Board of Governors Meeting
via Teleconference/Webinar

March 22, 2016

12:00-1:30 p.m. ET

pcori®

PATIENT-CENTERED OUTCOMES RESEARCH INSTITUTE

Welcome and Introductions

Larry Becker
Chair, Finance and Administration Committee
Acting Chairperson

Joe Selby, MD, MPH
Executive Director

Agenda

Time	Agenda Item
12:00	Call to Order, Roll Call, and Welcome
12:00 – 12:05	**Consider for Approval:** Minutes of the February 23, 2016 Board Meeting
12:05 – 12:30	**Consider for Approval:** PCORnet Patient-Powered Research Networks (PPRN) Demonstration Projects
12:30 – 12:55	**Consider for Approval:** Management of Chronic Low Back Pain Targeted PCORI Funding Announcement (PFA)
12:55 – 1:20	Review of Q1 FY-2016 Dashboard
1:20 – 1:25	Re-Release of the Hepatitis C Targeted PFA
1:25	Wrap up and Adjournment

Welcome to PCORI's Newest Board Member

- We are honored to welcome Sharon Arnold, PhD, as PCORI's newest board member
- Dr. Arnold currently serves as Acting Director of the Agency for Healthcare Research and Quality (AHRQ)

Board Vote

Call for a Motion to:	• **Approve** the minutes of the February 23, 2016 Board of Governors 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

Consider for Approval: PCORnet Patient-Powered Research Networks (PPRN) Demonstration Projects

Joe Selby, MD, MPH
Executive Director, PCORI

Project Development

- Patient-powered research networks (PPRNs) were funded by PCORI with the intent of supporting communities or networks of patients motivated to participate in clinical research through the National Patient-Centered Clinical Research Network (PCORnet) and to develop their capacity to govern the research activities of their networks

- As PCORnet moved into Phase II, an opportunity emerged for the PPRNs to bring participants' voices to nationwide clinical research and develop true participant partnerships

- PCORI sought to fund PPRN-initiated research based on questions that have been generated and prioritized by participants within the PPRN community

Merit Review Criteria

The following criteria were used to evaluate the submitted applications:

- **Potential for study to improve health care and outcomes**
  - Potential to lead to meaningful improvement in the quality and efficiency of care and to improvements in outcomes important to the PPRN participant community
- **Technical merit**
  - Sufficient technical merit in the research design to ensure that the study goals will be met
- **Patient-centeredness**
  - Demonstrates patient-centeredness at every stage of the research
- **Patient and stakeholder engagement**
  - Demonstrates that people representing the population of interest and other relevant stakeholders are leading in ways that are appropriate and partnering with other relevant stakeholders where necessary in a given research context

Application Review: Process Overview

- PCORI received 15 Letters of Intent (LOI)
- 10 were asked to submit a full application (67% of all LOIs)
- We are proposing to fund 5 out of 10 responsive applications (50%)

Slate Summary

*5 Recommended Projects**

Project Title	Total Budget
Comparative effectiveness of specific carbohydrate and Mediterranean diets to induce remission in patients with Crohn's disease	$2.50M
Resiliency Education to Reduce Depression Disparities	$2.50M
Harnessing PCORnet to Study Comparative Effectiveness and Safety of Biologic Therapies	$2.50M
Healthy Hearts Healthy Minds: A PPRN Demonstration Pragmatic Trial	$2.50M
Monitoring and Peer Support to Improve Treatment Adherence and Outcomes in Patients with Overlap Chronic Obstructive Pulmonary Disease and Sleep Apnea via a Large PCORnet Collaboration	$2.48M

**All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*

Proposed Collaborations

Network	Project 1	Project 2	Project 3	Project 4	Project 5
PPRN 1	•		•		
PPRN 2		•			
PPRN 3				•	
PPRN 4	•		•		
PPRN 5	•		•		
PPRN 6					•
PPRN 7		•			
PPRN 8		•			•
PPRN 9				•	•
PPRN 10			•		
PPRN 11			•		
PPRN 12					•
PPRN 13					•
CDRN 1	•		•		
CDRN 2			•		
CDRN 3					•
Total Collaborations	3 PPRNs	3 PPRNs	5 PPRNs	2 PPRNs	5 PPRNs
	1 CDRN		2 CDRNs		1 CDRN

Contributions to Infrastructure Development

Project	Contributions to the PCORnet Commons
CE of Diets in Crohn's Disease	• Methodology that can be applied to other diseases and diets • Methodology for web-based PRO collection and collection of biosamples by mail.
Depression in LGB Communities	• Principles of research ethics and trust in research with underrepresented communities • Approaches to patient engagement with involving lay health workers in underserved communities
CE of Biologics in Multiple Conditions	• Methods for linking EHR data to claims data and pharmacy data
Increasing Exercise in Adults with Mood Disorders and CVD	• Guidance for integrating passive monitoring data into a shared database across PPRNs • Web-based intervention tools for managing depression and stress and increase physical activity
Adherence for COPD and OSA Patients	• Guidance for online tools to help recruit and enroll new participants

Slate Overview:
*Patient-Powered Research Networks Demonstration Projects**

5
New Projects

PFA	Allotted	Proposed Total Budget**	*Difference*	Average Project Budget**
Patient-Powered Research Networks Demonstration Projects	$18M	$12.5M	***$5.5M***	$2.5M

**All proposed projects, including requested budgets and project periods, are approved subject to a programmatic and budget review by PCORI staff and the negotiation of a formal award contract.*

***Total budget = direct + indirect costs*

Board Vote

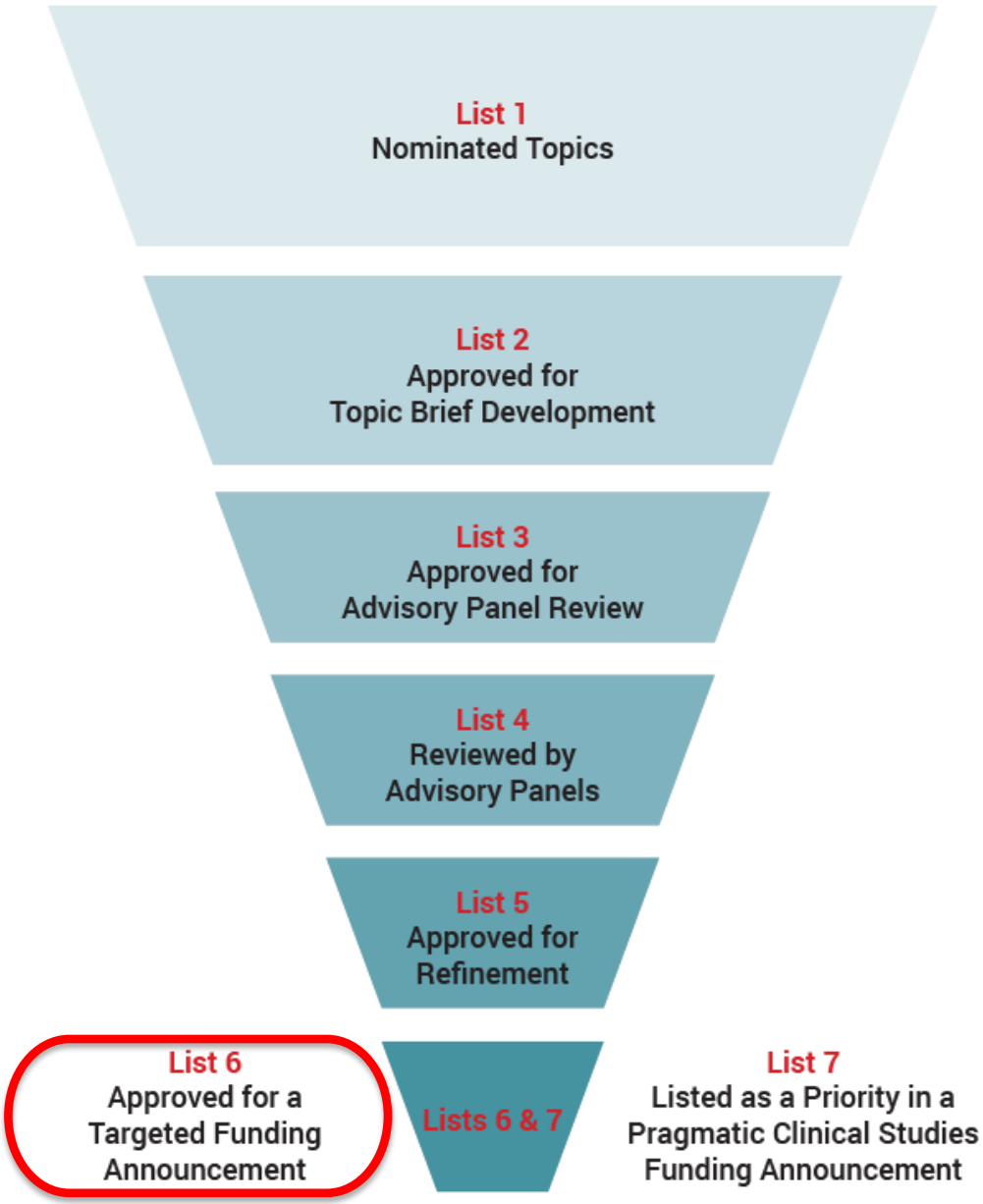
Call for a Motion to:	• **Approve** funding for the recommended slate of awards for the Patient-Powered Research Networks (PPRN) Demonstration Projects
Call for the Motion to Be Seconded:	• **Second** the Motion • If further discussion, may propose an **Amendment** to the Motion or an **Alternative** Motion
Roll Call Vote:	• Vote to **Approve** the **Final** Motion • Ask for votes in favor, opposed, and abstentions

Targeted PCORI Funding Announcement Development: Management of Chronic Low Back Pain

Robert Zwolak, MD, PhD
Science Oversight Committee Chair

Evelyn P. Whitlock, MD, MPH
Chief Science Officer

PCORI Topic Prioritization Pathway



8 Awarded Targeted PFAs to Date

Title	Date Awarded	# of Projects	$ Awarded	Approximate Completion Date
Treatment Options for African Americans and Hispanics/Latinos with Uncontrolled Asthma	December 17, 2013	8	$23	Q2—2017
Treatment Options in Uterine Fibroids (Administered by AHRQ)	September 30, 2014	1	$20	Q4—2019
The Effectiveness of Transitional Care	September 30, 2014	1	$15	Q1—2017
Clinical Trial of a Multifactorial Fall Injury Prevention Strategy in Older Persons (Administered by NIA)	June 4, 2014	1	$30	Q4—2018
Obesity Treatment Options Set in Primary Care for Underserved Populations	September 30, 2014	2	$20	Q2—2018
Optimal Maintenance Aspirin Dose for Patients with Coronary Artery Disease	May 4, 2015	1	$14	Q4—2018
Testing Multi-Level Interventions to Improve Blood Pressure Control in High-risk Populations (Administered by NHBLI)	September 4, 2015	2	$25	Q4—2020
Clinical Management of Hepatitis C Infection	September 28, 2015	2	$39	Q2—2021

4 Approved Targeted PFAs

Title	Expected Award Date	# of Projects	Budget
Treatment-Resistant Depression	Summer 2016	Up to 3	Up to $30M
New Oral Anticoagulants	Summer 2016	Up to 3	Up to $40M
Treatment Strategies for Managing and Reducing Long-Term Opioid Treatment for Chronic Pain	Summer 2016	Up to 2	Up to $30M
Treatment of Multiple Sclerosis	Summer 2016	Up to 8	Up to $50M

Targeted PFA Pending Approval

- For Board Vote Today:
  - Management of Chronic Low Back Pain

Action	Date
Board of Governors Vote	**March 22, 2016**
Release Date	**April 4, 2016**
Letter of Intent Due	**May 4, 2016**
Application Deadline	**August 8, 2016**
Merit Review	**November 14, 2016**
Awards Announced	**January 2017**

Management of Chronic Low Back Pain

Overview

- In 2010, low back pain (LBP) was the third-largest contributor to disability-adjusted life years in the United States. The total costs of back pain exceed $100 billion per year in the United States
- Chronic LBP is defined as low back pain occurring on at least half of the days in a 6-month period
- A large majority of chronic LBP sufferers have non-specific LBP, defined as the absence of neurological symptoms and signs (e.g., leg pain, numbness or weakness in a nerve root pattern)
- LBP has been on PCORI's radar screen for investment for several years, however, this is the first proposed targeted PCORI funding announcement

Development Timeline

Action	Date
Initial Ad Hoc Workgroup Meeting	March 21, 2013
Back Pain Task Force Meeting	August 9, 2013
Advisory Panel	April 17, 2015
Multi-stakeholder Workshop	June 9, 2015
SOC Endorsement	July 7, 2015
Multi-stakeholder Webinar	January 7, 2016
SOC Endorsement	March 1, 2016
Board of Governors Vote	**March 22, 2016**
Release Date	April 4, 2016

Stakeholder Perspectives

- Chronic non-specific low back pain is important to:
  - *Patients and Families:* Up to 84% of adults have LBP at some point in their lives, with 10% becoming chronic, and considerable associated functional impairment
  - *Clinicians*: Back pain is one of the most frequent reasons for adult health care visits. Many different types of clinicians are involved in various phases of LBP management. Clinicians need curative or corrective treatments that can be matched to individual patient needs and preferences
  - *Payers*: Payers have strong interest in this topic due to escalating rates of imaging, electro-diagnostic testing, spinal injections and surgery, often without strong evidence of benefit
  - *Purchasers*: LBP causes more disability globally than any other condition, and in the United States, an estimated 149M work days are lost yearly due to LBP

Stakeholder-Prioritized Research Themes—Currently Funded PCORI Research Projects

- **Methods for Classifying Patients for Treatment** *(Evaluation of a Patient-Centered Risk Stratification Method for Improving Primary Care for Back Pain. Cherkin, 2012-2015, Broad.)*

- **Effectiveness of Treatment Options**
  - To prevent development of Chronic Low Back Pain *(Targeted Interventions to Prevent Chronic Low Back Pain in High Risk Patients: A Multi-Site Pragmatic RCT. DeLitto, 2015-2020, Pragmatic.)*
  - To manage types of Acute, Subacute, and Chronic Low Back Pain *(Long-term Outcomes of Lumbar Epidural Steroid Injections for Spinal Stenosis. Friedly, 2013-2016, Broad; Acupuncture Approaches to Decrease Disparities in Outcomes of Pain Treatment- A Two Arm Comparative Effectiveness Trial. McKee, 2014-2017, Broad; Comparative Effectiveness of Postoperative Management for Degenerative Spinal Conditions. Archer, 2013-2016, Broad.)*
  - To compare multidisciplinary with single treatment approaches in those with Chronic Low Back Pain
  - To predict responders to treatment approaches, particularly surgery
- **Strategies for Engaging Patients in Care** *(Measuring Patient Outcomes from High Tech Diagnostic Imaging Studies. Solberg, 2012-2015, Pilot; Comparing Engagement Techniques for Incorporating Patient Input in Research Prioritization. Lavallee, 2014-2016, Broad; Evaluating Methods to Engage Minority Patients and Caregivers as Stakeholders. Evaluating Methods to Engage Minority Patients and Caregivers as Stakeholders. Turner, 2013-2015, Broad.)*
- Relapse Prevention and Self-Management
- Measurement of Patient-Important Outcomes
- Healthcare Systems
- **Education of Health Care Providers to Improve Knowledge and Practice** *(Promoting Patient-Centered Counseling to Reduce Inappropriate Diagnostic Tests. Fenton, 2012-2014, Pilot.*
  Fenton JJ, Kravitz RL, Jerant A, Paterniti DA, Bang H, Williams D, Epstein RM, Franks P. Promoting Patient-Centered Counseling to Reduce Use of Low-Value Diagnostic Tests: A Randomized Clinical Trial. *JAMA Intern Med*. 2015 Dec 7:1-7)

Evidence Gaps/Need for Further Research

- Despite published clinical guidelines, the management of LBP appears to increasingly be discordant with recommended care, with potential for quality and cost improvement (Mafi JN, McCarthy EP, Davis RB, Landon BE. Worsening Trends in the Management and Treatment of Back Pain. *JAMA Intern Med*. 2013;173(17):1573-1581)

- Despite weak evidence for effectiveness, lumbar fusion surgery increased 2.4-fold from 174,223 to 413,171 discharges from 1998 to 2008, while the national bill for this surgery increased 7.9-fold (Rajaee SS, Bae HW, Kanim LE, Delamarter RB. Spinal fusing in the United States: analysis of trends from 1998 to 2008. *Spine* (Phila Pa 1976). 2012 Jan 1;37(1):67-76)
  - 90% of lumbar vertebral fusion surgery is done for chronic non-specific LBP. Payers are very interested in assessing its value vs. other interventions
  - Of the 164 randomized studies listed as ongoing in ClinicalTrials.gov, only 5 had target enrollments of 500 or greater and none had greater than 1000 enrollees
  - None of the 5 large studies addressed the effect of surgery for chronic non-specific LBP
  - Studies of longer-term outcomes (beyond 12 months) and that address heterogeneity of treatment effect are missing from the current evidence base, and most strongly needed
- This is an area of very high priority to fill, but could represent the first in several targeted PFAs in this clinical area

Proposed Research Question

- **Research Question:** What is the comparative effectiveness of lumbar fusion surgery vs. an optimized multi-disciplinary non-surgical rehabilitation program for chronic LBP?
- **Population:** Adults with chronic non-specific LBP (no neurological symptoms or structural abnormalities other than disc degeneration) on at least 50% of days during the past six months despite self-care, physical therapy, muscle relaxants, NSAIDS, etc., who have already failed some non-surgical alternatives and remain troubled by disability/pain; must be considered surgical candidates
- **Interventions:** 1) Referral to back surgery center for consideration of surgery; 2) Referral to comprehensive multi-component non-surgical care
- **Outcomes:** Primary: PROMIS measures, Legacy measures (Oswestry, Roland Morris Disability Questionnaire (RMDQ)), outcomes based on recommendations of NIH Low Back Pain Task Force (function, pain, sleep, mood, medication use, productivity, use of opioids); Secondary: care utilization (emergency room visits, surgery, hospital admissions), safety (major complications of treatment, infections)

Proposed Research Question (cont.)

- **Study Design:** Large pragmatic randomized controlled trial comparing optimal clinical strategies that do and do not include spinal fusion surgery
- **Setting:** Participants would be recruited in primary care settings
- **Time:** Follow-up for primary end points for 2 years
- **Research Commitment:** 1-2 studies, up to $22M (total costs)

Proposed Timeline

Action	Date
Board of Governors Vote	March 22, 2016
Release Date	April 4, 2016
Letter of Intent Due	May 4, 2016
Application Deadline	August 8, 2016
Merit Review	November 14, 2016
Awards Announced	January 2017

Board Vote

Call for a Motion to:	• **Approve** $22M (total costs) for the Management of Chronic Low Back Pain targeted PFA development
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

Dashboard Review
First Quarter of FY-2016

Joe Selby, MD, MPH
Executive Director

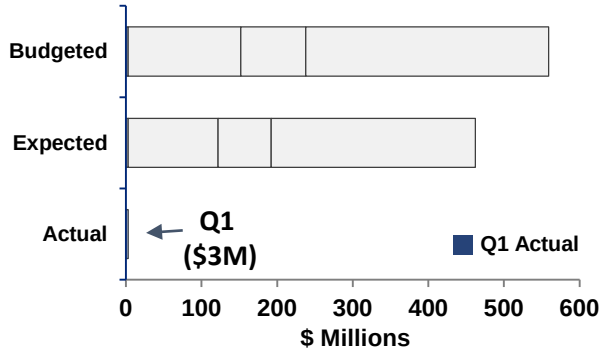
Michele Orza, ScD
Senior Advisor to the Executive Director

Board of Governors FY-2016 Dashboard
Q1 (As of 12/31/2015)
Our Goals: Increase Information, Speed Implementation, and Influence Research

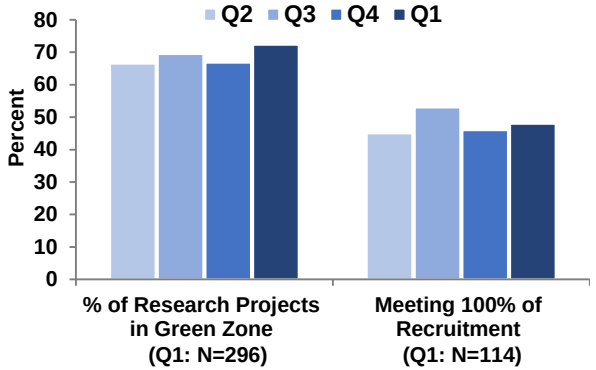
On Target
Off Target
Needs Attention
Projected/Target
Q2 2015
Q3 2015
Q4 2015
Q1 2016

Inputs
Process
Outputs
Uptake
Use
Impact

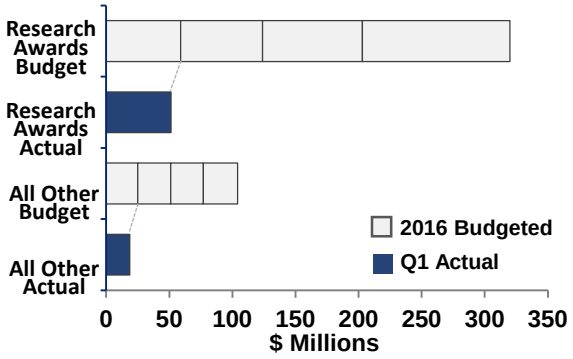
Funds Committed to Research, Up to $554M



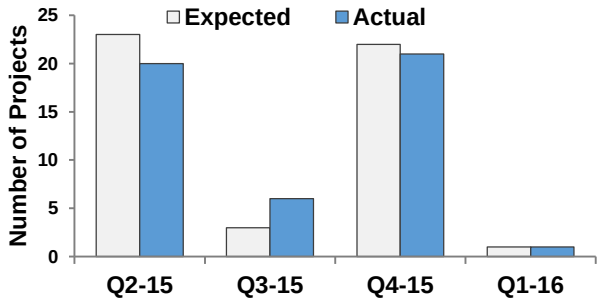
% of Research Projects on Track



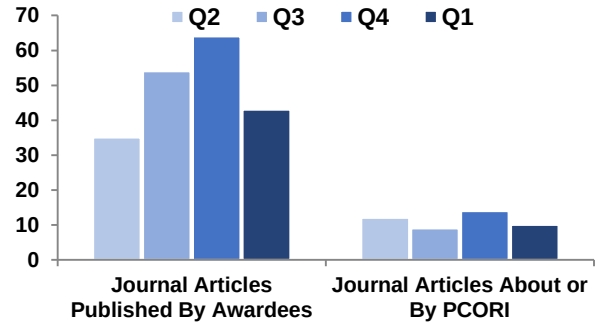
Expenditures – Total Budget, Up to $424M



Final Progress Reports Submitted

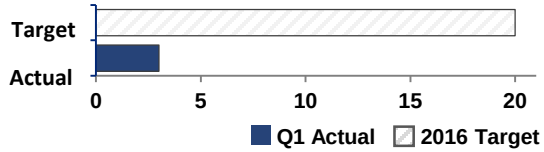


Journal Articles

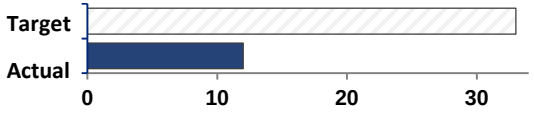


PCORnet Phase II

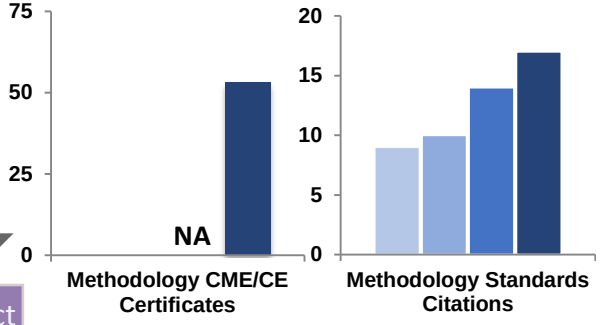
Research Projects Underway in PCORnet



Networks Engaged in Research Projects



Methodology Standards Uptake



Results: Increasing Information

A study aiming to improve patient-centeredness and reduce ordering of low-value tests found that physician education alone may not be sufficient to induce lasting changes in behavior.

Results: Increasing Information

A study on chronic opioid therapy found greater reductions in high-dose prescriptions in group practice settings, which had additional physician initiatives, than among contracted physicians.

Results of Engagement in Research

In this study, **engagement of patients led to the design of a peer-driven intervention for sleep apnea treatment:** a Peer-Buddy approach, which is being compared to standard care. Experienced patients are helping those newly diagnosed with sleep apnea learn to use a challenging but effective treatment. If successful, the Peer-Buddy approach may be a useful tool for treatment of other chronic diseases, such as diabetes, heart failure, and HIV.

Goal 1 Results: Health Plan Initiative to Mitigate Chronic Opioid Therapy Risks

Von Korff M, Dublin S, et al. **The Impact of Opioid Risk Reduction Initiatives on High-Dose Opioid Prescribing for Patients on Chronic Opioid Therapy**. J Pain, Jan 2016. Epub Oct 2015.

- Awarded 2013, Improving Healthcare Systems Project
- Principal Investigator: Michael Von Korff, ScD, Group Health Research Institute

This observational cohort study evaluated a healthcare system initiative to reduce risks of long-term opioid use, **comparing its group practice physicians with its contracted physicians**.

Group practice physicians were exposed to the health plan's multi-part initiative to reduce high-dose chronic opioid therapy by changing physician expectations regarding appropriate prescribing. Contracted physicians were exposed only to statewide guidelines and legislation.

Reductions in prescribing of high opioid dose, average daily dose, and excess opioid days supplied **were substantially greater among group practice physicians** exposed to additional initiatives to alter shared physician expectations, compared with the contracted physicians.

Goal 1 Results: Reducing Low-Value Test Ordering in Primary Care

Fenton JJ, Kravits, et al. **Promoting Patient-Centered Counseling to Reduce Use of Low-Value Diagnostic Tests: A Randomized Clinical Trial**. JAMA Internal, Feb 2016.

- Awarded 2012, Pilot Project
- Principal Investigator: Joshua Fenton, MD MPH, University of California, Davis

Randomized clinical trial to evaluate the effectiveness of a standardized patient-based intervention to **enhance patient-centeredness and skill in handling patient requests for low-value diagnostic tests** among primary care residents.

Residents received either a standard e-mail containing relevant clinical guidelines, or personalized feedback and education about patient-centered techniques to address patient concerns. The primary outcome was whether residents ordered low-value tests in 3 unannounced standardized patient follow-up visits.

The educational intervention did not improve patient-centeredness or rates of low-value test ordering. **Education alone may not be sufficient to induce lasting changes in test ordering behavior.**

In the top 5% of all research outputs scored by Altmetric

Results of Engagement in Research: Design of a Peer-Driven Intervention for Treatment of Sleep Apnea

PCORI Study: **Peer-Driven Intervention as an Alternative Model of Care Delivery and Coordination for Sleep Apnea**

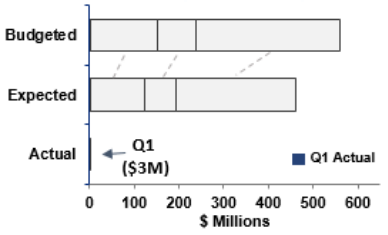
- Awarded 2013, Improving Healthcare Systems project
- Principal Investigator: Sairam Parthasarathy, MD, University of Arizona

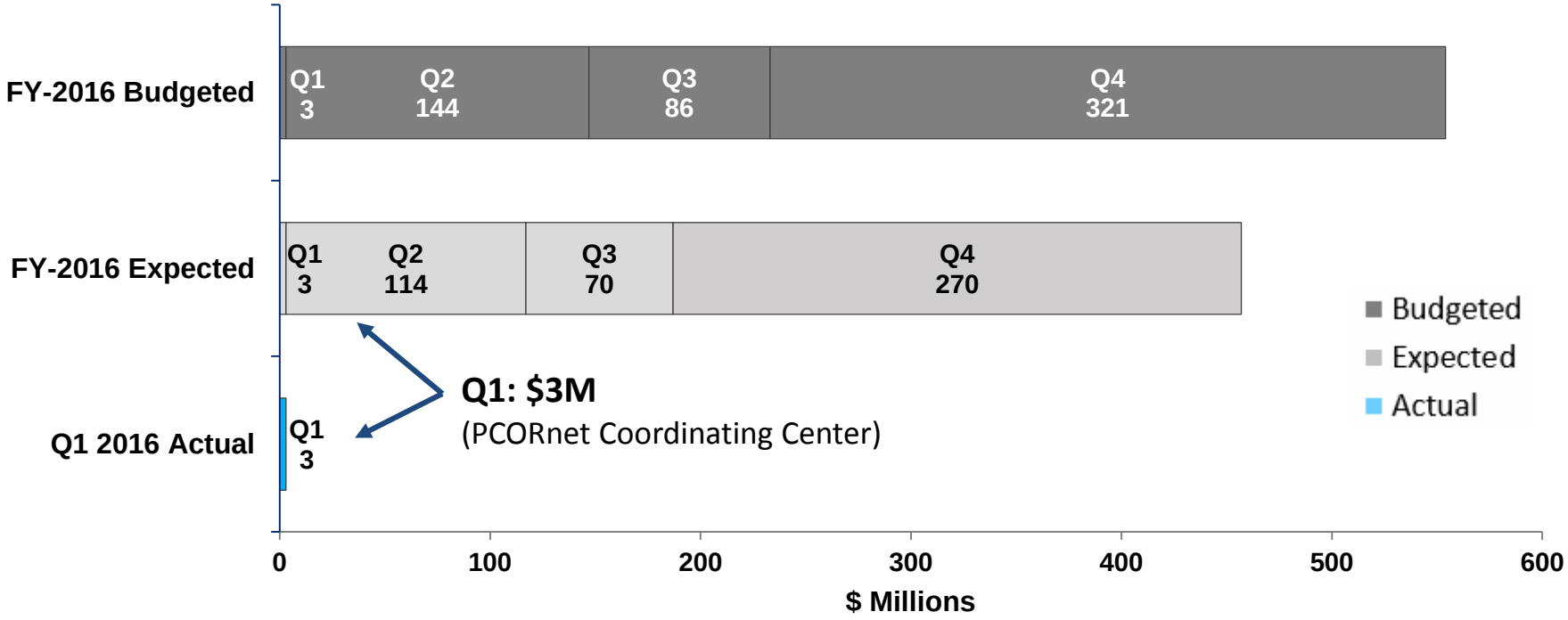
Engagement of patients led to the design of a peer-driven intervention for treatment of sleep apnea: a Peer-Buddy approach, which is being compared to standard care. In the intervention, experienced patients are trained as mentors to help others newly diagnosed with sleep apnea learn to use the challenging but effective treatment, continuous positive airway pressure (CPAP)**.**

The original idea came from a patient that had success with CPAP, and offered to help other patients struggling with treatment. Further engagement with patients also brought other ideas to use, including a planner to help patients track appointments and a laminated contact list attached to the CPAP machine.

If successful, the Peer-Buddy approach may be a useful tool for treatment of other chronic diseases, such as diabetes, heart failure, and HIV.

Funds Committed by Quarter
FY-2016 Budget: Up to $554M





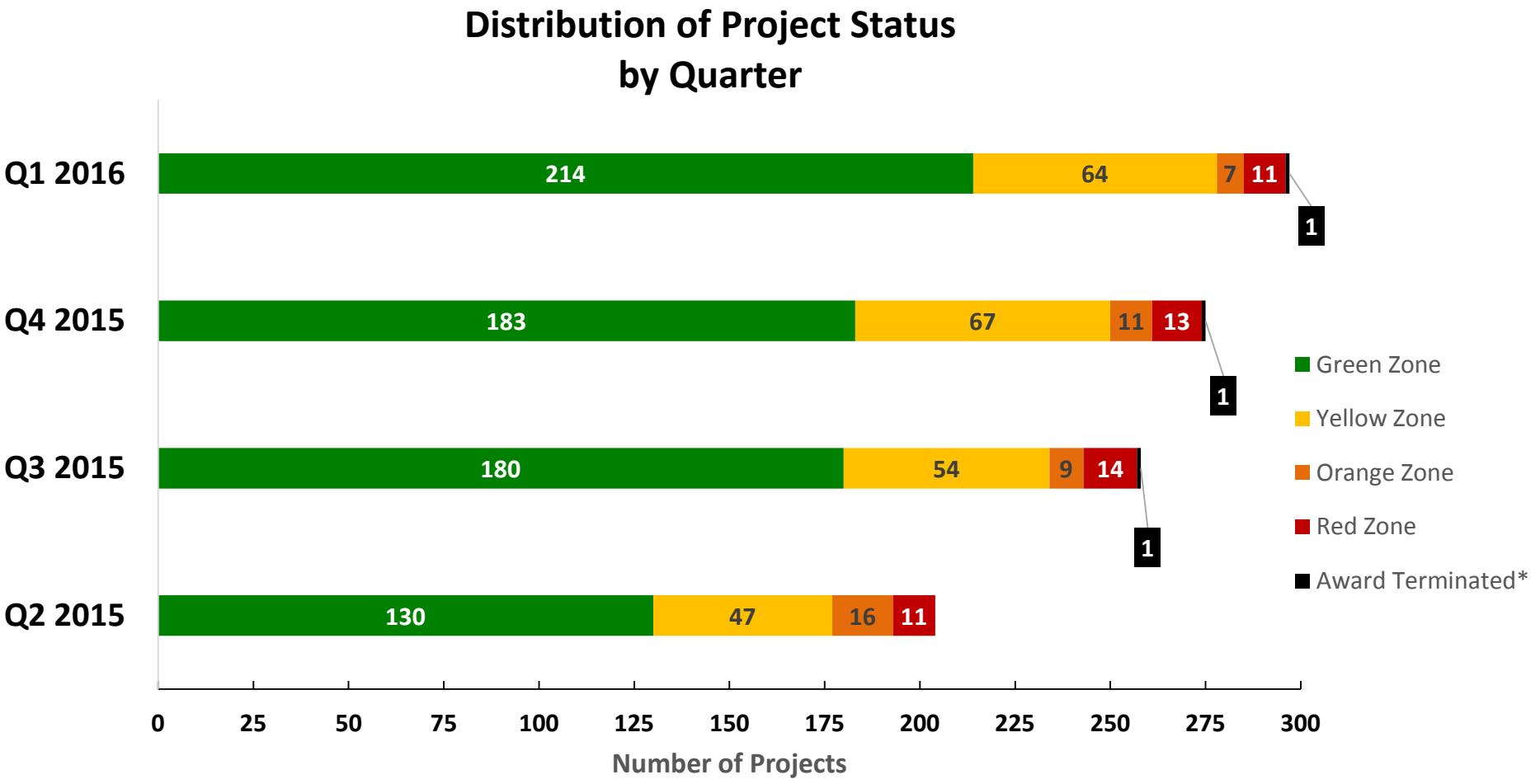
Expected bar: Based on historical averages for each type of PFA

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	**Does not meet Green criteria**	**Does not meet Yellow criteria**	**Does not meet Orange criteria**
≥ 75% of Recruitment	**<75% and ≥50% Recruitment**	**<50% of Recruitment**	**<50% Recruitment, persistently**
-AND-			
≥ 85% Milestones on Time	**<85% and ≥65% Milestones**	**<65% and ≥50% Milestones**	**<50% All Milestones**
-AND-			
PO has confidence in project.	PO has some concerns about project.	PO has serious concerns; Modifications to the Milestone Schedule and/or project plan are likely required.	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

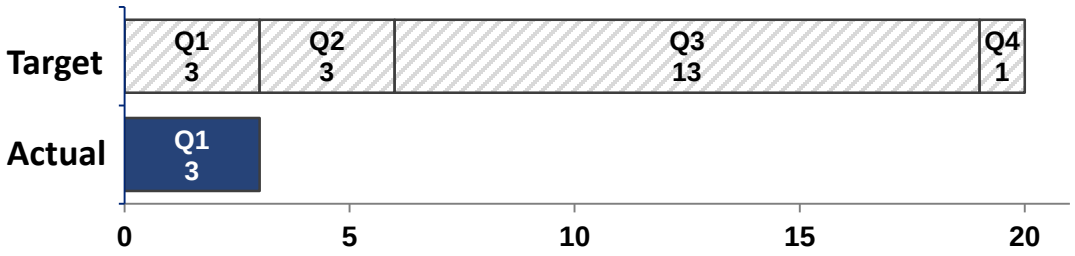
The majority of our projects are on track and we are giving additional attention to those that are not



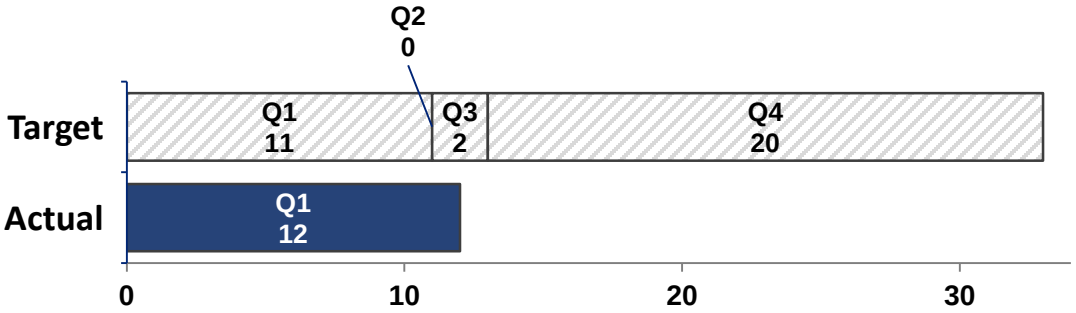
*Notice of Termination Issued

Progress of PCORnet Phase II

Research Projects Underway in PCORnet



Networks Engaged in Research Projects



FY-2016 Targets by Quarter

Q1 Actual

***Projects:** Numbers in Quarter 1 represent the 3 PCORI-funded Demonstration Projects (ADAPTABLE) and two Obesity Studies. In future quarters, total projects will also include projects funded by others.

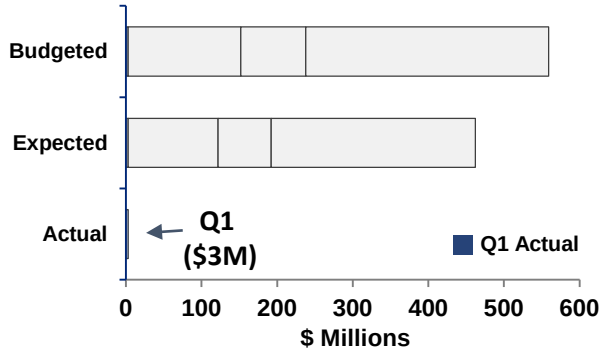
***Networks:** Some networks will be involved in multiple projects. Future metrics will track this.

Board of Governors FY-2016 Dashboard
Q1 (As of 12/31/2015)
Our Goals: Increase Information, Speed Implementation, and Influence Research

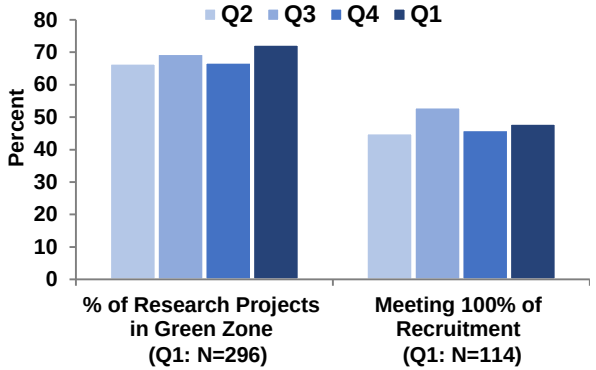
On Target
Off Target
Needs Attention
Projected/Target
Q2 2015
Q3 2015
Q4 2015
Q1 2016

Inputs
Process
Outputs
Uptake
Use
Impact

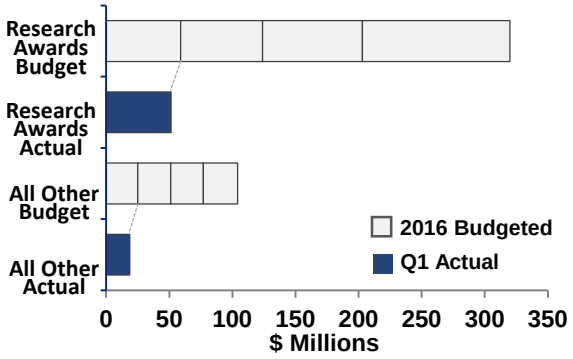
Funds Committed to Research, Up to $554M



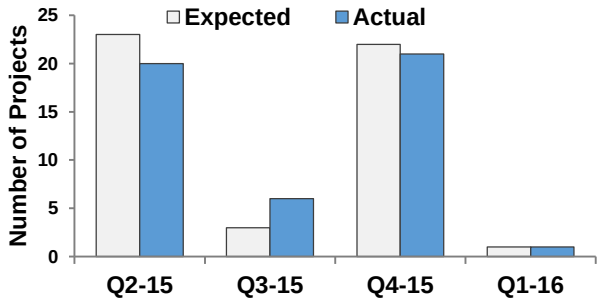
% of Research Projects on Track



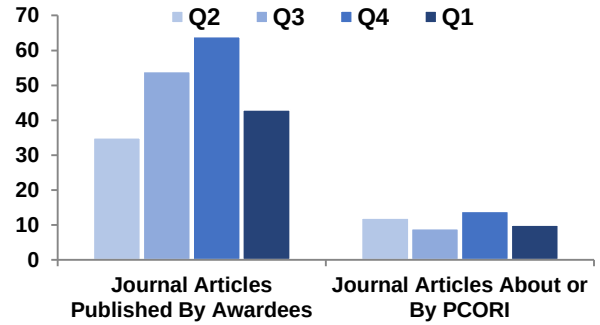
Expenditures – Total Budget, Up to $424M



Final Progress Reports Submitted

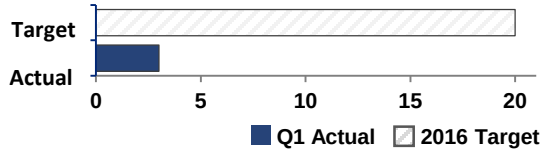


Journal Articles

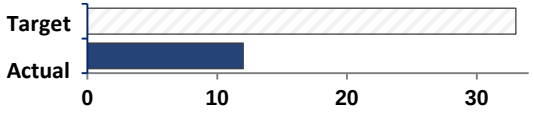


PCORnet Phase II

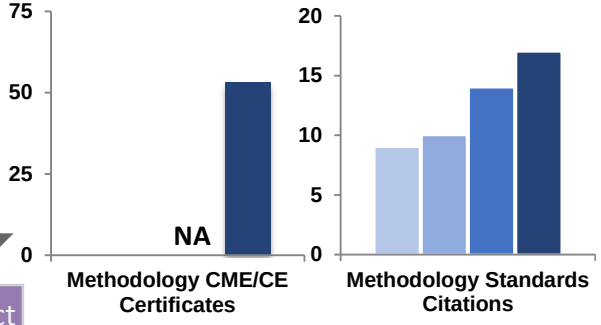
Research Projects Underway in PCORnet



Networks Engaged in Research Projects



Methodology Standards Uptake



Results: Increasing Information

A study aiming to improve patient-centeredness and reduce ordering of low-value tests found that physician education alone may not be sufficient to induce lasting changes in behavior.

Results: Increasing Information

A study on chronic opioid therapy found greater reductions in high-dose prescriptions in group practice settings, which had additional physician initiatives, than among contracted physicians.

Results of Engagement in Research

In this study, **engagement of patients led to the design of a peer-driven intervention for sleep apnea treatment:** a Peer-Buddy approach, which is being compared to standard care. Experienced patients are helping those newly diagnosed with sleep apnea learn to use a challenging but effective treatment. If successful, the Peer-Buddy approach may be a useful tool for treatment of other chronic diseases, such as diabetes, heart failure, and HIV.

Informational Item: Re-release of the Clinical Management of Hepatitis C Infection Targeted PFA

Robert Zwolak, MD, PhD
Science Oversight Committee Chair

Additional Hepatitis C Funding Opportunity

- December 2014: Board approved the Hepatitis C targeted PFA for development: 4 research questions, up to $50M in total
- February 2015: Hepatitis C PFA released
- September 2015: Board awarded 2 projects for a total of $29M
- March 2016: The SOC endorsed the re-release of the Hepatitis C PFA to address 2 revised research questions, up to $21M funds remaining

Funding will be available to fund up to two large clinical comparative effectiveness studies to:

- Assess the short-term patient-centered outcomes relative to timing of treatment (e.g., quality of life, cognitive function, depression, satisfaction, fatigue)
- Ascertain risk for disease progression in early disease patients not authorized for direct-acting antiviral (DAA) treatment

Research Question 1

Patient-Centered Outcomes in Optimal Timing for Hepatitis C Treatments

- Among patients with early stage hepatitis C infection (defined as fibrosis stage 0-2) who do not have immediate access to direct-acting antiviral (DAA) treatment, what are the short-term benefits and harms of DAA treatment on patient-centered outcomes such as quality of life, fatigue, depression, malaise, etc., at treatment end and at 1 year?

  - Note: PCORI encourages a double-blind randomized controlled trial (of immediate treatment vs. placebo) in clinical settings with policies restricting access to DAA treatment in patients with early stage hepatitis C liver disease with up to 1 year follow up looking at various patient reported outcomes such as patient assessment of disease progression, quality of life, functional status and symptoms, as well as clinical outcomes including SVR.

Research Question 2

Long-Term Patient Outcomes in Treatments for Hepatitis C

- What are the long-term outcomes, in terms of liver disease progression and extra-hepatic complications of HCV infection, experienced by patients with early stage (fibrosis stage 0-2) HCV infection, who have not yet received treatment with direct-acting antivirals?
  - PCORI is especially interested in focusing on disproportionally disadvantaged populations who often experience challenges in access to health care services, misuse of alcohol and other substances, and have multiple co-morbid conditions
  - It is envisioned that the proposed research should be a prospective cohort study, such as a patient registry with active surveillance and rich clinical practice-based data, that incorporates patient-centered outcome measures

Timeline

Action	Date
Release Date	April 4, 2016
Letter of Intent Due	May 4, 2016
Application Deadline	August 8, 2016
Merit Review	November 14, 2016
Awards Announced	January 2017

Wrap Up and Adjournment

Larry Becker

Chair, Finance and Administration Committee
Acting Chairperson