (Co-Chair)**

Director of Pediatric Bioethics & Professor of Pediatrics,  
Children's Mercy Hospital



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# Housekeeping

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- Today's webinar is open to the public and is being recorded.
- Members of the public are invited to listen to this teleconference and view the webinar.
- Anyone may submit a comment through the webinar chat function or by emailing [advisorypanels@pcori.org](mailto:advisorypanels@pcori.org).
- Visit [www.pcori.org/events](http://www.pcori.org/events) for more information.
- Chair Statement on COI and Confidentiality



# Goals for the Day

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To provide advice to PCORI on next steps regarding:

- PCORI oversight of recruitment, accrual, and retention in clinical trials and the development of best practices
- Cluster designed trials
- Revisions to the Common Rule
- Use of protocol templates by PCORI awardees
- PCORI's Open Science pilot



# Today's Agenda

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Start Time	Item	Speaker
9:00 a.m.	Welcome and Goals for the Day	A. Trontell E. Stuart J. Lantos
9:15 a.m.	2016 CTAP Accomplishments & Plans for 2017	A. Trontell
9:45 a.m.	Recruitment, Accrual, and Retention (RAR)	A. Trontell M. Orza
10:20 a.m.	Break	
10:30 a.m.	Recruitment, Accrual, and Retention Issues Raised at the 2016 PCORI Annual Meeting	C. Girman A. Ambrosio
11:00 a.m.	Panel Discussion and Advice: Best Practice Development for Recruitment, Accrual, and Retention	C. Girman
11:30 a.m.	Recognition of Panelists	A. Trontell



# Today's Agenda (cont.)

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Start Time	Item	Speaker
11:45 a.m.	Lunch	
12:45 p.m.	Board of Governors Recommendations on Priorities of Clinical Trial Monitoring	E. Whitlock
1:00 p.m.	PCORI's Cluster Designed Trials	D. Hickam
1:30 p.m.	Implications of Changes to the Common Rule	J. Lantos
2:00 p.m.	Protocol Guidance for Awardees	H. Sox
2:30 p.m.	Break	
2:45 p.m.	Open Science Update	J. Gerson
3:15 p.m.	Wrap Up and Next Steps	E. Stuart J. Lantos A. Trontell
3:30 p.m.	Adjourn	



# 2016 CTAP Accomplishments & Plans for 2017

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**Anne Trontell, MD, MPH**

Associate Director, Clinical Effectiveness and Decision Science, PCORI



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# CTAP Progress to Date

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## Accomplishments 2016

- New members successfully recruited
- Planning instituted for short- and longer-term goals and activities
- Recruitment, Accrual, and Retention Subcommittee
  - Contribution of patient-centered recruitment, accrual, and retention principles under Methodology Standards Associated with Patient-Centeredness
  - Proposal for patient-centered informed consent deferred by Methodology Committee
- Subcommittee on Standardization of Complex Concepts & Terminology
  - Draft document prepared on pragmatic clinical studies (PCS)



# Plans for 2017

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- Subcommittee activities and products
  - Subcommittee on Standardization of Complex Concepts & Terminology
    - PCORI Guidance on Pragmatic Clinical Studies
    - Companion journal commentary by Merrick Zwarenstein
  - Recruitment, Accrual, and Retention Subcommittee
    - Development of PCORI Guidance on Best Practices in RAR
    - Advice on PCORI monitoring practices for RAR
- Pilot Implementation of CTAP members on Study Advisory Committees
- CTAP input, feedback, & advice on above as well as additional topics on approximately quarterly basis



# Recruitment, Accrual, and Retention

## Recruitment Progress in PCORI Trials: Q3 2016 Recruitment Data

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**Michele Orza, ScD**

Senior Advisor to the Executive Director, PCORI

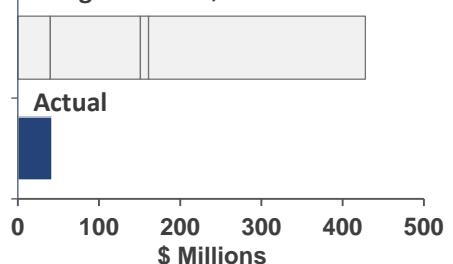


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### Funds Committed to Research

Includes funds committed to PCORnet

Budgeted      \$428M for FY-2017

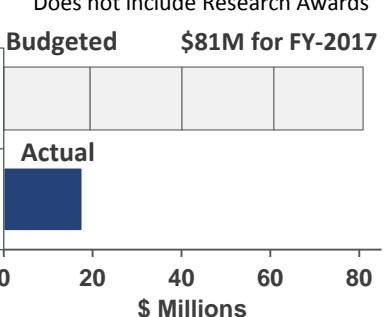
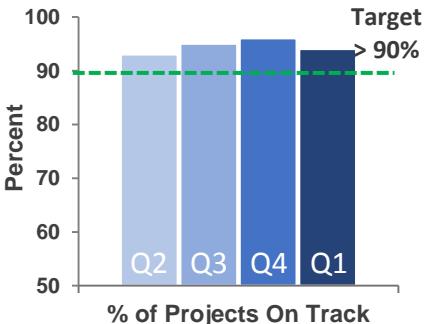


### Project Performance

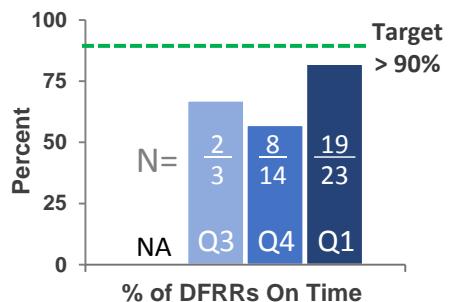
### Operating Budget

Does not include Research Awards

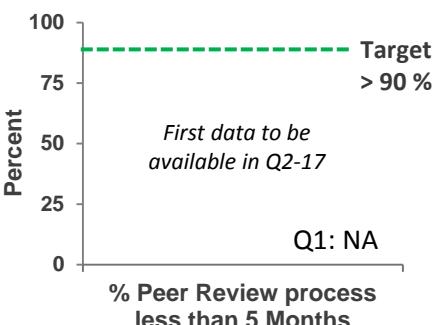
Budgeted      \$81M for FY-2017



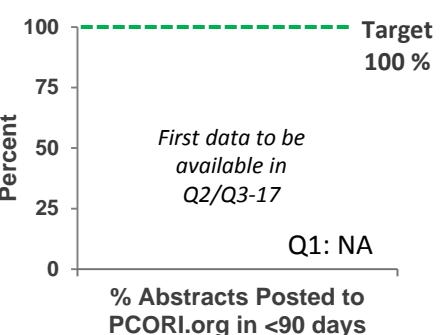
### Draft Final Research Reports



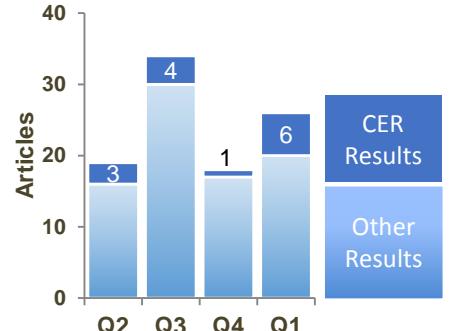
### PCORI Peer Review



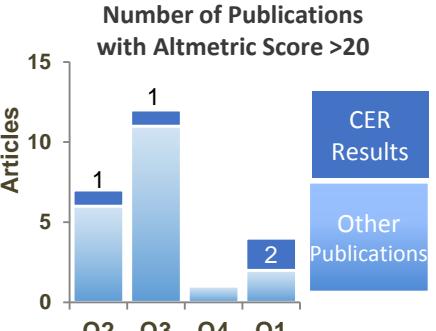
### Public Reporting of Research Findings



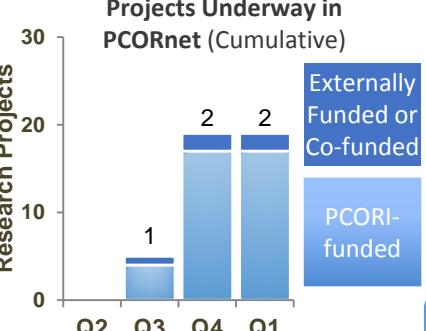
### Results Published in Literature



### Altmetrics



### Research in PCORnet



Inputs

Process

Outputs

Uptake

Use

Impact

### Narrative Examples

#### Goal One Increasing Information

Use of a decision aid in patients with low risk **chest pain** increased understanding of risk and safely decreased the rate of admission to an observation unit for cardiac testing

#### Goal Two Speeding Implementation

We awarded one of our first D&I projects to a PCORI-funded study on preventing non-administration of **VTE prophylaxis** to implement the intervention in two large hospital settings

#### Goal Three Influencing Research

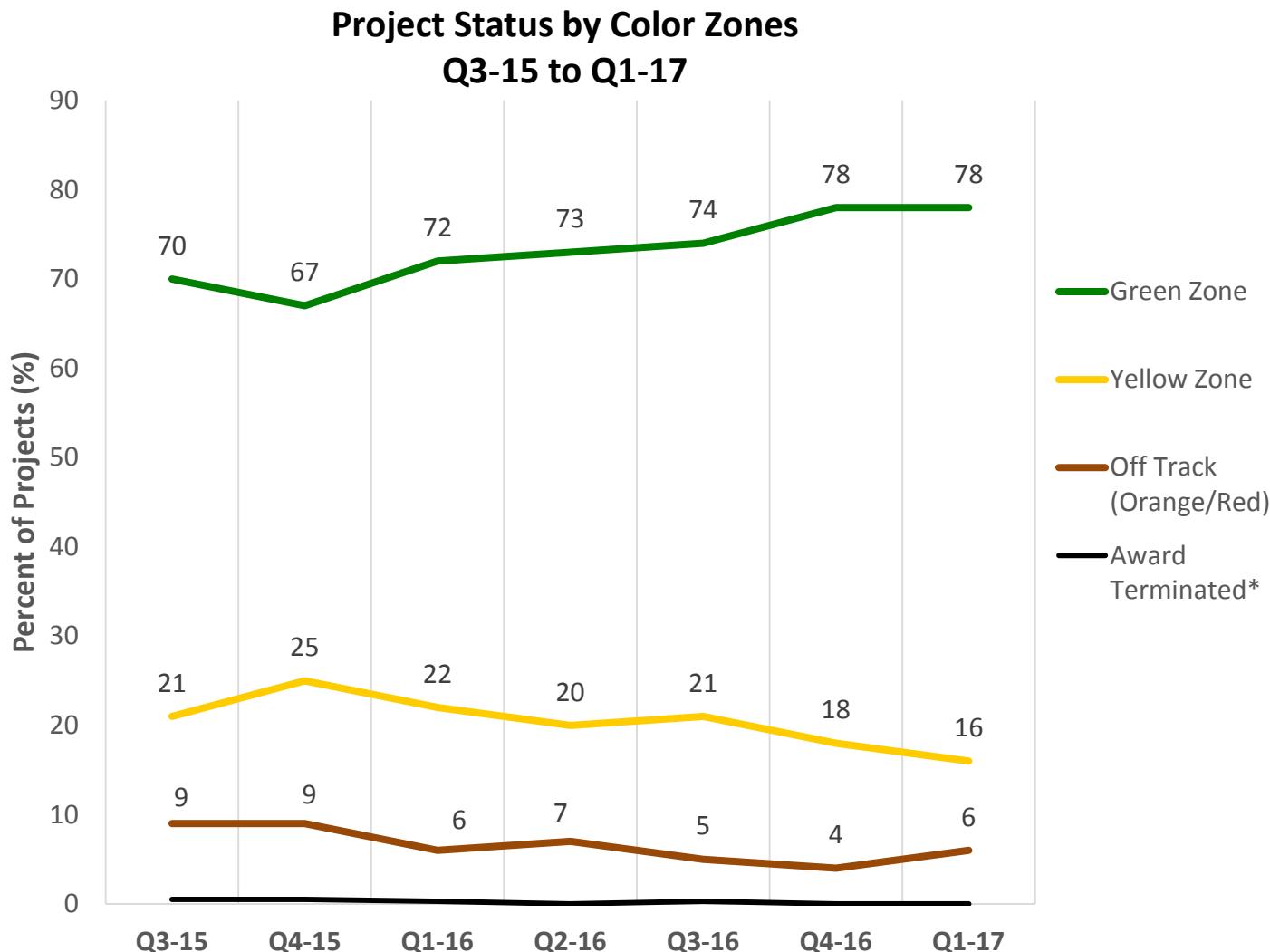
PCORI is credited as a model for Henry Ford Health System's **Patient Engagement Research Center (PERC)**, which brings together researchers and patient advisory groups to improve patient care

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

GREEN	YELLOW	ORANGE	RED
<p><b>GREEN</b> The Project is meeting &gt;85% of milestones on time</p> <p>-AND-</p> <p><b>Recruitment occurring on schedule, at expected rate</b></p> <p>-AND-</p> <p>PO judges that the project has a high probability of meeting its objectives as planned. PO judgment is based on close review of study progress, including recruitment status.</p>	<p><b>YELLOW</b> Project does not meet all criteria for "Green"</p> <p>-AND-</p> <p>Project is meeting <math>\geq 65\%</math> of milestones on time.</p> <p>-OR-</p> <p><b>Recruitment is <math>\leq 75\%</math> and <math>&gt;50\%</math> of target accrual</b></p> <p>-OR-</p> <p>PO has concerns that without remediation efforts the project will not be able to meet objectives within project period.</p>	<p><b>ORANGE</b> Project does not meet all criteria for "Yellow"</p> <p>-AND-</p> <p>Project is meeting <math>\geq 50\%</math> of milestones on time.</p> <p>-OR-</p> <p><b>Recruitment is <math>\leq 50\%</math> of target accrual</b></p> <p>-OR-</p> <p>PO has concerns that the project will not meet objectives within the approved project period. Modifications to the Milestone Schedule and/or project plan are likely required.</p>	<p><b>RED</b> Project does not meet all criteria for "Orange"</p> <p>-AND-</p> <p>Project is meeting <math>&lt;50\%</math> of milestones on time.</p> <p>-OR-</p> <p><b>Recruitment is persistently and significantly <math>\leq 50\%</math> of target</b></p> <p>-OR-</p> <p>PO has significant concerns that the project cannot meet its original objectives. Major modifications to Milestone Schedule are required for the project to be completed.</p>
<b>Next Steps</b>			
Continue monitoring project through active portfolio management and per SOPs.	Increased communication with the PI to monitor and assist with getting the project back on track	Placed Under Review at PCORI to determine if it is able to meet its original project plan.	Project Remediation Plan (PRP) memo sent to PI with a 30-day completion date deadline.
		Pursue modifications to project plan or milestone schedule as appropriate.	Inform Leadership of Status



# We are monitoring trends and shifts in project status

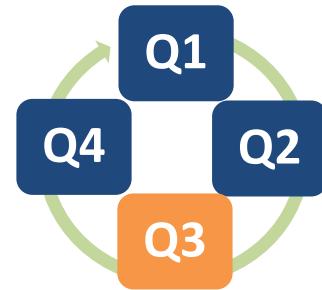


\*Notice of Termination Issued,  
<1% in each quarter



# Q3-16 Focus on Recruitment

Recruitment in Focus: Q3



# Identifying Benchmarks

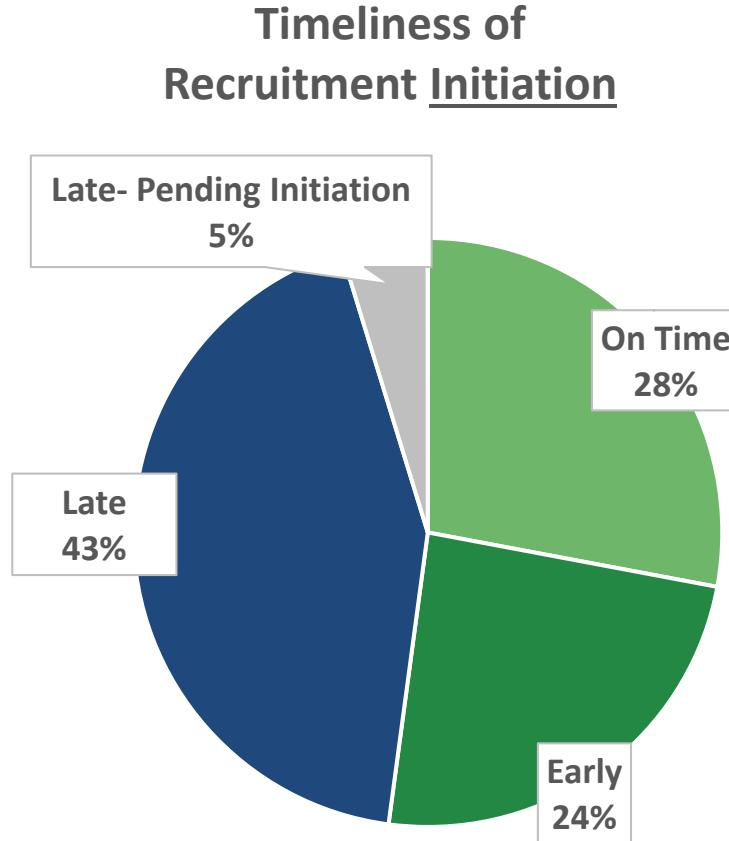
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- **Limited Benchmarks for Portfolio Management**- Through literature searches and working with other funders, we identified *points of reference for research projects*:
  - A target of 100% of projects on time for 100% of their milestones, is not approached, let alone attained, by other funders
  - Most research projects fall behind, especially on recruitment, require extensions and/or additional funding in order to be successfully completed
  - A significant proportion of research projects are not successfully completed –around 10%, or even higher for projects that involve recruitment
- **Limited Benchmarks for Recruitment/Enrollment** (primarily pharmaceutical trials):
  - **47% of studies meet enrollment timeline**<sup>1,2,3</sup> while other half are delayed. Study timelines are typically extended to nearly double their original duration to meet desired enrollment levels<sup>3</sup>. **Startup phase is a bottleneck**, and ability to reduce start-up time decreases overall study costs<sup>4</sup>.
    - Mary Jo Lamberti et al. Evaluating the Impact of Patient Recruitment and Retention Practices. *Clinical Trials*, 2012
    - Kenneth Getz. Enrollment Performance- Weighing the “Facts.” *Applied Clinical Trials*, 2012
    - 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.
    - Mary Jo Lamberti et al. Benchmarking the Study Initiation Process. *Clinical Trials*, 2013



# Did Projects Initiate Recruitment on Time? (N=211)

For all projects that have or should have initiated recruitment



## Reasons for Delayed Initiation

- Subcontract negotiation
- IRB Approval
- Staff turnover

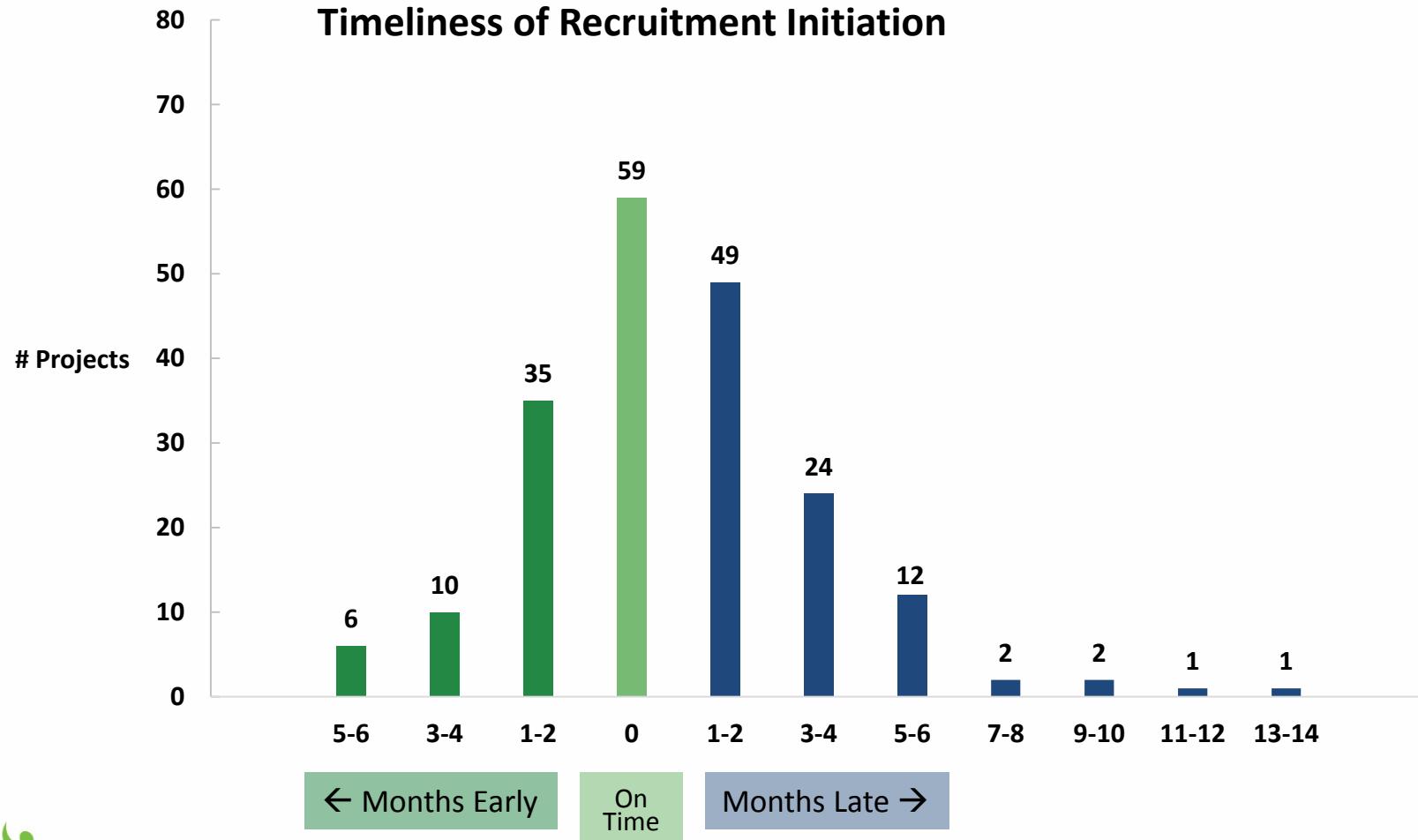
\*Includes currently recruiting, finished recruiting, and not yet recruiting (late)

\*On time = within 15 days of target start date



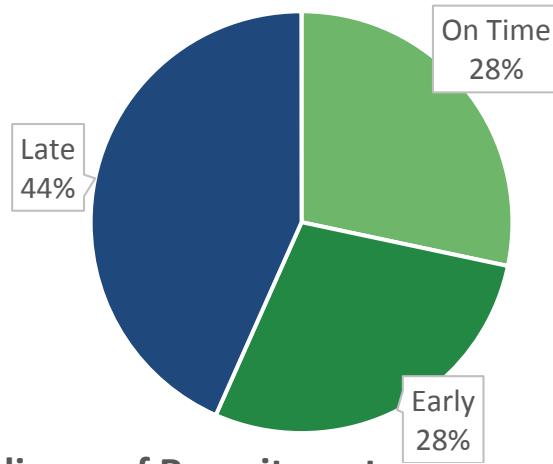
# Recruitment Initiation (N=201)

For all projects that have initiated recruitment, either early, on time, or late

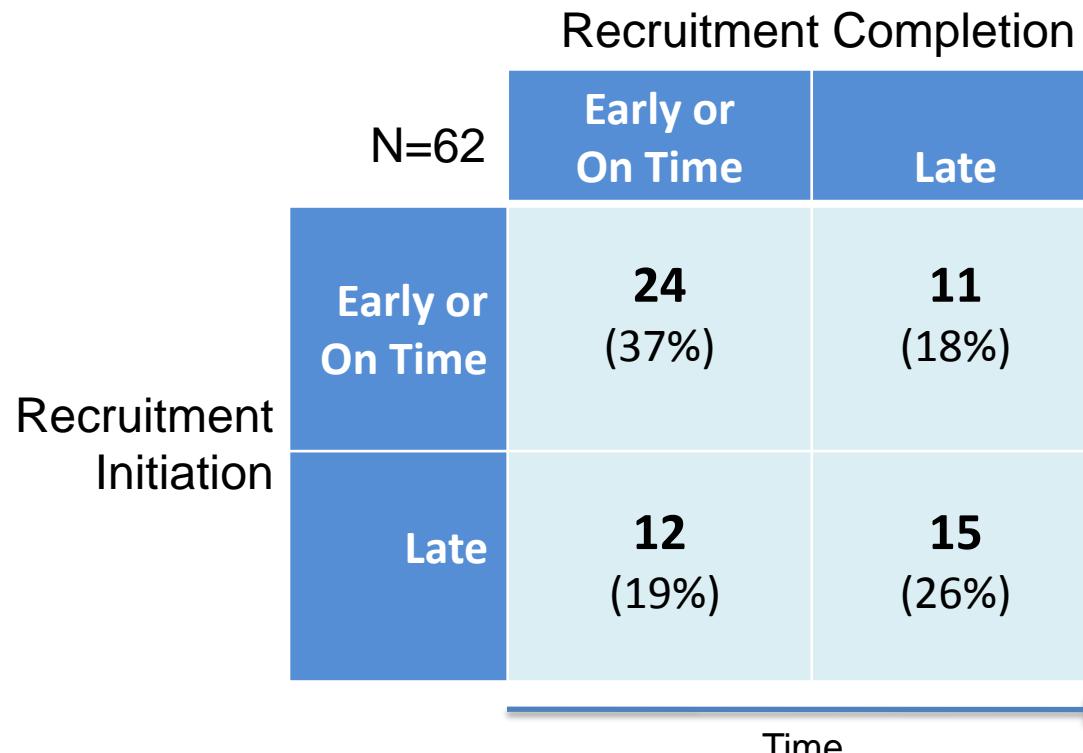
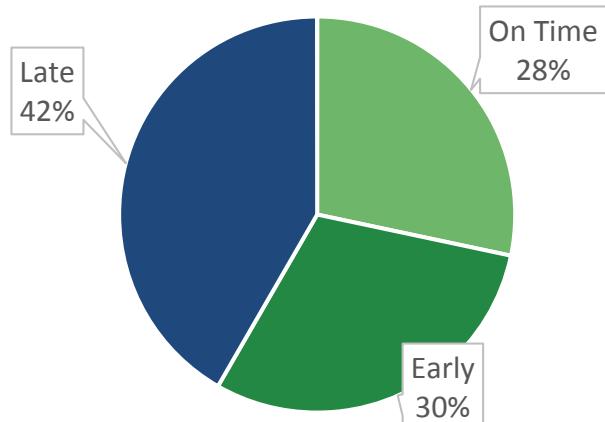


# Completed Recruitment (N=62)

Timeliness of Recruitment Initiation (N=62)



Timeliness of Recruitment Completion (N=62)



- 63% Stay in same timeliness category
- Of those that started late, 44% ended on time
- Of those that started early, 31% ended late



# Discussion

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- Are there other sources of information that we could use for benchmarking?
- How can we use our portfolio data to examine important issues in study recruitment?
- General thoughts or advice on future analyses?



# Break

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10:35 – 10:45 a.m.



# Recruitment, Accrual, and Retention Issues Raised at the 2016 PCORI Annual Meeting

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# PCORI 2016 Annual Meeting Symposium:

## Know Before You Go: Planning Upstream for Successful Recruitment in PCOR and Clinical Trials

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**Cynthia Girman, DrPH**

Ex-Officio CTAP Member from the PCORI Methodology Committee



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# Symposium Overview

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- Planning strategies for recruitment & retention
- Barriers and facilitators of recruitment
- Strategies to assess trial feasibility and sites selection, effective partnerships & communication planning
  - Data driven approaches
  - Effective budgeting of outreach / communication for recruiting
  - Planning risk mitigation strategies upfront
  - Engaging patients and stakeholders early and throughout study
  - Monitoring of recruitment



# Symposium Data-Driven Planning Strategies

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## Tapping Into Electronic Data Sources

- **Eligibility**
  - use of electronic health records and other clinical data sources
  - Anonymized data sources for important background data :
    - Prevalence, incidence and natural history
    - Common comorbidities and concomitant meds
    - Common treatment strategies
    - Profile and patients characteristics for potential comparators
- **Feasibility of site & patient recruitment**
- **Mitigation Planning** – contingency planning for common shortfalls in site initiation & patient recruitment



# Speaker Recommendations

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## “Sweat Equity” – some Do’s and Don’ts

- **DO**

- Spend time at recruitment site(s)
- Budget for community/patient partners, including patient coordinators within the medical community
- Multiple communication methods (on-site meetings, phone, text, email, shared files)
- Value patient contributions to recruitment, planning, and monitoring

- **DO NOT**

- Send minions to do on-site work
- Rely on fliers only
- Neglect community partners





# Speaker Recommendations

## Awareness, Trust, Risk as 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
- Lack of trust
- Too risky
- Adverse health outcomes
- Little or no monetary compensation
- Privacy issues
- Too much time
- Not sure

53%  
53%  
51%  
44%  
35%  
27%  
27%  
11%

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



# Speaker Recommendations

## Capturing the Patient Perspective: 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”

# Participant Recommendations to PCORI

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- Provide guidance or best practices related to recruitment
- Establish a forum for PCORI investigators to share ‘real-time’ best practices within each other
- Develop centralized, data-driven resources for investigators



# **Challenges and Best Practices for Communication, Patient Recruitment, and Site Management: Program Manager Session 2016 PCORI Annual Meeting**

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**Allison Ambrosio, MPH**

Program Associate, Clinical Effectiveness and Decision Science



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# Session Description

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- Primary aim: Highlight systems or processes used by PCORI-funded studies to successfully begin and manage their clinical trials
- Provide a forum for study managers to identify and distinguish best practices among studies
  - Can learn from each other and perhaps apply to their own work
- PCORI neither endorsed nor recommended practices; session was simply a platform for study managers to share
- Session divided into 2 parts:
  - Enhancing Site and Investigator Communications
  - Fishbowl Discussions on Patient Recruitment and Site Management
- 20 studies from Pragmatic Clinical Studies portfolio were represented



# Program Manager Shared Lessons: Site and Investigator Communications

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- Discussion of Initiation and Timing of Communication to Sites & PIs
  - When to begin?
  - How much is too much?
  - What should be included?
  - Maintaining confidentiality and HIPAA compliance
- Potential solutions discussed
  - Quarterly and Monthly Newsletters
  - Patient Portal Websites
  - Site Teleconferences
  - Site Visits and Audits



# Program Manager Session Shared Lessons: Site Management Suggestions

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- Share best practices and efficiencies both within and across sites
- Consider non-financial incentives to promote site recruitment and retention
- Maintain appropriate PI oversight across all sites
- Consider different strategies and solutions to working with IRBs in ensuring timely site activation



# Program Manager Session Shared Lessons: Patient Recruitment

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- Consider creative ways to use social media or websites to recruit patients and/or maintain adherence to study protocol
- Shared methods to recruit specific subpopulations of interest/those populations that are hard to reach
- Shared ways of managing and working to remediate sites that are performing poorly in terms of lagging recruitment, poor patient retention, lack of protocol adherence, etc.



# Program Manager Session: Recommendations of Next Steps

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- Great discussion and enthusiasm among study managers
  - Sites are eager to cull best practices in site communications, patient recruitment, and site management
- Can PCORI establish a more regular forum for discussion to prevent & mitigate study risks?

## Examples included

- Sharing of e-mails or a listserv of PCORI study managers
- Developing a monthly teleconference or newsletter



## Panel Discussion & Advice

# Best Practice Development in Recruitment, Accrual, and Retention

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**Cynthia Girman, DrPH**

Ex-Officio CTAP Member from the PCORI Methodology Committee



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# Opportunities for CTAP Input

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- Suggestions of evidence-based best practices to share
- Value of an information-sharing forum & caveats about contents
- Review or oversight needed for PCORI to offer guidance about best practices
- Priority topics to address in RAR for comparative effectiveness
  - Unique challenges
  - Preparatory data about population, care processes, clinical flow, etc.
  - Communication practices
  - Problem-solving specific challenges (recruitment and retention)



# Proposed Next Steps for CTAP

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- RAR Subcommittee be re-formulated to address these questions and report back to CTAP
- Goals:
  - Advise PCORI on best processes to collect and vet best practices among its awardee community
  - Assist in the development of PCORI guidance, rubric, handbook of tips, or other published resource guide on RAR in CER
  - Product above to be developed over the next year (by April 2018)
- Action item
  - Solicit volunteers from CTAP and recommendations of others to include in subcommittee



# Recognition of Panelists

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**Anne Trontell, MD, MPH**

Associate Director, Clinical Effectiveness Research,  
PCORI



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# Lunch

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11:45 a.m. – 12:45 p.m.



# Board of Governors Recommendations on Priorities of Clinical Trial Monitoring

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**Evelyn P. Whitlock, MD, MPH**

Chief Science Officer, PCORI



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# 2016 Achievements

## Total Number of LOIs Submitted



## Number of Applications Submitted



Budgeted **\$390M**; Funded **\$293M**

\*PFAs are awarded the FY after they are posted.

Posted **18** Funding Announcements

Posted **6** Targeted Funding Announcements



Back Pain



Opioid Misuse:  
Prevention



Palliative Care



Sickle Cell



MS (Reposted)

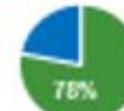


Opioid Use  
(Reposted)

Posted **2** PCS Funding Announcements



Areas of Special Emphasis



**78%** of research projects in  
the green zone



Of the projects recruiting, **60%** were  
meeting all recruitment milestones



Launched **peer review & research synthesis programs**



**33** draft final research reports  
submitted



**166** articles by awardees



# PCORI's Cluster Designed Trials

**David Hickam, MD, MPH**

Program Director, Clinical Effectiveness and Decision Science, PCORI



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# Terminology

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- **Cluster Design:** Comparison of groups of patients. Group membership is defined by location or identity of care providers.
- **Cluster Randomized Trial:** Patients are randomized in groups (usually on basis of clinic or provider), but data are analyzed at the level of the individual patients.
- **Stepped Wedge Design:** A new clinical approach is introduced for clinics or providers in a systematic way over a period of time. Randomization can be used to define the time that a particular clinic crosses over to the new approach. Patients are assigned to groups based on when they are seen in the clinic.



# Characteristics of the Portfolio

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- 47 projects using cluster design have been funded through March 2017.
  - 46 are randomized trials.
  - 1 natural experiment
- Distribution among PCORI National Research Priorities:
  - Improving Healthcare Systems: 20 projects (43%)
  - Addressing Disparities: 8 Projects
  - Communication and Dissemination Research: 8 projects
  - Assessment of Prevention, Diagnosis and Treatment Options: 9 projects
  - Improving Methods for CER: 2 projects



# Target Enrollment of Patient Participants

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- 9% sample size  $\leq 300$
- 23% sample size 301–700
- 23% sample size 701–1200
- 20% sample size 1201–2000
- 25% sample size  $>2000$



# Recruitment and Retention in Cluster Trials

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- 18 cluster RCTs have launched since early 2014.
- 11 (61%) had reached recruitment and retention goals through mid-2016.
  - 4 (22%) had not reached the recruitment target.
  - 3 (17%) had retention problems.



# Types of Interventions in the Cluster RCTs

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- Complex interventions involving patient or provider behaviors
  - Implementation of educational programs for providers and/or patients
  - Decision Making Tools
    - Choice of mental health services
    - Advance care planning
- Organizational re-design
  - Clinic-based care vs. telecare
  - Re-organization of care within primary care or specialty clinics
  - Transitional care programs
- Specific clinical services
  - Referral to ancillary services for patients with acute pain
  - Schedule of surveillance for pulmonary nodules
  - Counseling patients to improve risk factors
  - Scope of practice for emergency care providers



# Methodological Challenges

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- Number of clusters: some projects have fewer than 10 clusters per arm.
- Achieving balance of cluster size: considerable variation in the number of patients enrolled within individual clusters.
- Monitoring receipt of services within the study arms: challenges of measuring adherence for complex interventions.



# Lessons Learned

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- High level of interest in cluster designs among PCORI applicants.
- Many examples of interventions that have the potential to improve quality of care and are well suited to cluster designs.
- It is likely that PCORI will continue to receive proposals for new projects using cluster designs.



# Implications of Changes to the Common Rule

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**John D. Lantos, MD**

Co-Chair, Advisory Panel on Clinical Trials, PCORI



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# Revised Common Rule

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- Informed consent must begin with a concise and focused presentation of the key information that is most likely to assist a prospective subject or legally authorized representative in understanding the reasons why one might or might not want to participate in the research. This part of the informed consent must be organized and presented in a way that facilitates comprehension.
- Informed consent as a whole must present information in sufficient detail relating to the research, and must be organized and presented in a way that does not merely provide lists of isolated facts, but rather facilitates the prospective subject's or legally authorized representative's understanding of the reasons why one might or might not want to participate.
- <http://www.nejm.org/doi/full/10.1056/NEJMp1700736>



# Protocol Guidance for Awardees

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**Harold Sox, MD**

Program Director, Peer Review, PCORI



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# References or Benchmarks to Use as Guidance for Study Protocols

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- Goal: When questions arise, what can serve as a reference example of the elements to include in a clinical study protocol?



# Background on SPIRIT

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- The 2013 SPIRIT Statement was written as a guideline for the minimum content of a clinical trial protocol.
- SPIRIT group included leading clinical epidemiologists/statisticians
- Process of developing SPIRIT
  - Consultation with 115 stakeholders
  - 2 systematic reviews of existing protocols
  - Delphi consensus process
  - 2 face-to-face meetings
  - Pilot testing
- Publication in Annals of Internal Medicine (509 citations in 4 years)



# References or Benchmarks to Use as Guidance for Study Protocols

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- Does SPIRIT offer a good basis for the fundamental components of a study protocol?
  - If not, what others should be referenced?
  - If yes, are there key components that PCORI should call out specifically for inclusion in protocol submissions?
- What else does CTAP recommend about the content of submitted protocols?



# Break

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2:30 – 2:45 p.m.



# Open Science Update

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**Jason Gerson, PhD**

Senior Program Officer, Clinical Effectiveness and Decision Science, PCORI



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# Overview

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- Public Posting of Studies and Findings
- Draft Data Access and Data Sharing Policy
- Data Sharing Pilot Project
- Discussion



# Public Posting of Studies & Findings

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- PCORI-funded studies are required to be registered on ClinicalTrials.gov and information is available as well on the PCORI website
- Research findings are also made available to public via:
  - Results tables available in ClinicalTrials.gov
  - Research findings available on PCORI's website, including the technical and lay abstracts
  - Final research reports will be published on PCORI website no more than one year after approval and acceptance by PCORI.
- PCORI's 'Public Access to Journal Articles Presenting Findings from PCORI-Funded Research Policy'
  - Final peer-reviewed journal manuscripts from PCORI-funded research must be deposited in PubMed Central
  - PCORI funding is available to provide free public access to peer-reviewed journal articles



# Draft Data Access and Data Sharing Policy

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- Set forth expectations and guidelines for PCORI Research Awardees for management of their data in order to:
  - Promote data sharing to enable conduct of additional analyses using data from PCORI-funded studies, thereby augmenting the knowledge generated from the original study.
  - Facilitate reproduction of original analyses to increase the integrity of PCORI-funded research findings.
- Highlights of draft policy include:
  - PCORI funding to support deposition of data and data documentation (study protocol, metadata, analytic code) for studies funded through Pragmatic Clinical Studies (PCS) and Targeted Funding Announcement mechanisms.
  - Requirement for all other studies to prepare data for data sharing (with funding provided by PCORI if a need for data sharing arises).
  - Requirement to maintain data in repository for minimum of seven (7) years.
- Drafted in a manner that will enable PCORI to incorporate additional operational details and procedures over time.



# Update on Draft Policy: Public Comment

- The draft policy was posted for public comment November 1-January 23. Thirty-two comments were received and posted. NIH submitted comments/questions to PCORI staff prior to the public comment period.

Community	Number of responses/notable respondents
<b>Stakeholder</b> <ul style="list-style-type: none"><li><b>Health Researcher</b></li><li><b>Hospital &amp; Health Systems</b></li><li><b>Other</b></li><li><b>Clinician</b></li></ul>	17 (YODA, Breast Cancer Surveillance Consortium, UNC, Duke, CHOP, University of Michigan, UPMC, Memorial Sloan-Kettering) 5 (Geisinger, Group Health, Kaiser Permanente) 3 (AMIA, AAMC) 2
<b>Patient</b> <ul style="list-style-type: none"><li><b>Advocacy Organization</b></li><li><b>Patient</b></li><li><b>Caregiver</b></li><li><b>Consumer</b></li></ul>	2 (National MS Society) 1 1 1

- A table summarizing the public comments has been provided for your review. It is organized by policy feature (e.g. data retention period), whether the current policy addresses that feature, a summary of comments received about that feature, as well as a tentative recommendation/next steps concerning that feature.



# Data Sharing Pilot Project

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- “Learning by doing” approach to inform PCORI’s implementation and details of the policy:
  - Obtaining information through experience from leaders in the field on repositories, data issues, technological issues, cost, governance
  - Demonstrating ability to progress on open science through motivated repositories and awardees (both institutions and PIs)
- Questions Pilot Project Will Help Address:
  - What are key characteristics of a good repository for hosting clinical trial data?
  - What factors contribute to successful data sharing by awardees and why?
  - What are the key constraints/impediments to sharing data by awardees and how can they be eliminated?
  - What are costs of cleaning, editing, curating data?
  - What are costs of maintaining data for potential future sharing?
  - What are costs of depositing data in an existing repository?



## Wrap Up and Next Steps

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### **Elizabeth A. Stuart, PhD, AM (Chair)**

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

### **John D. Lantos, MD (Co-Chair)**

Professor of Pediatrics, Children's Mercy Hospital

### **Anne Trontell, MD, MPH**

Associate Director, Clinical Effectiveness and Decision Science, PCORI



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# Thank You!



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